As confidentially submitted to the Securities and Exchange Commission on February 14, 2020.
This draft registration statement has not been publicly filed with the Securities and Exchange Commission and all information herein remains strictly confidential.

Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

HARMONY BIOSCIENCES HOLDINGS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**2834**	**82-2279923**
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification No.)

630 W. Germantown Pike, Suite 215
Plymouth Meeting, PA 19462
Telephone: (484) 539-9800
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

John C. Jacobs
Chief Executive Officer
Harmony Biosciences, LLC
630 W. Germantown Pike, Suite 215
Plymouth Meeting, PA 19462
Telephone: (484) 539-9800
(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies to:

Christopher D. Lueking, Esq. **Latham & Watkins LLP** **330 North Wabash Avenue, Suite 2800** **Chicago, IL 60611** **Telephone: (312) 876-7700**	**Stephanie Wisdo** **General Counsel** **Harmony Biosciences, LLC** **630 W. Germantown Pike, Suite 215** **Plymouth Meeting, PA 19462** **Telephone: (484) 539-9800**	**Robert Puopolo, Esq.** **Seo Salimi, Esq.** **Goodwin Procter LLP** **100 Northern Avenue** **Boston, MA 02210** **Telephone: (617) 570-1000**

APPROXIMATE DATE OF COMMENCEMENT OF PROPOSED SALE TO THE PUBLIC: AS SOON AS PRACTICABLE AFTER THIS REGISTRATION STATEMENT IS DECLARED EFFECTIVE.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☐ Accelerated filer ☐ Non-accelerated filer ☒ Smaller reporting company ☒ Emerging growth company ☒

If an emerging growth company, that prepares its financial statements in accordance with U.S. GAAP, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act. ☐

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities to be Registered	**Proposed Maximum Aggregate Offering Price(1)(2)**	**Amount of Registration Fee(3)**
Common Stock, par value $0.00001 value per share	$	$

(1) Includes the aggregate offering price of common stock that may be sold if the option to purchase additional shares of our common stock granted by the Registrant to the underwriters is exercised. See "Underwriting."
(2) Estimated solely for the purpose of calculating the amount of the registration fee in accordance with Rule 457(o) of the Securities Act of 1933, as amended.
(3) To be paid in connection with the initial public filing of the registration statement.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

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The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell nor does it seek an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to Completion. Dated , 2020.

Shares

Common Stock

This is an initial public offering of shares of common stock of Harmony Biosciences Holdings, Inc.

We are offering shares of our common stock.

Prior to this offering, there has been no public market for our common stock. It is currently estimated that the initial public offering price per share of our common stock will be between $ and $. We intend to apply to list our common stock on the under the symbol " ."

We are an "emerging growth company," as that term is used in the Jumpstart Our Business Startups Act of 2012 and, as such, we have elected to comply with certain reduced public company reporting requirements.

Investing in our common stock involves a high degree of risk. See "Risk Factors" beginning on page 14 to read about factors you should consider before buying shares of our common stock.

Neither the Securities and Exchange Commission nor any other regulatory body has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial public offering price	$	$
Underwriting discount(1)	$	$
Proceeds, before expenses, to Harmony Biosciences Holdings, Inc.	$	$

(1) See "Underwriting" for a description of the compensation payable to the underwriters.

To the extent that the underwriters sell more than shares of our common stock, the underwriters have the option to purchase up to an additional shares from us at the initial price to the public less the underwriting discount.

The underwriters expect to deliver the shares of our common stock against payment in New York, New York on , 2020.

Goldman Sachs & Co. LLC **Jefferies** **Piper Sandler**

Prospectus dated , 2020.

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We and the underwriters have not authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any related free writing prospectuses. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. This prospectus is an offer to sell only the shares offered by this prospectus, but only under circumstances and in jurisdictions where it is lawful to do so. The information contained in this prospectus is current only as of its date. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside the United States: We have not, and the underwriters have not, done anything that would permit this offering or the possession or distribution of this prospectus or any free writing prospectus we may provide to you in connection with this offering in any jurisdiction where action for purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of our common stock and the distribution of this prospectus outside the United States. See "Underwriting."

BASIS OF PRESENTATION

As used in this prospectus, unless the context otherwise requires, references to *"we," "us," "our," the "Company," "Harmony," "Harmony Biosciences"* and similar references refer to Harmony Biosciences Holdings, Inc. together with its subsidiary.

Our consolidated financial statements have been prepared in accordance with generally accepted accounting principles in the United States, or GAAP. Our fiscal year ends on December 31 of each year. References to fiscal 2019 and 2019 are references to the year ended December 31, 2019. Our most recent fiscal year ended on December 31, 2019.

In accordance with the policy pronouncements of the staff of the U.S. Securities and Exchange Commission, we are omitting from this draft registration statement our consolidated financial statements for the year ended December 31, 2017 and the nine months ended September 30, 2019 because they relate to historical periods that we believe will not be required to be included in the prospectus at the time we file this registration statement publicly. We intend to amend this registration statement to include all financial information required by Regulation S-X prior to or at the date of such public filing, including the consolidated audited financial statements as of and for the year ended December 31, 2019.

Certain monetary amounts, percentages and other figures included in this prospectus have been subject to rounding adjustments. Percentage amounts included in this prospectus have not in all cases been calculated on the basis of such rounded figures, but on the basis of such amounts prior to rounding. For this reason, percentage amounts in this prospectus may vary from those obtained by performing the same calculations using the figures in our consolidated financial statements included elsewhere in this prospectus. Certain other amounts that appear in this prospectus may not sum due to rounding.

TRADEMARKS

This prospectus includes certain trademarks and trade names, including the registered trademark product name "WAKIX," which we have in-licensed from Bioprojet Société Civile de Recherche, or Bioprojet, for use in the United States, and the registered trademark "KNOW NARCOLEPSY," which are protected under applicable intellectual property laws. We also have trademark applications pending with the U.S. Patent and Trademark Office for "REM AT THE WRONG TIME" and "NON-REM AT THE WRONG TIME" as well as our brand and logo "HB," "HB HARMONY BIOSCIENCES" and "HARMONY BIOSCIENCES." This prospectus also contains trademarks, trade names and service marks of other companies, which are the property of their respective owners. Solely for convenience, trademarks, trade names and service marks referred to in this prospectus may appear without the ®, ™ or SM symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent permitted under applicable law, our rights or the right of the applicable licensor to these trademarks, trade names and service marks. We do not intend our use or display of other parties' trademarks, trade names or service marks to imply, and such use or display should not be construed to imply, a relationship with, or endorsement or sponsorship of us by, these other parties.

PROSPECTUS SUMMARY

This summary highlights selected information contained elsewhere in this prospectus. This summary does not contain all of the information that you should consider before deciding to invest in our common stock. You should read the entire prospectus carefully, including the "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and the related notes included elsewhere in this prospectus, before making an investment decision. Some of the statements in this prospectus constitute forward-looking statements. See "Cautionary Note Regarding Forward-Looking Statements."

Overview

We are a commercial-stage pharmaceutical company focused on developing and commercializing innovative therapies for patients with rare neurological disorders living with unmet medical needs. Our product, WAKIX (pitolisant), is a first-in-class molecule with a novel mechanism of action, or MOA, specifically designed to increase histamine signaling in the brain by binding to H_3 receptors. In August 2019, WAKIX was approved by the U.S. Food and Drug Administration, or the FDA, for the treatment of our lead indication, excessive daytime sleepiness, or EDS, in adult patients with narcolepsy, and its U.S. commercial launch was initiated in November 2019. WAKIX is the first-and-only approved product for patients with narcolepsy that is not scheduled as a controlled substance. We plan to expand the label for WAKIX in narcolepsy and expect to initiate a Phase 3 clinical trial in pediatric patients in pursuit of indications for both EDS and cataplexy, as well as to pursue pediatric exclusivity. In addition, we are evaluating our options regarding the approach to take with the FDA in pursuit of a cataplexy indication in adult patients with narcolepsy. We believe that pitolisant's ability to regulate histamine gives it the potential to provide therapeutic benefit in other rare neurological disorders that are mediated through H_3 receptors and histamine signaling. We are initially focusing on the treatment of EDS associated with Prader-Willi Syndrome, or PWS, and myotonic dystrophy type 1, or DM1. We intend to commence a Phase 2 clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS in the first half of 2020, with topline results expected in the second half of 2021. We are also planning to commence a Phase 2 clinical trial for DM1 in the second half of 2020, with topline results expected in the first half of 2022. Beyond these indications, we intend to further explore pitolisant in other rare neurological disorders in which fatigue and cognitive impairment are prominent symptoms with significant impact on daily functioning.

Narcolepsy is a rare, chronic and debilitating neurologic disorder of sleep-wake state instability that is estimated to affect anywhere from 135,000 to 200,000 Americans, with fewer than 50% diagnosed. Narcolepsy is characterized by EDS, which is present in all patients with narcolepsy and is the primary reason why patients seek treatment. EDS is the inability to stay awake or alert throughout the day, including an irrepressible need for sleep, with lapses into drowsiness or sleep, which has a significant impact on a patient's ability to function. Additional symptoms of narcolepsy may include cataplexy (which is characterized by sudden and transient episodes of muscle weakness accompanied by full conscious awareness), hallucinations, sleep paralysis and disrupted nighttime sleep. In most patients, narcolepsy is caused by the loss of hypocretin, a neuropeptide in the brain that, along with histamine, works to support sleep-wake state stability. The U.S. narcolepsy market had an approximate net sales value of $1.8 billion in 2019, which is expected to grow due to the addition of newly approved therapies, increased physician education and patient awareness, and increased diagnosis rates, among other factors.

Prior to the approval of WAKIX, there were six approved medications to treat patients with narcolepsy, all of which are scheduled as controlled substances: Xyrem (sodium oxybate), Provigil

(modafinil), Nuvigil (armodafinil), methylphenidate, amphetamine and Sunosi (solriamfetol). Other prescription drugs are used off-label for the treatment of either EDS or cataplexy in patients with narcolepsy, including stimulants and antidepressants. Some of the current therapies have significant side effects (such as increased heart rate and blood pressure) and boxed warnings due to the risk of respiratory depression, abuse and dependence. These therapies also have the potential for rebound and withdrawal symptoms. The Voice of the Patient report from the FDA's patient-focused drug development initiative, published in 2014, concluded that, based on the overall benefit-risk assessment of current medications, there is a continued need for additional effective and tolerable treatment options for patients with narcolepsy. Similarly, in market research sponsored by us prior to the commercial release of WAKIX, both patients and healthcare professionals, or HCPs, expressed frustration and dissatisfaction with then-existing therapies, reflecting current unmet medical needs. These unmet needs included, in order of importance, the availability of: (i) non-scheduled treatment options, (ii) more tolerable treatment regimens, (iii) more effective treatment options, (iv) novel MOAs beyond currently available therapies and (v) once-daily treatment options.

Clinical Development of WAKIX (pitolisant)

The strategy behind the clinical development of pitolisant is based on its MOA. Pitolisant is a first-in-class molecule with a novel MOA, acting as a potent and highly selective antagonist/inverse agonist of the H_3 receptor. It activates histaminergic neurons in the brain, a neuronal system involved in the maintenance of wakefulness, attention, vigilance and cognition. Pitolisant binds to H_3 receptors on presynaptic neurons and blocks the normal negative feedback mechanism for histamine release, resulting in increased release of this wake-promoting neurotransmitter. It also functions as an inverse agonist, resulting in enhanced histamine synthesis and release from presynaptic neurons. Increased histamine available in the synapse binds to postsynaptic H_1 receptors, activating postsynaptic neurons, which stimulate wake-promoting brain regions and inhibit sleep-promoting regions of the brain.

Pitolisant also stimulates the release of other wake-promoting neurotransmitters (dopamine, norepinephrine, serotonin and acetylcholine) via H_3 heteroreceptors within those neuronal systems. Importantly, pitolisant does not increase dopamine levels in the striatum, including the nucleus accumbens, which is the pleasure center of the brain where increase in dopamine levels is correlated with abuse potential. This feature of pitolisant's MOA, along with primarily working through the histaminergic system, are two of the aspects that differentiate pitolisant from all other currently approved treatments for narcolepsy.

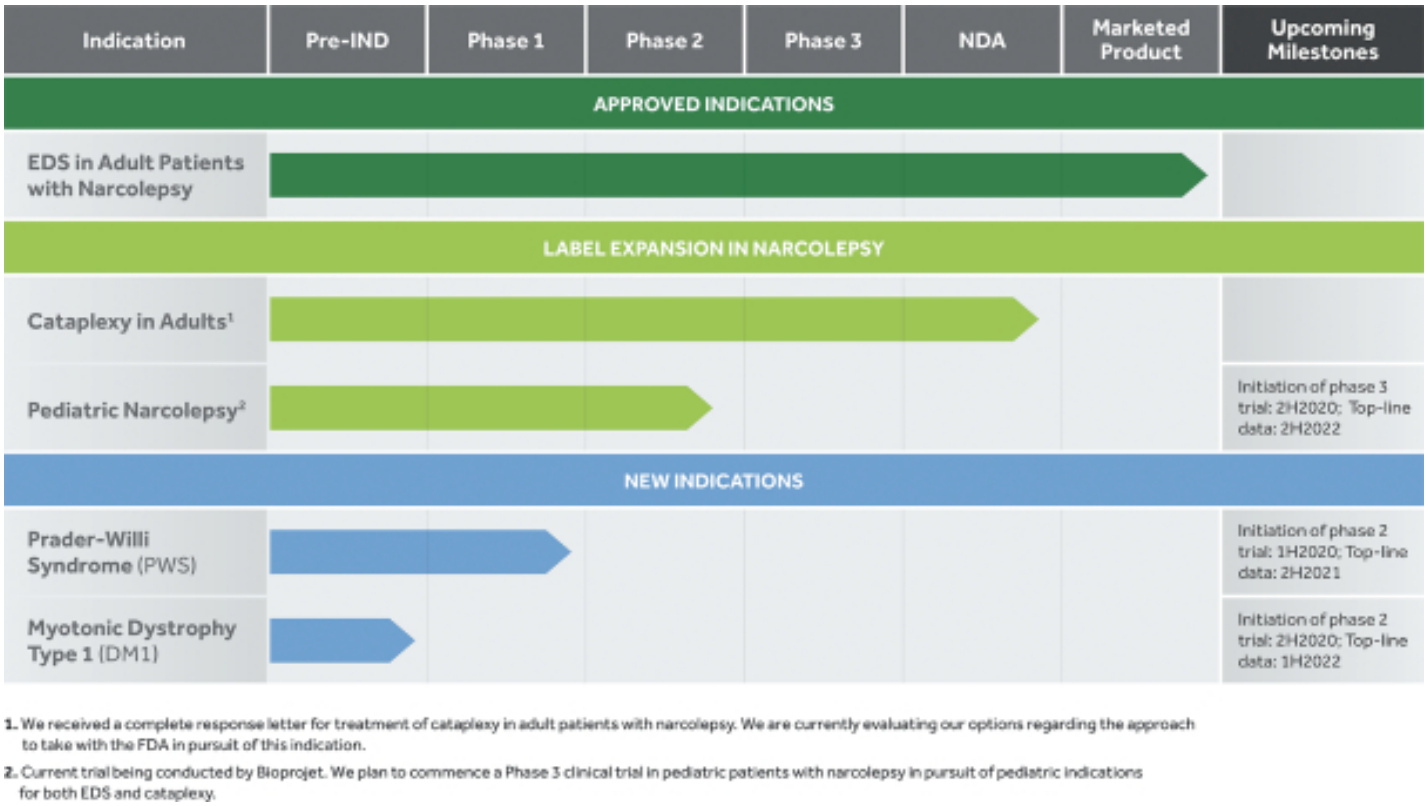
The safety profile of pitolisant is based on pooled safety data from 22 Phase 2/3 clinical trials conducted by our licensor Bioprojet Société Civile de Recherche, or Bioprojet, eight of which were in patients with narcolepsy and 14 of which were in other indications. These trials included a total of 1,513 unique patients, of whom 1,043 received pitolisant in double-blind, placebo-controlled studies, and others received pitolisant in single-blind or open-label trials. Three successful pivotal trials in narcolepsy, HARMONY 1, HARMONY 1bis, and HARMONY CTP, were completed in Europe by Bioprojet and served as the foundation for the approval of pitolisant by the European Medicines Agency, or EMA, in 2016 for the treatment of narcolepsy in adults with or without cataplexy. Pitolisant was evaluated in a long-term safety and tolerability trial, HARMONY 3, which further supported the results observed in HARMONY 1, HARMONY 1bis, and HARMONY CTP. We submitted the data from these same trials, along with a human abuse potential, or HAP, trial, to the FDA as part of the NDA for WAKIX (pitolisant), which the FDA approved on August 14, 2019 for the treatment of EDS in adult patients with narcolepsy.

WAKIX for Narcolepsy

WAKIX (pitolisant) represents a novel approach to narcolepsy treatment. We believe that WAKIX offers a meaningfully differentiated product profile over current treatment options for the following reasons:

- ***First-in-class molecule with a novel MOA***. WAKIX is the only selective H_3 receptor antagonist/inverse agonist approved by the FDA for the treatment of EDS in adult patients with narcolepsy and is the only narcolepsy treatment that works primarily through histamine, a major wake-promoting neurotransmitter.
- ***First-and-only non-scheduled treatment for narcolepsy***. WAKIX is the first-and-only FDA-approved treatment for narcolepsy that is not scheduled as a controlled substance by the U.S. Drug Enforcement Administration, or the DEA. In a clinical trial, pitolisant demonstrated statistically significantly lower drug liking compared to phentermine (a Schedule IV stimulant), consistent with its lack of abuse potential.
- ***WAKIX is not a stimulant***. Unlike stimulants, WAKIX has shown no evidence for the development of drug tolerance or withdrawal symptoms. Therefore, there is no need for patients to temporarily stop the medication to reset efficacy. In addition, unlike stimulants, WAKIX does not increase dopamine levels in the pleasure center of the brain, which contributes to its lack of abuse potential.
- ***WAKIX can be used as monotherapy or administered concomitantly with other narcolepsy treatments***. Narcolepsy is a difficult disorder to manage and the majority of narcolepsy patients often require multiple medications to treat their symptoms. WAKIX was studied in combination with each of modafinil and sodium oxybate (two common treatments for narcolepsy) and demonstrated no effect on the pharmacokinetic, or PK, profile of either treatment, and neither treatment had a clinically relevant effect on the PK profile of WAKIX.
- ***WAKIX is a once-daily oral tablet administered in the morning upon wakening***. Patients have identified a need for treatment options that are easier to take and are dosed less frequently. We believe that once-daily dosing with WAKIX addresses this need and may help improve patient compliance with treatment.

Overview of Development Pipeline



1. We received a complete response letter for treatment of cataplexy in adult patients with narcolepsy. We are currently evaluating our options regarding the approach to take with the FDA in pursuit of this indication.
2. Current trial being conducted by Bioprojet. We plan to commence a Phase 3 clinical trial in pediatric patients with narcolepsy in pursuit of pediatric indications for both EDS and cataplexy.

Potential New Indications for Pitolisant

Label Expansion

We are actively working on label expansion for WAKIX in narcolepsy, including a Phase 3 clinical trial in pediatric patients to support FDA approval for indications for both EDS and cataplexy in pediatric patients. We also intend to work with the FDA toward obtaining pediatric exclusivity for WAKIX. In addition, following the FDA's decision not to grant approval for the treatment of cataplexy in adult patients with narcolepsy, we are evaluating our options regarding the approach to take with the FDA in pursuit of this indication.

Additional Indications

We believe that pitolisant's ability to regulate histamine gives it the potential to provide therapeutic benefit in other rare neurological disorders that are mediated through the H_3 receptor and histamine signaling. We plan to explore the potential benefit of pitolisant in additional rare neurological indications beyond narcolepsy, initially focusing on the treatment of EDS associated with PWS and DM1.

PWS is a rare genetic disorder caused by a loss of function of specific genes on chromosome 15 resulting in hypothalamic dysfunction. The hypothalamus controls both sleep-wake states and hunger-satiety. Therefore, two of the main symptoms in patients with PWS are EDS and insatiable hunger, or hyperphagia. It is estimated that approximately one in 12,000 to 15,000 people in the United States suffers from PWS. We completed a Phase 1 PK clinical trial in pediatric patients with PWS in the fourth quarter of 2019, and initiated a long-term, open-label safety study in these patients. We intend to commence a Phase 2 clinical trial in patients with PWS in the first half of 2020. Topline results from this clinical trial are expected in the second half of 2021.

DM1 is a rare, multi-system genetic disease that affects the neuromuscular system as well as several other systems. It is inherited in an autosomal dominant pattern and the underlying cause of DM1 is a mutation in the myotonic dystrophy protein kinase gene on chromosome 19. DM1 is the most common form of adult-onset muscular dystrophy and affects as many as 40,000 patients in the United States. EDS and fatigue are hallmark clinical characteristics in patients with DM1 and are referred to as the most frequent non-muscular symptoms in patients with DM1. Cognitive impairment is also a prominent symptom in patients with DM1 and all of these symptoms are thought to be mediated through H_3 receptors and histaminergic pathways located throughout the central nervous system, or CNS. We anticipate commencing a Phase 2 clinical trial in patients with DM1 in the second half of 2020. Topline results from this clinical trial are expected in the first half of 2022.

Our Strategy

Our goal is to become a leading pharmaceutical company dedicated to developing and commercializing novel treatment options for patients with rare neurological disorders living with unmet medical needs, beginning with a focus on narcolepsy. The key elements of our strategy are to:

- ***Commercialize WAKIX in the United States***. We have assembled a team of approximately 150 professionals that possess comprehensive life sciences experience. We have also established a robust company infrastructure to execute on our core business and growth strategies. This team includes over 70 dedicated and experienced sales professionals who call on the approximately 8,000 HCPs who treat the majority of narcolepsy patients in the United States. In the fourth quarter of 2019, we launched WAKIX in the United States and the product became commercially available in early November 2019.
- ***Expand our Label in Narcolepsy***. Building upon an EDS indication in adult patients with narcolepsy, we plan to initiate a pediatric development program in the second half of 2020 with the goal of gaining a pediatric indication for both EDS and cataplexy as well as obtaining pediatric exclusivity. In addition, we are evaluating our options regarding the approach to take with the FDA in pursuit of a cataplexy indication in adult patients with narcolepsy.
- ***Expand Into New Indications Beyond Narcolepsy***. We believe that pitolisant's ability to regulate histamine gives it the potential to provide therapeutic benefit in other rare neurological disorders that are mediated through the H_3 receptor and histamine signaling. We plan to explore the potential benefit of pitolisant in additional rare neurological indications beyond narcolepsy, initially focusing on the treatment of EDS associated with PWS and DM1. Beyond these indications, we intend to further explore pitolisant in other rare neurological disorders in which fatigue and cognitive impairment are prominent symptoms with significant impact on daily functioning.
- ***Explore Expansion of our Product Portfolio***. We plan to explore obtaining additional licensing rights from Bioprojet to expand into certain international markets with WAKIX. As we continue our commercial growth and develop a global footprint, we will assess in-licensing or acquiring complementary rights, assets or product candidates that allow us to leverage our existing infrastructure and expand within our strategic areas of focus.

Company History and Management Team

Our operating subsidiary, Harmony Biosciences, LLC, was formed in May 2017. We were formed in July 2017 as Harmony Biosciences II, LLC, a Delaware limited liability company, and we converted to a Delaware corporation named Harmony Biosciences II, Inc. in September 2017. We concurrently acquired an exclusive license to develop, manufacture and commercialize pitolisant in the United States from Bioprojet. In February 2020, we changed our name to Harmony Biosciences Holdings, Inc. Since

founding, we have assembled an experienced leadership team with a track record of developing and commercializing products to treat rare neurological disorders. Our President and Chief Executive Officer is John Jacobs, who has held a variety of senior leadership roles of increasing responsibility throughout his career including roles in marketing, commercial, operations and general management in both U.S. and global markets. Jeffrey Dierks, our Chief Commercial Officer, has over 20 years of commercial leadership experience with demonstrated success in leading product launches. Jeffrey Dayno, MD, our Chief Medical Officer, is a neurologist with 10 years of experience in clinical and academic medicine followed by over 20 years of experience in research and development leadership roles at Merck & Co., Inc., Cephalon, Inc. and ViroPharma Incorporated.

Summary Risk Factors

Investing in our common stock involves substantial risk. Our ability to execute our strategy is also subject to certain risks. The risks described under the heading “Risk Factors” included elsewhere in this prospectus may cause us not to realize the full benefits of our strengths or may cause us to be unable to successfully execute all or part of our strategy. Some of the most significant challenges and risks include the following:

- We have incurred significant losses since our inception, expect to incur significant losses for the foreseeable future and may never achieve or maintain profitability.
- We have only generated limited revenue from product sales and may never be profitable.
- We have a limited operating history and no history of commercializing drugs, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.
- We have only limited capital and, even if we consummate this offering, may need to raise additional capital before we become profitable.
- Raising additional funds by issuing securities may cause dilution to existing shareholders, raising additional funds through debt financings may involve restrictive covenants, and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights to our technologies or product candidates.
- Our management has expressed substantial doubt about our ability to continue as a going concern.
- We may be required to make significant payments to Bioprojet under our licensing and collaboration agreements for pitolisant.
- We are substantially dependent on our ability to successfully commercialize WAKIX, which is currently our only approved product. If we are unable to successfully commercialize WAKIX, our ability to generate revenue and our financial condition will be adversely affected.
- The commercial adoption of WAKIX and any other product candidates we develop will depend on the degree of their market acceptance.
- We rely on our license agreement with Bioprojet to provide rights to the core intellectual property relating to pitolisant, and any termination or loss of significant rights under the agreement would adversely affect our development and/or commercialization of pitolisant.
- Because a number of companies compete with us, many of which have greater resources than we do, and because we face rapid changes in science in our industry, we cannot be certain that our products will be accepted in the marketplace or capture market share.

- The regulatory approval process of the FDA is costly, lengthy and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for pitolisant in other potential indications for which we may seek to develop pitolisant, our business will be substantially harmed.
- If we fail to obtain and sustain an adequate level of coverage and reimbursement for WAKIX and other product candidates by third-party payors, sales would be adversely affected.
- WAKIX has been approved by the FDA for the treatment of EDS in adult patients with narcolepsy. Regulatory approval is limited by the FDA to the specific indication for which approval has been granted and, unless we seek regulatory approval for additional indications, we will be prohibited from marketing pitolisant for other indications. We may be subject to fines, penalties or injunctions if we are determined to have promoted or be promoting the use of pitolisant for unapproved or "off-label" uses, resulting in damage to our reputation and business.

Our Corporate Information

Our corporate headquarters are located at 630 W. Germantown Pike, Suite 215, Plymouth Meeting, Pennsylvania 19462. Our telephone number is (484) 539-9800. Our principal website address is *www.harmonybiosciences.com*. The information on or accessed through our website is not incorporated in this prospectus or the registration statement of which this prospectus forms a part.

Implications of Being an Emerging Growth Company

We qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. An emerging growth company may take advantage of certain reduced reporting and other requirements that are otherwise generally applicable to public companies. As a result:

- we are required to present only two years of audited financial statements and two years of related selected financial data and Management's Discussion and Analysis of Financial Condition and Results of Operations disclosure;
- we are not required to engage an auditor to report on our internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act;
- we are not required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board, or the PCAOB, regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements (i.e., critical audit matters);
- we are not required to submit certain executive compensation matters to stockholder advisory votes, such as "say-on-pay," "say-on-frequency" and "say-on-golden parachutes;" and
- we are not required to comply with certain disclosure requirements related to executive compensation, such as the requirement to disclose the correlation between executive compensation and performance and the requirement to present a comparison of our Chief Executive Officer's compensation to our median employee compensation.

We may take advantage of these reduced reporting and other requirements until the last day of our fiscal year following the fifth anniversary of the completion of this offering, or such earlier time that we are no longer an emerging growth company. However, if certain events occur prior to the end of such five-

year period, including if we have more than $1.07 billion in annual gross revenue, have more than $700 million in market value of our common stock held by non-affiliates, or issue more than $1.0 billion of non-convertible debt over a three-year period, we will cease to be an emerging growth company prior to the end of such five-year period. We may choose to take advantage of some but not all of these reduced burdens. We have elected to adopt the reduced requirements with respect to our financial statements and the related selected financial data and Management's Discussion and Analysis of Financial Condition and Results of Operations disclosure. As a result, the information that we provide to stockholders may be different from the information you may receive from other public companies in which you hold equity.

In addition, under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to take advantage of the longer phase-in periods for the adoption of new or revised financial accounting standards under the JOBS Act until we are no longer an emerging growth company. Our election to use the phase-in periods permitted by this election may make it difficult to compare our financial statements to those of non-emerging growth companies and other emerging growth companies that have opted out of the longer phase-in periods permitted under the JOBS Act and who will comply with new or revised financial accounting standards. If we were to subsequently elect instead to comply with public company effective dates, such election would be irrevocable pursuant to the JOBS Act.

We are also a "smaller reporting company" as defined in the rules promulgated under the Securities Exchange Act of 1934, as amended, or the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates on the last business day of our second fiscal quarter is less than $250.0 million, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and nonvoting common stock held by non-affiliates on the last business day of our second fiscal quarter in that fiscal year is less than $700.0 million.

THE OFFERING

Common stock offered by us	shares.
Option to purchase additional shares	shares.
Common stock to be outstanding after this offering	shares (or shares if the underwriters exercise their option to purchase additional shares in full).
Use of proceeds	We estimate, based upon an assumed initial public offering price of $ per share (which is the midpoint of the price range set forth on the cover page of this prospectus), that we will receive net proceeds from this offering of approximately $ million (or $ million if the underwriters exercise their option to purchase additional shares of common stock in full), after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. We currently estimate that we will use the net proceeds from this offering to fund the clinical development of additional indications for pitolisant in PWS, DM1 and pediatric narcolepsy, and for working capital, business development opportunities, potential milestone payments to Bioprojet and general corporate purposes, including to support the continued commercialization of WAKIX in the United States. See “Use of Proceeds.”
Risk factors	See “Risk Factors” beginning on page 14 and the other information included in this prospectus for a discussion of factors you should carefully consider before deciding to invest in shares of our common stock.
Dividend policy	The terms of our current certificate of incorporation provide that, upon the conversion of our Series A preferred stock, our Series B preferred stock and our Series C preferred stock into shares of our common stock upon the closing of this offering, each holder of our Series A preferred stock, our Series B preferred stock and our Series C preferred stock will receive a cumulative accrued dividend calculated at a rate per annum of 10% of the applicable issue price of such series of preferred stock, in each case, compounded annually, payable, at the determination of our board of directors, in either (i) shares of common stock or (ii) cash in an aggregate amount equal to the cumulative accrued dividend. We intend to pay the

	cumulative accrued dividend in shares of common stock. Assuming we pay the cumulative accrued dividend in shares of common stock, the cumulative accrued dividend will be issued to each holder of preferred stock as of immediately prior to the closing of this offering a number of shares of common stock equal to (x) the aggregate amount of the accrued dividend held by such holder and not previously paid as of immediately prior to the closing of this offering divided by (y) the actual price per share of common stock sold to the public in this offering. Based on the midpoint of the price range set forth on the cover page of this prospectus, we expect to issue (i) shares of our common stock for cumulative accrued dividends to holder of our Series A preferred stock, (ii) shares of our common stock for cumulative accrued dividends to holders of our Series B preferred stock and (iii) shares of our common stock to holders of our Series C preferred stock. The stock dividends will not be paid on any shares of our common stock purchased in this offering. We do not pay dividends on our common stock and do not anticipate paying any dividends on our common stock for the foreseeable future. Any future determinations relating to our dividend policy will be made at the discretion of our board of directors and will depend on various factors. See "Dividend Policy."
Proposed Market symbol	" "

The number of shares of common stock to be outstanding after this offering is based on shares of our common stock outstanding as of December 31, 2019, and includes an additional shares of our common stock issuable upon (i) the conversion of all outstanding shares of our convertible preferred stock immediately prior to the closing of this offering into shares of common stock and (ii) the payment of an accrued dividend to holders of our convertible preferred stock in the aggregate amount of shares of our common stock which becomes due and payable to such holders upon the conversion of their convertible preferred stock upon the closing of this offering, and excludes:

- shares of common stock issuable upon exercise of outstanding stock options granted under the Harmony Biosciences II, Inc. Equity Incentive Plan, or the Equity Incentive Plan, as of December 31, 2019, at a weighted average exercise price of $ per share;
- shares of common stock available for future issuance under the Equity Incentive Plan as of December 31, 2019; and
- shares of our common stock that will become available for future issuance under our 2020 Incentive Award Plan, or the 2020 Plan, which will become effective in connection with the completion of this offering.

Unless we indicate otherwise or the context otherwise requires, all information in this prospectus assumes or gives effect to:

- the filing and effectiveness of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws, each of which will occur immediately prior to the closing of this offering;
- the conversion of all outstanding shares of our Series A preferred stock, Series B preferred stock and Series C preferred stock into shares of our common stock immediately prior to the closing of this offering;
- a for split of our common stock, effected on , 2020;
- no exercise of the outstanding options described above after December 31, 2019;
- no exercise by the underwriters of their option to purchase up to additional shares of common stock; and
- an initial public offering price of $ per share of common stock, which is the midpoint of the range set forth on the cover page of this prospectus.

Summary Consolidated Financial Data

The following tables present our summary consolidated financial data. We have derived the summary consolidated statements of operations data for the year ended December 31, 2018 and 2019 and the summary consolidated balance sheet data as of December 31, 2018 and 2019 from our audited consolidated financial statements included elsewhere in this prospectus. You should read this data together with our consolidated financial statements and related notes included elsewhere in this prospectus and the sections titled "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations." Our historical results for any prior period are not necessarily indicative of our future results.

Consolidated Statement of Operations Data: *(U.S. dollars in thousands except share and per share data)*	**Year Ended December 31, 2018**	**Year Ended December 31, 2019**
Net product revenue	$ —	$
Cost of product sales	—	
Gross profit	—	
Operating expenses:		
Research and development	$ 12,372	$
Sales and marketing	16,861	
General and administrative	12,206	
Total operating expenses	41,439	
Operating loss	(41,439)	
Interest income (expense)	1,541	
Loss before taxes	(39,898)	
Income taxes	—	
Net loss and comprehensive loss	$ (39,898)	$
Accumulation of yield on preferred stock	(30,185)	
Net loss available to common stockholders	$ (70,083)	$
Loss per share:		
Loss per share, basic and diluted(1)	$ (0.96)	$
Weighted average number of common stock, basic and diluted	72,765,366	
Pro Forma net loss per share, basic and diluted (unaudited)(1)	$	$
Pro Forma weighted average shares of common stock outstanding, basic and diluted (unaudited)		

(1) See Note 12 to our financial statements for the year ended December 31, 2018 appearing at the end of this prospectus for further details on the calculation of basic and diluted net loss per share attributable to common stockholders.

Consolidated Balance Sheet Data: *(U.S. dollars in thousands except share and per share data)*	**As of December 31, 2019**		
	Actual	**Pro Forma(2)**	**Pro Forma As Adjusted(3)**
Cash and cash equivalents	$		
Working capital(1)			
Total assets			
Long-term debt, net of current portion			
Convertible preferred stock			
Total stockholders' (deficit) equity			

(1) We define working capital as current assets less current liabilities.
(2) The pro forma balance sheet data give effect (i) the conversion of all outstanding shares of our convertible preferred immediately prior to the closing of this offering into shares of common stock and (ii) the payment of an accrued dividend to holders of our convertible preferred stock in the aggregate amount of shares of our common stock which becomes due and payable to such holders upon the conversion of their convertible preferred stock upon the closing of this offering.
(3) The pro forma as adjusted balance sheet data give further effect to our issuance and sale of shares of our common stock in this offering at an assumed initial public offering price of $ per share (which is the midpoint of the price range set forth on the cover page of this prospectus) after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share (which is the midpoint of the price range set forth on the cover page of this prospectus) would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Each increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by $ million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this prospectus, including our consolidated financial statements and the related notes and the section "Management's Discussion and Analysis of Financial Condition and Results of Operations," before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could materially and adversely affect our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline, and you may lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also impair our business operations.

Risks Related to Our Financial Condition and Capital Requirements

We have incurred significant losses since our inception, expect to incur significant losses for the foreseeable future and may never achieve or maintain profitability.

Investment in pharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that a product candidate will fail to gain regulatory approval or fail to become commercially viable. We have only recently begun to generate revenue from product sales and have incurred losses in each year since our inception. Our ability to generate revenue and achieve profitability depends on our ability to successfully commercialize WAKIX for the treatment of excessive daytime sleepiness, or EDS, in adult patients with narcolepsy, and to successfully develop and obtain the regulatory approvals necessary to commercialize pitolisant for other indications. We expect to continue to incur losses for the foreseeable future, and we anticipate these losses will increase as we commercialize WAKIX and as we continue to develop and potentially commercialize pitolisant for other indications.

We have only generated limited revenue from product sales and may never be profitable.

Other than WAKIX, we do not currently have any products that are available for commercial sale, and we may never achieve profitability. Our net loss was $39.9 million and $ million for the years ended December 31, 2018 and 2019, respectively. As of December 31, 2019, we generated revenue of $ million and had an accumulated deficit of $ million. Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue until we further commercialize WAKIX and obtain regulatory approval for potential additional indications for pitolisant, or any other product candidates we may develop. Successful commercialization will require achievement of many key milestones, including demonstrating safety and efficacy in clinical trials, obtaining regulatory approval, including marketing approval for these product candidates, manufacturing, marketing and selling those products for which we, or any of our future collaborators, may obtain regulatory approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately and precisely predict the timing and amount of revenues, the extent of any further losses or if or when we might achieve profitability. We and any future collaborators may never succeed in these activities and, even if we do, or any future collaborators do, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Our failure to become and remain profitable may depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. If we continue to suffer losses as we have in the past, investors may not receive any return on their investment and may lose their entire investment.

We have a limited operating history and no history of commercializing drugs, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We commenced operations in 2017, and our operations to date have been largely focused on staffing our company, business planning, raising capital, acquiring the rights to pitolisant, seeking registration in the United States for our product WAKIX, which is approved for the treatment of EDS in adult patients with narcolepsy, commercialization efforts associated with WAKIX and preparing to develop pitolisant for other potential indications. This has included preparing the application for regulatory approval and other activities that were required for us to obtain approval of our New Drug Application, or NDA, and activities related to preparing for the commercialization of WAKIX. WAKIX is our only drug candidate for which we have obtained regulatory approval. We have not yet demonstrated our ability to successfully manufacture a drug on a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing drugs.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We need to continue to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors, and may not be successful in such a transition.

We have only limited capital and, even if we consummate this offering, may need to raise additional capital before we become profitable.

As of December 31, 2019, we had revenue of $ million, an accumulated deficit of $ million and available cash of $ million. We have $200.0 million of debt outstanding under our credit agreement, or the Credit Agreement, with OrbiMed Royalty & Credit Opportunities III, LP, or OrbiMed. We believe that our existing cash as of , 2020 and the estimated net proceeds from this offering will be sufficient to meet our anticipated cash requirements through at least . This estimate is based on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we currently expect. Because the length of time and activities associated with the successful development of our product candidates is highly uncertain, we are unable to estimate with certainty the actual funds we will require for development and any approved marketing and commercialization activities.

To fund future operations to the point at which we are able to generate positive cash flow from sales of WAKIX or other potential product candidates, we may need to raise significant additional capital. The amount and timing of future funding requirements will depend on many factors, including, but not limited to:

- the progress and results of our commercialization of WAKIX;
- the effect of competing technological and market developments;
- the cost and timing of commercial-scale manufacturing activities;
- the payment of licensing fees to Bioprojet Société Civile de Recherche, or Bioprojet;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA and other regulatory authorities;
- the willingness of the FDA and other comparable regulatory authorities to accept our clinical trial designs, as well as data from our completed and planned clinical trials and preclinical

studies and other work, as the basis for the review and approval of pitolisant for other potential indications or of any other product candidates;

- the potential expansion of our current development programs to seek new indications for pitolisant, potential new development programs for additional indications, and related general and administrative support;
- the initiation, progress, timing, and results of our clinical trials through all phases of development for pitolisant as a treatment for other indications and any other product candidates;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights, in-licensed or otherwise;
- the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us for pitolisant or future product candidates;
- the cost of acquiring rights to other pharmaceutical products in the future to further develop and commercialize;
- the cost of general operating expenses;
- the cost of establishing sales, marketing and distribution capabilities for our product candidates in regions where those product candidates are approved and where we choose to commercialize our products on our own; and
- the costs of operating as a public company.

Other than our Credit Agreement with OrbiMed, we have no committed source of additional capital and we anticipate that we may seek to fund our operations through public or private equity offerings, debt financings, collaborations, licensing arrangements or other sources, or any combination of the foregoing. We cannot assure you that anticipated additional financing will be available to us on favorable terms, or at all. Although we have been successful in obtaining financing through the issuance of our equity securities and debt facilities, we cannot assure you that we will be able to do so in the future. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us to fund our commercialization of WAKIX and clinical development and commercialization of pitolisant for other indications, if approved, and other business activities, we could be forced to significantly delay, scale back, or discontinue the development or commercialization of our product candidates or curtail or cease our operations.

Raising additional funds by issuing securities may cause dilution to existing shareholders, raising additional funds through debt financings may involve restrictive covenants, and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights to our technologies or product candidates.

We expect that significant additional capital will be needed in the future to continue our planned operations. Until such time, if ever, that we can generate substantial product revenue from the sale of WAKIX, we expect to finance our cash needs through a combination of equity offerings, debt financings, including our Credit Agreement, strategic alliances and license and development agreements or other collaborations. To the extent that we raise additional capital by issuing equity securities, our existing shareholders' ownership may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that could adversely affect the rights of a common shareholder. Additionally, any agreements for future debt or preferred equity financings, if available, may involve covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely affect our ability to conduct our business.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise develop and market ourselves.

Our management has expressed substantial doubt about our ability to continue as a going concern.

The consolidated financial statements have been prepared as though we will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. We have incurred operating losses and negative cash flows from operations since inception. As of December 31, 2018, and 2019, we have an accumulated deficit of $242.7 million and $ million, respectively. Management expects to continue to incur operating losses and negative cash flows from operations in 2020. In addition, we are subject to two further milestone payments pursuant to our license agreement with Bioprojet: (i) a milestone payment of $40.0 million upon the attainment of aggregate net sales of WAKIX in the United States of $500.0 million subsequent to the date of NDA approval by the FDA and (ii) a milestone payment of $102.0 million if we receive NDA approval from the FDA for a cataplexy indication. We have financed our operations to date with proceeds from the sale of preferred securities and drawing down on (i) a loan agreement with CRG Servicing LLC that has since been repaid in full and (ii) our Credit Agreement.

If we are unable to successfully complete this offering, we will need to create alternate financing or operational plans to continue as a going concern. There can be no assurance that such alternate financing, if available, can be obtained on acceptable terms. If we are unable to obtain such alternate financing, future operations would need to be scaled back or discontinued.

Accordingly, these factors raise substantial doubt about our ability to continue as a going concern within one year after the date the consolidated financial statements are issued. The consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might be necessary should we be unable to continue as a going concern.

We may be required to make significant payments to Bioprojet under our licensing and collaboration agreements for pitolisant.

Under our agreements with Bioprojet, we are subject to significant obligations, including payment obligations upon the achievement of specified milestones and payments based on product sales, as well as other material obligations. Certain of the milestone payments payable by us under these agreements were paid prior to our commercialization of WAKIX. We may be required to make additional milestone payments of up to $142.0 million in the future prior to the time at which we are able to generate significant revenue from sales of WAKIX. There can be no assurance that we will have the funds necessary to make such payments, or be able to raise such funds when needed, on terms acceptable to us, or at all. If we fail to comply with our payment obligations, Bioprojet may have the right to terminate the license agreement, in which event we would not be able to develop, manufacture or market WAKIX or any other pitolisant-based product candidate. Furthermore, if we are forced to raise additional funds to make such payments, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts.

Our ability to utilize our net operating loss carryforwards may be limited.

As of December 31, 2019, we had U.S. federal and state net operating loss carryforwards of approximately $ million and $ million, respectively. Our ability to utilize our federal net operating loss carryforwards may be limited under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code. The limitations apply if we experience an "ownership change," which is generally defined as a greater than 50 percentage point change (by value) in the ownership of our equity by certain stockholders over a rolling three-year period. Similar provisions of state tax law may also apply to limit the use of our state net operating loss carryforwards. We have not assessed whether such an ownership change has previously occurred. If we have experienced an ownership change at any time since our incorporation, we may already be subject to limitations on our ability to utilize our existing net operating loss carryforwards to offset taxable income. In addition, future changes in our stock ownership, which may be outside of our control, may trigger an ownership change and, consequently, the limitations under Section 382 of the Code. As a result, if or when we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset such taxable income may be subject to limitations, which could adversely affect our future cash flows.

Our credit agreement contains restrictive and financial covenants that may limit our operating flexibility.

Our Credit Agreement with OrbiMed contains certain restrictive covenants that either limit our ability to, or require a mandatory prepayment in the event that, we engage in new lines of business, incur additional indebtedness or liens, make certain investments, make certain payments, pay cash dividends, merge with other companies or consummate certain changes of control, acquire other companies, transfer or dispose of certain assets, liquidate or dissolve, amend certain material agreements, enter into sale and leaseback transactions, enter into various other specified transactions, or change our name, location, executive office or executive management without notice. We, therefore, may not be able to engage in any of the foregoing transactions unless we obtain the consent of OrbiMed or prepay the outstanding amount under the Credit Agreement. The Credit Agreement also contains certain financial covenants, including minimum revenue and cash balance requirements (which include maintaining minimum liquidity of $12.5 million), and financial reporting requirements. Our obligations under the Credit Agreement are secured by all of our property, with certain exceptions. We may not be able to generate sufficient cash flow or sales to meet the financial covenants or pay the principal and interest under the Credit Agreement. Furthermore, our future working capital, borrowings or equity financing could be unavailable to repay or refinance the amounts outstanding under the Credit Agreement. In the event of a liquidation, OrbiMed would be repaid all outstanding principal and interest prior to distribution of assets to unsecured creditors, and the holders of our common stock would receive a portion of any liquidation proceeds only if all of our creditors then existing, including OrbiMed, were first repaid in full.

Risks Related to Our Business

We are substantially dependent on our ability to successfully commercialize WAKIX, which is currently our only approved product. If we are unable to successfully commercialize WAKIX, our ability to generate revenue and our financial condition will be adversely affected.

Since our inception, we have invested substantially all of our capital resources on the development, registration and commercialization of WAKIX, which was approved for the treatment of EDS in adult patients with narcolepsy in August 2019. We cannot be certain that WAKIX will be successfully commercialized.

Our ability to generate revenue from product sales depends heavily on our success in many areas, including but not limited to:

- successfully commercializing WAKIX, either independently or with marketing service providers;

- the effectiveness of our sales and marketing strategy and operations, and obtaining market acceptance of WAKIX, including garnering market share from existing and future treatment alternatives;
- maintaining compliance with all regulatory requirements applicable to WAKIX and our commercial activities, including the post-marketing requirements and post-marketing commitments required by the FDA;
- obtaining coverage and adequate reimbursement from third-party payors for each of our product candidates;
- the continued acceptability of the safety profile of WAKIX and the occurrence of any unexpected side effects, adverse reactions or misuse, including potential business impact such as the need to withdraw the product (either voluntarily or as mandated by the FDA), loss of support by the advocacy communities or loss of positive corporate reputation resulting in related unfavorable media coverage in these areas;
- successfully managing third-party service providers involved in the manufacturing and development of pitolisant;
- successfully completing the development of pitolisant in other indications by demonstrating safety, tolerability and efficacy profiles that are satisfactory to the FDA;
- obtaining regulatory approvals to market pitolisant for other indications;
- complying with the terms of the license agreement with Bioprojet;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- maintaining, protecting and expanding the portfolio of intellectual property rights, including patents, trade secrets and knowhow; and
- attracting, hiring and retaining qualified personnel.

In our efforts to market WAKIX for the treatment of EDS in adult patients with narcolepsy, our revenue will be dependent, in part, on the size of the markets in the United States, or in other territories where we may seek and obtain regulatory approval, the number of competitors in such markets, the acceptance of the price of the product in those markets and the ability to obtain reimbursement at any price. If the number of our addressable patients is not as large as we estimate or the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products. If we are not able to generate substantial revenue from the sale of approved products, we may never become profitable.

The commercial adoption of WAKIX and any other product candidates we develop will depend on the degree of their market acceptance.

Even with the requisite approvals from the FDA and other regulatory authorities, the commercial adoption of WAKIX for the treatment of EDS in adult patients with narcolepsy, and any other product candidates we may develop, will depend on the degree of their acceptance by physicians, patients, third-party payors and others in the medical community. If WAKIX or any other product candidates we develop do not achieve an adequate level of market acceptance, we may not generate significant product revenues or any profits from operations. The degree of market acceptance of WAKIX or any other product candidates we develop, if approved for commercial sale, will depend on a number of factors, some of which are beyond our control, including:

- the safety and efficacy of the product as demonstrated in clinical trials;

- the perception of physicians, patients, third-party payors and others in the medical community of the relative safety, efficacy, convenience, effect on quality-of-life and cost-effectiveness of the product, compared to those of other available treatments;
- the product's approved labeling, including the description of the product's approved indications, the description of its efficacy, including the endpoints in which it showed an improvement, and the prevalence and severity of any side effects, including any associated limitations or warnings;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to differentiate WAKIX or other approved products from other treatments in the same space;
- the adoption of WAKIX as a first-line therapy for EDS in adult patients with narcolepsy;
- the prevalence and severity of any side effects, including those that may be discovered following approval and commercialization;
- the willingness of the target patient population to try new treatments and of physicians to prescribe these treatments;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- the publicity concerning our products or competing products and treatments;
- product liability litigation alleging injuries relating to our products or similar classes of drugs;
- any post-approval study requirements for our products and the results thereof; and
- sufficient third-party insurance coverage and reimbursement.

Our continuing efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits and risks of WAKIX may require significant resources and may never be successful. The adoption of WAKIX could be limited if physicians prescribe it only as a second line therapy. Physicians may opt to prescribe the products of our competitors for a variety of reasons. For example, WAKIX did not demonstrate non-inferiority to modafinil and, as such, physicians and patients may choose modafinil rather than WAKIX. Furthermore, because the clinical response to WAKIX may take several weeks before addressing EDS symptoms, patients and physicians may choose other fast acting, stimulant and wake promoting agents over WAKIX. If WAKIX fails to achieve an adequate level of acceptance by physicians, patients, third-party payors and others in the medical community, we will not be able to generate sufficient revenue to become or remain profitable.

We cannot guarantee that WAKIX or any other product candidates we may seek to develop will ever be commercially successful, and to the extent they are not commercially successful, such product candidates would incur significant expense with no corresponding revenue. Because we expect the sales of WAKIX to generate substantially all of our revenue for the foreseeable future, the failure of WAKIX to find market acceptance would substantially harm our business and could require us to seek additional financing.

The market opportunity for WAKIX or any future product candidate we develop may be smaller than we estimate.

The potential market opportunity for WAKIX and any future product candidate is difficult to precisely estimate. Our estimates of the potential market opportunity for our product candidates include

several key assumptions of the current market size and current pricing for commercially available products and are based on industry and market data obtained from industry publications, studies conducted by us, our industry knowledge, third-party research reports and other surveys. While we believe our estimates are reasonable and reliable, they may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of diseases and disorders. The number of patients may turn out to be lower than expected. Additionally, the potentially addressable patient population for WAKIX or any future product candidate we develop may be limited or may not be amenable to treatment with WAKIX or such future product candidate, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business.

We rely on our license agreement with Bioprojet to provide rights to the core intellectual property relating to pitolisant, and any termination or loss of significant rights under the agreement would adversely affect our development and/or commercialization of pitolisant.

We have licensed our core intellectual property relating to pitolisant from Bioprojet. If, for any reason, our license and commercialization agreement with Bioprojet is terminated or we otherwise lose those rights, it would materially adversely affect our business. Pursuant to our license and commercialization agreement, we obtained intellectual property rights in connection with the commercialization of pitolisant in the United States and its territories, commonwealths and protectorates, including Puerto Rico, which includes an exclusive license to use certain intellectual property owned by Bioprojet related to clinically developing and commercializing the pitolisant product candidate for narcolepsy, obstructive sleep apnea, idiopathic hypersomnia and Parkinson's Disease. Under the license agreement, Bioprojet is responsible for conducting all preclinical studies and clinical trials necessary for achieving and maintaining regulatory approval in the United States for narcolepsy and cataplexy indications, including all costs and expenses. We are responsible for all other costs associated with other development and regulatory activities, unless Bioprojet otherwise agrees to participate in funding such activities. We must obtain consent from Bioprojet before commencing any clinical trials related to pitolisant. Our ability to pursue indications other than the ones specifically enumerated in the license agreement is also contingent on mutual agreement of Bioprojet and us as to those indications and such agreement may be withheld at Bioprojet's discretion. If Bioprojet denies consent for us to conduct clinical trials or pursue any such other indication for any reason, we will not have the right under our license and commercialization agreement to commercialize our product for such indication. In such event, Bioprojet may pursue commercialization of such indication for itself in our territory, or it may license the right to commercialize such indication in our territory to third parties, including our competitors.

Our license and commercialization agreement also imposes on us obligations relating to exclusivity, territorial rights, development, commercialization, funding, payment, diligence, sublicensing, insurance, intellectual property protection and other matters. If we breach any material obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages to Bioprojet, and Bioprojet may have the right to terminate our license, which would result in us being unable to develop, manufacture and sell pitolisant and would materially adversely affect our business. See "Business—Strategic Agreement—License and Commercialization Agreement with Bioprojet" for further information.

We may not be successful in our efforts to identify, in-license or acquire, discover, develop or commercialize additional product candidates, or identify other indications for pitolisant beyond EDS in adult patients with narcolepsy.

Although a substantial amount of our effort will focus on the commercialization of WAKIX for the treatment of EDS in adult patients with narcolepsy, we also may seek to identify, in-license or acquire,

discover, develop and commercialize additional product candidates in the rare neurological disorders field, and to identify other indications for pitolisant beyond EDS in adult patients with narcolepsy. We cannot assure you that our efforts to do so will be successful. Even if we are successful at in-licensing or acquiring additional product candidates, their requisite development activities may require substantial resources, and we cannot assure you that these development activities will result in regulatory approvals. We also cannot assure you that our efforts to develop and commercialize pitolisant for other indications beyond EDS in adult patients with narcolepsy will be successful.

Our business, products or product pricing could be subject to negative publicity, which could have a material adverse effect on our reputation, business, financial position, results of operations, liquidity and cash flows.

In recent years, the pharmaceutical industry has been the subject of public complaints and significant publicity regarding the pricing of pharmaceutical products, including publicity and pressure resulting from prices charged by competitors and peer companies for new products as well as price increases by competitors and peer companies on older products that the public has deemed excessive. We may experience downward pricing pressure on the price of WAKIX and any other future approved products due to social or political pressure to lower the cost of drugs, which could reduce our revenue and future profitability. Orphan drugs in particular have received recent negative publicity for the perceived high prices charged for them by their manufacturers, and as a result orphan drug developers such as us may be negatively impacted by such publicity and any U.S. or other government regulatory response. Due to these factors, we may suffer public criticism and negative publicity in media coverage, by industry trade associations and legislators.

Any of the events or developments described above could result in reputational harm and reduced market acceptance and demand for our products, could harm our ability to market our products in the future, could cause us to incur significant expense, could cause our senior management to be distracted from execution of our business strategy, and could have a material adverse effect on our business, reputation, financial condition, results of operations, liquidity, cash flows and/or share price.

Third-party relationships are important to our business. If we are unable to enter into and maintain strategic collaborations or if these relationships are not successful, our business could be adversely affected.

We have limited capabilities for product development and do not yet have any capability for manufacturing or distribution. In addition, we may enter into collaborations for the development and commercialization of certain of our product candidates. If we enter into such collaborations, we will have limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on any future collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms. Relationships we enter into may pose a number of risks, including the following:

- current or future third parties have, and future third-party collaborators may have, significant discretion in determining the efforts and resources that they will apply;
- third parties may not perform their obligations as expected;
- third parties may not pursue development and commercialization of any product candidates that we decide to develop as drugs and that achieve regulatory approval or may elect not to

continue or renew development or commercialization programs based on clinical study or trial results, changes in the third parties' strategic focus or available funding, or external factors, such as a strategic transaction that may divert resources or create competing priorities;

- third parties may delay preclinical studies or clinical trials, provide insufficient funding for a preclinical study or clinical trial, stop a preclinical study or clinical trial or abandon one of our product candidates, repeat or conduct clinical studies or new clinical trials or require a new formulation of a product candidate for clinical testing;
- third parties could independently develop, or develop with other third parties, products that compete directly or indirectly with our products and product candidates if the third parties believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our current or future collaborators as competitive with their own product candidates or products, which may cause such third parties to cease to devote resources to the commercialization of our product candidates;
- third parties may fail to comply with applicable regulatory requirements regarding the development, manufacture, packaging, labeling, holding, distribution and/or marketing of a product candidate or product;
- third parties with marketing and distribution rights to pitolisant or any future product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with third parties, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of pitolisant or any future product candidates, might lead to additional responsibilities for us with respect to pitolisant or any future product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- third parties may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- third parties may infringe the intellectual property rights of other third parties, which may expose us to litigation and potential liability;
- if one of our third parties is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any product candidate licensed to it by us; and
- relationships may be terminated by the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If our relationships do not result in the successful discovery, development and commercialization of products or if a third party terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under any third party agreements we enter into, our development of pitolisant or any future product candidates could be delayed and we may need additional resources. Additionally, if any third party terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

Relationships are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of future collaborators. We face significant competition in seeking appropriate collaborators. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. If we are unable to reach agreements with suitable third parties on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms, or at all. If we fail to enter into relationships or do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates, bring them to market and generate revenue from sales of drugs or continue to develop our technology, and our business may be materially and adversely affected.

We expect to rely on third parties to conduct our clinical trials for pitolisant and any future product candidate that we decide to develop. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval of or commercialize any potential product candidates on a timely basis or at all.

We will continue to rely upon third parties, including independent investigators, to conduct preclinical studies or clinical trials under agreements with universities, medical institutions, contract research organizations, or CROs, strategic partners and others. We expect to have to negotiate budgets and contracts with CROs and study or trial sites, which may result in delays to our development timelines and increased costs.

We will have to rely heavily on third parties over the course of our preclinical studies and clinical trials and, as a result, will have limited control over the clinical investigators and limited visibility into their day-to-day activities, including with respect to their compliance with the approved clinical protocol and regulatory requirements. Nevertheless, we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with Good Clinical Practice, or GCP, requirements for clinical trials, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCP requirements through periodic inspections of study or trial sponsors, clinical investigators and trial sites. If we or any of these third parties fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to suspend or terminate these clinical trials or perform additional clinical trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the GCP or other applicable requirements. In addition, our clinical trials must be conducted with drug products produced under current Good Manufacturing Practices, or cGMP, requirements and may require a large number of patients. Our failure or any failure by these third parties to comply with these regulations, which would delay the regulatory approval or commercialization process. Moreover, our business may be implicated if any of these third parties

violates federal or state laws or regulations including fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Any parties conducting our future clinical trials, if any, generally will not be our employees and, except for remedies that may be available to us under our agreements with the third parties conducting such clinical trials, if any, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our current and future product candidates. As a result, our financial results and the commercial prospects for our current and future product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

If any of our relationships with these third-party CROs or others terminate, we may not be able to enter into contractual and other arrangements with alternative CROs or other third parties in a timely manner to meet projected clinical development deadlines or to do so on commercially reasonable terms. Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO begins work. As a result, delays may occur, which can materially affect our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

If we experience delays in meeting or fail to meet the regulatory requirements for commercialization of our current or future potential product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenues will be materially impaired.

We rely completely on third parties to manufacture and distribute our supply of WAKIX, including certain sole-source suppliers and manufacturers, and intend to rely on third parties to manufacture and distribute any future product candidates.

We do not currently have, nor do we plan to acquire, the infrastructure or capability to manufacture or distribute commercial quantities of WAKIX. Our ability to commercially supply WAKIX depends, in part, on the ability of third-party manufacturers to supply and manufacture the raw materials, active pharmaceutical ingredient, or API, and other important components related to the manufacture of WAKIX. We also rely on third parties to package the finished product. These third-party manufacturers have limited experience manufacturing the raw materials and API for WAKIX to be supplied to patients in the United States. Prior to the approval of WAKIX, we experienced minor issues related to product specifications and other minor delays in supply related to our third-party suppliers and manufacturers. While we continue to work with our third-party suppliers and manufacturers to optimize the manufacturing process for WAKIX and will work to optimize the manufacturing process for any future product candidates, we cannot guarantee that even minor changes in the process will result in products that are safe and, where applicable, effective. If we fail to develop and maintain supply relationships with these third parties, we may be unable to continue to successfully commercialize WAKIX.

We rely and will continue to rely on certain third parties as the sole source of the materials they supply or the finished products they manufacture. For example, we rely on Interor S.A., Corden Pharma Chenôve SAS and Patheon UK Limited to provide intermediate supply ingredients, API and finished products, respectively. Additionally, we rely on our suppliers and manufacturers to purchase materials from other third parties. Any of our existing suppliers or manufacturers may:

- fail to supply us with product on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities or equipment or otherwise;
- fail to increase manufacturing capacity and produce drug product and components in larger quantities and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our commercial needs;
- be unable to meet our production demands due to issues related to their reliance on sole-source suppliers and manufacturers;
- supply us with product that fails to meet regulatory requirements;
- become unavailable through business interruption or financial insolvency;
- lose regulatory status as an approved source;
- be unable or unwilling to (i) honor current supply agreements or (ii) renew current supply agreements when such agreements expire on a timely basis, on acceptable terms or at all; or
- discontinue production or manufacturing of necessary drug substances or products.

In the event of any of the foregoing, if we do not have an alternative supplier or manufacturer in place, we would be required to expend substantial management time and expense to identify, qualify and transfer technical processes to alternative suppliers or manufacturers. Transferring technology to other sites may require additional processes, technologies and validation studies, which are costly, may take considerable amounts of time, may not be successful and, in most cases, require review and approval by the FDA. Any need to find and qualify new suppliers or manufacturers could significantly delay production of WAKIX, adversely impact our ability to market WAKIX and adversely affect our business. There can be no assurance that replacements would be available to us on a timely basis, on acceptable terms or at all. Additionally, we and our manufacturers do not currently maintain significant inventory of drug substances and other materials beyond our currently forecasted needs. Any interruption in the supply of a drug substance or other material or in the manufacture of WAKIX could have a material adverse effect on our business, financial condition, operating results and prospects.

Additionally, although we are ultimately responsible for ensuring compliance with regulatory requirements such as cGMPs, we are dependent on our contract suppliers and manufacturers for day-to-day compliance with cGMP for production of both drug substances and finished products. Facilities used by our contract suppliers and manufacturers to produce the drug substances and materials or finished products for commercial sale must pass inspection and be approved by the FDA and other relevant regulatory authorities. A number of our contract suppliers and manufacturers must comply with cGMP requirements enforced by the FDA through its facilities inspection program and review of submitted technical information. If the safety of WAKIX is compromised due to a failure to adhere to applicable laws or for other reasons, we may not be able to successfully commercialize our product and we may be held liable for injuries sustained as a result. In addition, the manufacturing facilities of certain of our suppliers are located outside of the United States. This may give rise to difficulties in importing our product into the United States or other countries as a result of, among other things, regulatory agency approval requirements, taxes, tariffs, local import requirements such as import duties or inspections, incomplete or inaccurate import documentation or defective packaging. Any of these factors could adversely impact our ability to effectively commercialize WAKIX.

Because a number of companies compete with us, many of which have greater resources than we do, and because we face rapid changes in science in our industry, we cannot be certain that our products will be accepted in the marketplace or capture market share.

Competition from other biotechnology and pharmaceutical companies is intense and is expected to increase. There may be a number of companies pursuing the development of pharmaceuticals in rare neurological disorders, our area of focus. These companies may be very large, and may have financial, technical, sales and distribution and other resources substantially greater than ours. The greater resources of these competitors may enable them to develop, obtain regulatory approval for or market competing products more quickly or effectively, making it extremely difficult for us to capture a share of the market for our product. We also face competition, and may in the future face additional competition, from manufacturers of generic drugs. Certain U.S. state laws allow for, and in some instances in the absence of specific instructions from the prescribing physician mandate, the dispensing of generic products rather than branded products when a generic version is available. Generic competition often results in decreases in the prices at which branded products can be sold. The commercial potential of our current products and any future products may be reduced or eliminated if our competitors develop or acquire and commercialize generic or branded products that are safer or more effective, have fewer side effects, are easier to administer or are less expensive than our products. We also face competition from off-label uses of approved drugs. Additionally, the biotechnology and pharmaceutical industries are subject to rapid changes in science, and our competitors may develop and market products with improved therapeutic profiles relative to pitolisant or any future product candidates that would render pitolisant or any future product candidates noncompetitive.

We may need to increase the size and capabilities of our organization based on business need, and we may experience difficulties in managing our growth.

We commenced operations in 2017 and, as of December 31, 2019, had approximately 150 employees. As we advance the development of pitolisant in other indications and commercialize WAKIX as a treatment for EDS in adult patients with narcolepsy, we must continue to grow the size of the organization. Future growth will impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, retaining and motivating additional employees;
- effectively managing our development efforts, including the clinical development and FDA or other regulatory authority review processes for pitolisant or any future product candidates;
- effectively managing any third-party service providers involved in the development and manufacture of pitolisant or any future product candidates; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to successfully develop and commercialize WAKIX or any future product candidates will depend, in part, on our ability to effectively manage any future growth. Our management will have to dedicate a significant amount of its attention to managing these growth activities. In addition, we expect to incur additional costs in hiring, training and retaining such additional personnel.

If we are not able to effectively expand our organization, we may not be able to successfully execute the tasks necessary to further develop and commercialize pitolisant or any future product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Our future success depends on our ability to retain our key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the principal members of our management and scientific teams. We do not maintain “key person” insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

To encourage valuable employees to remain at our company, in addition to salary and cash incentives, we have provided equity award grants that vest over time. The value to employees of these equity grants that vest over time may be significantly affected by changes in the price of our common stock that are beyond our control, and may at any time be insufficient to retain employees who receive more lucrative offers from other companies. Any of our employees could leave our employment at any time, with or without notice.

Recruiting and retaining qualified operations, finance and accounting, quality and compliance, scientific, clinical, manufacturing and sales and marketing personnel or consultants will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms, given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. If we are unable to attract, retain and motivate qualified and experienced personnel, it could harm our business, results of operations and financial condition. Even if we are successful in attracting and retaining such personnel, competition for such employees may significantly increase our compensation costs and adversely affect our business, results of operations and financial condition.

The loss of the services of any of our executive officers, key employees or consultants could seriously harm our ability to successfully implement our business strategy. Replacing executive officers, key employees or consultants may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel or consultants on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel.

We may hire part-time employees or use consultants. As a result, certain of our employees, officers, directors or consultants may not devote all of their time to our business, and may from time to time serve as employees, officers, directors and consultants of other companies.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics, power shortage, telecommunication failure or other natural or manmade accidents or incidents that result in us being unable to fully utilize our facilities, the manufacturing facilities of our third-party contract manufacturers or our or their distribution networks, may have a material and adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Loss of access to these facilities may result in increased costs, or interruptions in the commercialization of WAKIX or our business operations. Natural disasters could further disrupt our operations, and have a material and adverse effect on our business, financial condition, results of operations and prospects. If a natural disaster,

power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our research facilities, the manufacturing facilities of our third-party contract manufacturers or our or their distribution networks, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure our investors that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities or the manufacturing facilities of our third-party contract manufacturers are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any business interruption may have a material and adverse effect on our business, financial condition, results of operations and prospects.

We depend on our information technology systems, and any failure of these systems could harm our business. Any real or perceived security breaches, loss of data, and other disruptions or incidents could compromise the privacy, security, integrity or confidentiality of sensitive information related to our business or prevent us from accessing critical information and expose us to liability and reputational harm, which could adversely affect our business, results of operations and financial condition.

We collect and maintain data and information that is necessary to conduct our business, and we are increasingly dependent on information technology systems and infrastructure to operate our business, including systems infrastructure operated and maintained by our third party suppliers or providers. In the ordinary course of our business, we collect, store and transmit large amounts of confidential information, including intellectual property, proprietary business information and personal information. It is critical that we do so in a secure manner to maintain the privacy, security, confidentiality and integrity of such confidential information. We have established physical, electronic and organizational measures to safeguard and secure our systems and facilities to prevent an information compromise, and rely on commercially available systems, software, tools and monitoring to provide security for our information technology systems and the processing, transmission and storage of digital information. We have also outsourced elements of our information technology infrastructure, and as a result, a number of third-party vendors may or could have access to our confidential information. Our internal information technology systems and infrastructure, and those of our current and any future collaborators, contractors and consultants and other third parties on which we rely, are vulnerable to damage or unauthorized access or use resulting from computer viruses, malware, natural disasters, terrorism, war, telecommunication and electrical failures, denial-of-service attacks, cyber-attacks or cyber-intrusions over the Internet, hacking, phishing and other social engineering attacks, attachments to emails, persons inside our organization (including employees or contractors), lost or stolen devices, or persons with access to systems inside our organization.

The risk of a security breach or disruption or data loss, particularly through social engineering attacks, cyber-attacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. In addition, the prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information or other intellectual property. The costs to us to mitigate, investigate and respond to potential security incidents, breaches, disruptions, network security

problems, bugs, viruses, worms, malicious software programs and security vulnerabilities could be significant, and while we have implemented security measures to protect our data security and information technology systems, our efforts to address these problems may not be successful, and these problems could result in unexpected interruptions, delays, cessation of service and other harm to our business and our competitive position. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product development programs. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Moreover, if a real or perceived security breach affects our systems (or those of our third party providers or suppliers) or results in the loss of or accidental, unlawful or unauthorized access to, use of, release of or other processing of personally identifiable information or clinical trial data, our reputation could be materially damaged. In addition, such a breach may require notification to governmental agencies, the media or individuals pursuant to various federal and state privacy and security laws, if applicable, including the Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Clinical Health Act of 2009, or HITECH, and its implementing rules and regulations, as well as regulations promulgated by the Federal Trade Commission and state breach notification laws. We would also be exposed to a risk of loss, negative publicity, harm to our reputation, governmental investigation and/or enforcement actions, claims or litigation and potential liability, which could materially adversely affect our business, results of operations and financial condition. The global data protection landscape is rapidly evolving, and we may be affected by or subject to new, amended or existing laws and regulations in the future, including as our operations continue to expand or if we begin to operate in foreign jurisdictions.

Our employees and independent contractors, including principal investigators, consultants, commercial collaborators, service providers and other vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have an adverse effect on our results of operations.

We are exposed to the risk that our employees and independent contractors, including principal investigators, consultants, any future commercial collaborators, service providers and other vendors may engage in misconduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or other unauthorized activities that violate the laws and regulations of the FDA and other similar regulatory bodies, including those laws that require the reporting of true, complete and accurate information to such regulatory bodies; manufacturing standards; U.S. federal and state healthcare fraud and abuse laws, data privacy and security laws and other similar non-U.S. laws; or laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs, and other business arrangements. Such misconduct also could involve the improper use or misrepresentation of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, the creation of fraudulent data in our preclinical studies or clinical trials, or illegal misappropriation of product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third-parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. In addition, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and financial results, including, without limitation, the imposition of

significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, possible exclusion from participation in Medicare, Medicaid and other U.S. federal healthcare programs or healthcare programs in other jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, imprisonment, other sanctions, contractual damages, reputational harm, diminished profits and future earnings and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Risks Related to Development, Regulatory Approval and Commercialization

The regulatory approval process of the FDA is costly, lengthy and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for pitolisant in other potential indications for which we may seek to develop pitolisant, our business will be substantially harmed.

Although the commercialization of WAKIX is our primary focus, as part of our longer-term growth strategy, we plan to evaluate pitolisant in other indications and develop other product candidates. The research, testing, manufacturing, labeling, approval, selling, import, export, pricing and reimbursement marketing and distribution of drug products are subject to extensive regulation by the FDA and other regulatory agencies in the United States. Although we have obtained regulatory approval for WAKIX in the United States for the treatment of EDS in adults with narcolepsy, it is possible that we may not obtain regulatory approval for pitolisant for other indications, including for the treatment of cataplexy, for which we may seek such approval, or for any other product candidates we may seek to develop in the future. We received a Complete Response Letter, or CRL, for pitolisant for the treatment of cataplexy in adult patients with narcolepsy. Obtaining regulatory approval of an NDA can be a lengthy, expensive and uncertain process.

The FDA can delay, limit or deny approval of a drug candidate for many reasons or require us to conduct additional preclinical or clinical testing, including, but not limited to, the following:

- a drug candidate may not be deemed safe or effective, or the clinical and other benefits may be deemed to not outweigh the candidate's risks;
- the FDA might not approve our trial design and analysis plan;
- the FDA may not find the data from nonclinical and clinical studies and trials sufficient or may disagree with our interpretation of data from nonclinical or clinical studies;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates, or other products containing the active ingredient in our product candidates;
- clinical inspection(s) by the FDA or other regulatory authorities may result in unacceptable findings that could negatively impact approval of pitolisant;
- the FDA might not accept or deem acceptable a third-party manufacturers' processes or facilities; or
- the FDA may change its approval policies or adopt new regulations.

Prior to obtaining approval to commercialize a drug candidate in the United States, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA, that such drug candidates are safe and effective for their intended uses. The number of nonclinical and clinical studies and trials that will be required for FDA approval varies depending on the drug candidate, the disease or condition that the drug candidate is designed to address, and the regulations applicable to any particular drug candidate. In addition, data obtained from preclinical trials and clinical trials are susceptible to varying interpretations, and regulatory

authorities may not interpret our data as favorably as we do, which may further delay, limit or prevent development efforts, clinical trials or marketing approval. Furthermore, as more competing drug candidates within a particular class of drugs proceed through clinical development to regulatory review and approval, the amount and type of clinical data that may be required by regulatory authorities may increase or change. Even if we believe the preclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. If pitolisant fails to demonstrate safety and efficacy in clinical trials or does not gain regulatory approval for other indications, our business and results of operations will be materially and adversely harmed. Additionally, if the FDA requires that we conduct additional clinical trials, places limitations on pitolisant in our label, delays approval to market pitolisant or limits the use of pitolisant, our business and results of operations may be harmed.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

If we fail to obtain and sustain an adequate level of coverage and reimbursement for WAKIX and other product candidates by third-party payors, sales would be adversely affected.

Successful sales of WAKIX and any other product candidates that may receive regulatory approval depend on the availability of coverage and adequate reimbursement from third-party payors. Patients who are prescribed medications for the treatment of their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Regulatory approvals, pricing and reimbursement for new drug products vary widely from country to country. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or HHS. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. Commercial third-party payors, such as private health insurers and health maintenance organizations, also decide which medications they will pay for and establish reimbursement levels, though commercial third-party payors often follow CMS' reimbursement determinations. The availability of coverage and the extent of reimbursement by governmental and private payors is essential for most patients to be able to afford treatments. Sales of WAKIX or other product candidates that we may identify will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors.

Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;

- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

We cannot be sure that reimbursement will be available for WAKIX and, if coverage and reimbursement are available, what the level of reimbursement will be. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition. If coverage and adequate reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

Obtaining coverage and reimbursement approval for a product from a government or other third-party payor can be an expensive and time-consuming process that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to the payor. The industry competition to be included in third-party payors' drug formularies, or lists of medications for which third-party payors provide coverage and reimbursement, often leads to downward pricing pressures on pharmaceutical products. In addition, third-party payors may refuse to include a particular branded drug in their formularies or otherwise restrict patient access through formulary controls or otherwise to a branded drug when a less costly generic equivalent or other alternative is available. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors, and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average manufacture price and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, the level of reimbursement.

In addition, there may be significant delays in obtaining such coverage and reimbursement for newly approved products, and coverage may be more limited than the purposes for which the product is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that a product will be paid for in all cases or at a rate that covers our costs, including research, development, intellectual property, manufacture, sale and distribution expenses.

Interim reimbursement levels for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the product and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost products and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors, by any future laws limiting drug prices and by any future relaxation of laws that presently restrict imports of product from countries where they may be sold at lower prices than in the United States.

Assuming we obtain coverage for WAKIX by a third-party payor, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high.

Patients are unlikely to use WAKIX unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of WAKIX. Therefore, coverage and adequate reimbursement is critical to new product acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new products when more established or lower cost therapeutic alternatives are already available or subsequently become available. We may suffer loss of corporate reputation due to industry-wide legislative or public scrutiny of our pricing decisions and practices within an increasingly price-sensitive environment.

Even if we do obtain formulary approval, we expect to experience pricing pressures in connection with the sale of WAKIX due to the trend toward cost containment, managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. Large public and private payors, managed care organizations, group purchasing organizations and similar organizations are exerting increasing influence on decisions regarding the use of, and reimbursement levels for, particular treatments. Such third-party payors, including Medicare, are questioning the coverage of, and challenging the prices charged for medical products and services, and many third-party payors limit coverage of, or reimbursement for, newly approved health care products. The downward pressure on healthcare costs in general, particularly prescription medicines, medical devices and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the successful commercialization of new products. Further, the adoption and implementation of any future governmental cost containment or other health reform initiative may result in additional downward pressure on the price that we may receive for WAKIX.

These cost-control initiatives could decrease the price we might establish for WAKIX, which could result in product revenues being lower than anticipated. The pricing, coverage and reimbursement of WAKIX must be adequate to support a commercial infrastructure. If the price for WAKIX decreases or if governmental and other third-party payors do not provide adequate coverage and reimbursement levels, our revenue, gross margins and prospects for profitability will suffer.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and other countries will likely put pressure on the pricing and usage of medical products. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for WAKIX. Accordingly, in markets outside the United States, the reimbursement for WAKIX may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

WAKIX has been approved by the FDA for the treatment of EDS in adult patients with narcolepsy. Regulatory approval is limited by the FDA to the specific indication for which approval has been granted and, unless we seek regulatory approval for additional indications, we will be prohibited from marketing pitolisant for other indications. We may be subject to fines, penalties or injunctions if we are determined to have promoted or be promoting the use of pitolisant for unapproved or "off-label" uses, resulting in damage to our reputation and business.

While we received approval for the indication of the treatment of EDS in adult patients with narcolepsy, WAKIX is not indicated for the treatment of cataplexy in adult patients with narcolepsy. We therefore are prohibited from promoting WAKIX for the treatment of cataplexy in narcolepsy unless we are granted FDA approval for such indication. The FDA strictly regulates the promotional claims that may be made about prescription products, and WAKIX may not be promoted for uses that are not

approved by the FDA as reflected in its approved labeling. If we are not able to obtain FDA approval for any desired future indications for our products and product candidates, our ability to effectively market and sell our products may be reduced and our business may be adversely affected.

While physicians may choose to prescribe products for uses that are not described in the product's labeling and for uses that differ from those tested in clinical trials and approved by the regulatory authorities, we are prohibited from marketing and promoting the products for indications that are not specifically approved by the FDA. These "off-label" uses are common across medical specialties and may constitute an appropriate treatment for some patients in varied circumstances. Regulatory authorities in the United States generally do not restrict or regulate the behavior of physicians in their choice of treatment within the practice of medicine. Regulatory authorities do, however, restrict communications by biotechnology or pharmaceutical companies on off-label use. If the FDA determines that our promotional activities constitute promotion of an off-label use, it could request that we modify our promotional materials and subject us to FDA regulatory or enforcement actions as well as actions by other agencies, including issuance of warning letters or untitled letters, suspension or withdrawal of an approved product from the market, mandatory or voluntary recalls, civil fines, disgorgement of money, operating restrictions, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement, injunctions or criminal prosecution, any of which could significantly harm our business.

WAKIX or any of our future product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, reduce the commercial attractiveness of a prescribing label or result in significant negative consequences following regulatory approval, if approved.

Clinical trials of WAKIX or other product candidates we may develop could reveal a high and unacceptable incidence and severity of undesirable side effects. Undesirable side effects could adversely affect patient enrollment in clinical studies, cause us or regulatory authorities to interrupt, delay or halt clinical studies or result in the delay, denial or withdrawal of regulatory approval by the FDA or other regulatory authorities. Undesirable or adverse side effects also could result in regulatory authorities mandating a more restrictive prescribing label for the product, which, in turn, could limit the market acceptance of the product even if approved for marketing and commercialization.

Drug-related side effects could result in potential product liability claims. We believe our product liability insurance coverage is sufficient in light of our clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts or maintain coverage at all to protect us against losses due to liability. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations, business and financial condition. In addition, regardless of merit or eventual outcome, product liability claims may result in impairment of our business reputation, significant negative media attention, withdrawal of clinical study participants, costs due to related litigation, distraction of management's attention from our primary business, initiation of investigations by regulators, substantial monetary awards to patients or other claimants, the inability to commercialize our current product candidate or any future product candidate, product recalls, restrictions on labeling, marketing or promotion, decreased demand for our product candidates, if approved for marketing, and loss of revenue.

Additionally, if we or others later identify undesirable side effects caused by WAKIX, either in the field or in clinical trials in other potential indications for which we develop pitolisant, or in clinical trials for other product candidates, a number of potentially significant negative consequences could result, including but not limited to:

- the delay, prevention or withdrawal of approvals by regulatory authorities;

- the requirement of additional warnings on the prescribing label;
- the requirement of a Risk Evaluation and Mitigation Strategy, or REMS, plan, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers and/or other elements to assure safe use;
- designation as a controlled substance by the U.S. Drug Enforcement Administration, or DEA;
- litigation and the potential to be held liable for harm caused to patients; and
- an adverse effect on our reputation.

Any of these events could prevent us from achieving or maintaining market acceptance of pitolisant and could significantly harm our business, results of operations, financial condition and prospects.

We have never commercialized a product candidate prior to WAKIX and we may lack the necessary expertise, personnel and resources to successfully commercialize WAKIX or any other potential product candidates that receive regulatory approval on our own or together with collaborators.

WAKIX is our first commercialized product. Prior to this, our operations had been limited to organizing and staffing our company, business planning, raising capital, acquiring the rights to our product candidates and undertaking preclinical studies and clinical trials of our product candidates. We currently have no in-house manufacturing, distribution or supply capabilities. To achieve commercial success of WAKIX or any other product candidate, if approved, we will have to develop our own manufacturing, distribution and supply capabilities or outsource these activities to a third party.

We are early in our commercialization efforts. Factors that may affect our ability to commercialize our product candidates on our own include recruiting and retaining adequate numbers of effective sales and marketing personnel, obtaining access to or persuading adequate numbers of physicians to prescribe our product candidates and other unforeseen costs associated with creating an independent sales and marketing organization. Developing a sales and marketing organization requires significant investment, is time-consuming and could delay the launch of our product candidates. We may not be able to build an effective sales and marketing organization in the United States or other key global markets. If we are unable to build our own distribution and marketing capabilities or to find suitable partners for the commercialization of our product candidates, we may have difficulties generating revenue from them.

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products appropriate periods of exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.

Once an NDA is approved, the product covered thereby becomes a “reference listed drug” in the FDA’s publication, “Approved Drug Products with Therapeutic Equivalence Evaluations,” commonly known as the Orange Book. Manufacturers may seek approval of generic versions of reference listed drugs through submission of abbreviated new drug applications, or ANDAs, in the United States. In support of an ANDA, a generic manufacturer need not conduct clinical trials. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labelling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug is typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The United States Federal Food, Drug, and Cosmetic Act, or FDCA, provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity, or NCE. Specifically, in cases where such exclusivity has been granted, an ANDA may not be submitted to the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference listed drug.

While we have received five years of NCE exclusivity for WAKIX, manufacturers may seek to launch generic products following the expiration of the applicable exclusivity period we obtain, even if we still have patent protection for our product.

Competition that our products may face from generic versions of our products could materially and adversely affect our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

We may incur unexpected costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

To obtain the requisite regulatory approvals to commercialize any of our product candidates, we must demonstrate through extensive preclinical studies and clinical trials that our product candidates are safe and effective in humans. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process and our future clinical trial results may not be successful.

We may experience delays in completing our clinical trials or preclinical studies and initiating or completing additional clinical trials. We may also experience numerous unforeseen events during our clinical trials that could delay or prevent our ability to receive marketing approval or commercialize the product candidates we develop, including:

- regulators, institutional review boards, or IRBs, or other reviewing bodies may not authorize us or our investigators to commence a clinical trial, or to conduct or continue a clinical trial at a prospective or specific trial site;
- we may not reach agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the number of subjects or patients required for clinical trials of pitolisant in additional indications or any other product candidate may be larger than we anticipate, enrollment in these clinical trials may be insufficient or slower than we anticipate, and the number of clinical trials being conducted at any given time may be high and result in fewer available patients for any given clinical trial, or patients may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors, including those manufacturing our product candidates or conducting clinical trials on our behalf, may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we may have to amend clinical trial protocols submitted to regulatory authorities or conduct additional studies to reflect changes in regulatory requirements or guidance, which we may be required to resubmit to an IRB and regulatory authorities for re-examination;
- regulators, IRBs or other reviewing bodies may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we enter into

agreement for clinical and commercial supplies, or the supply or quality of pitolisant or any other product candidate or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and

- the potential for approval policies or regulations of the FDA or the applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Regulators, IRBs of the institutions in which clinical trials are being conducted or data monitoring committees may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Negative or inconclusive results from our ongoing clinical trials of pitolisant for the treatment of narcolepsy, or any other clinical trial or preclinical studies in animals that we conduct, could mandate repeated or additional clinical trials and could result in changes to or delays in clinical trials in other indications. We do not know whether any clinical trials that we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market pitolisant for our initial or potential additional indications, or any other product candidate. If later stage clinical trials do not produce favorable results, our ability to obtain regulatory approval for pitolisant for initial or potential additional indications, or any other product candidate, may be adversely impacted.

Our failure to successfully initiate and complete clinical trials of pitolisant for potential additional indications or any other product candidate and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market pitolisant or any other product candidate would significantly harm our business. Our product candidate development costs will also increase if we experience delays in testing or regulatory approvals and we may be required to obtain additional funds to complete clinical trials. We cannot assure you that our clinical trials will begin as planned or be completed on schedule, if at all, or that we will not need to restructure our trials after they have begun. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates, which may harm our business and results of operations. In addition, many of the factors that cause, or lead to, delays of clinical trials may ultimately lead to the denial of regulatory approval of pitolisant or any other product candidate.

In addition, prior to our acquisition of the rights to pitolisant, we had no involvement with or control over the nonclinical or clinical development of pitolisant. Additionally, pursuant to our collaboration agreement with Bioprojet, we will rely on data generated by Bioprojet in connection with seeking regulatory approval of pitolisant in the territories in which we have rights to develop and commercialize pitolisant. We are dependent on Bioprojet having conducted such research and development in accordance with the applicable protocols and legal, regulatory and scientific standards, having accurately reported the results of all clinical trials and other research they conducted prior to our acquisition of the rights to pitolisant, having correctly collected and interpreted the data from these trials and other research, and having supplied us with complete information, data sets and reports required to adequately demonstrate the results reported through the date of our acquisition of these assets. Problems related to predecessors could result in increased costs and delays in the development of pitolisant for additional indications, which could adversely affect our ability to generate any future revenue from sales of pitolisant, if approved for additional indications.

Interim, "topline" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose interim, "topline" or preliminary data from our clinical trials, which is based on a preliminary analysis of then-available topline data, and the results and related findings and conclusions are subject to change following completion of the study or a full analyses of all data related to the particular trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the interim, "topline" or preliminary results that we report may differ from future results of the same trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. "Topline" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, "topline" data should be viewed with caution until the final data are available. We may also disclose interim data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our business in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular drug, product candidate or our business. If the interim, "topline" or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for and commercialize our product candidates, our business, operating results, prospects or financial condition may be harmed.

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials on our current timelines, or at all, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. Enrollment in our clinical trials may be slower than we anticipate, leading to delays in our development timelines. Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the nature of the trial protocol, our ability to recruit clinical trial investigators with the appropriate competencies and experience, the existing body of safety and efficacy data with respect to the study drug, the number and nature of competing treatments and ongoing clinical trials of competing drugs for the same indication, the proximity of patients to clinical sites, the eligibility criteria for the trial and the proportion of patients screened that meets those criteria, our ability to obtain and maintain patient consents, and the risk that patients enrolled in clinical trials will drop out of the trials before completion.

Furthermore, any negative results or new safety signals we or third parties may report in clinical trials of our product candidates may make it difficult or impossible to recruit and retain patients in our

clinical trials. Similarly, negative results reported by our competitors about their drug candidates may negatively affect patient recruitment in our clinical trials. In addition, marketing authorization of competitors in this same class of drugs may impair our ability to enroll patients into our clinical trials, delaying or potentially preventing us from completing recruitment of one or more of our trials. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop pitolisant or any future product candidates, or could render further development impossible. In addition, we expect to rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials, and, while we intend to enter into agreements governing their services, we will be limited in our ability to compel their actual performance.

Even though the FDA granted orphan drug designation to pitolisant for the treatment of narcolepsy, we may not be able to obtain or maintain orphan drug marketing exclusivity for this product candidate or any other product candidates.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. Pitolisant was granted orphan drug designation for the treatment of narcolepsy in 2010. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug for the same indication for that time period. Under the FDA's regulations, the FDA will deny orphan drug exclusivity to a designated drug upon approval if the FDA has already approved another drug with the same active ingredient for the same indication, unless the drug is demonstrated to be clinically superior to the previously approved drug. The applicable exclusivity period is seven years in the United States. Orphan drug exclusivity in the United States may be unavailable where the indication for which the product candidate is approved is broader than the orphan-designated indication, or is otherwise different from the orphan-designated indication. For example, the FDA granted orphan drug designation for pitolisant for the treatment of narcolepsy. This means that pitolisant for the treatment of cataplexy in adult patients with narcolepsy may not be covered by the scope of any orphan drug exclusivity that we may obtain in the future. Even if we obtain orphan drug exclusivity for a drug candidate, that exclusivity may not effectively protect the candidate from competition. WAKIX may face additional competition because different drugs with a different active moiety can still be approved for the same condition. Even after an approved drug is granted orphan exclusivity, exclusivity may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the drug to meet the needs of patients with the rare disease or condition following approval. In addition, the FDA can subsequently approve products with the same active moiety for the same condition if the FDA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. There have been legal challenges to aspects of the FDA's regulations and policies concerning the exclusivity provisions of the Orphan Drug Act, and future challenges could lead to changes that affect the protections afforded our product candidates in ways that are difficult to predict.

On August 3, 2017, Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when,

or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

We are subject to ongoing regulatory obligations and continued regulatory review with respect to WAKIX, which will result in significant additional expense. Additionally, WAKIX could be subject to labeling and other restrictions and market withdrawal, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with WAKIX.

WAKIX is subject to extensive and ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, distribution, import, export, record keeping and submission of safety and other post-market information, including both federal and state requirements in the United States. In addition, manufacturers and manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMP. As such, we and our contract manufacturers are subject to continual review and periodic inspections to assess compliance with cGMP. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Our regulatory approval for WAKIX for the treatment of EDS in adult patients with narcolepsy, and any other regulatory approvals we may receive for pitolisant or any future product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, which must comply with applicable GCP regulations. We could also be asked to conduct post marketing clinical studies to verify the safety and efficacy of future product candidates in general or in specific patient subsets. For example, as a part of the regulatory approval for WAKIX for the treatment of EDS in adult patients with narcolepsy, we are required to conduct post-marketing studies in women exposed to pitolisant in pregnancy, including a registry-based observational cohort study to assess maternal, fetal, and infant outcomes of women exposed to pitolisant during pregnancy, and another study of a different design such as a case control study or a retrospective cohort study using electronic medical record data, and a lactation study.

We will also be required to report certain adverse events and production problems, if any, to the FDA, and to comply with requirements concerning advertising and promotion for WAKIX. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote WAKIX for indications or uses for which it does not have FDA approval. The holder of an approved NDA must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling or manufacturing process.

If a regulatory agency discovers previously unknown problems with WAKIX, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing, or labeling of a product, the regulatory agency may impose restrictions on the product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning or untitled letters;
- impose civil or criminal penalties, including product seizures and injunctions;
- limit or suspend regulatory approval;

- suspend any of our ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our contract manufacturers' facilities, on the manufacturing of our products, or on the labeling or marketing of our products; or
- seize or detain products or require a product recall or withdrawal of the products from the market.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenues from WAKIX or future product candidates. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our operating results will be adversely affected. Additionally, if we are unable to generate revenues from the sale of WAKIX or future product candidates, our potential for achieving profitability will be diminished and the capital necessary to fund our operations will be increased.

The regulatory requirements and policies may change, and additional government regulations may be enacted for which we may also be required to comply. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or in other countries. If we or any future collaboration partner are not able to maintain regulatory compliance, we or such collaboration partner, as applicable, may face government enforcement action and our business will suffer.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. The policies of the FDA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, certain policies of the Trump administration may affect our business and industry. Namely, the Trump administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. It is difficult to predict how these executive actions will be implemented, and the extent to which they will affect the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

Non-compliance by us or any future collaborator with regulatory requirements, including safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population can also result in significant financial penalties.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to

broadly applicable federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. The laws that affect our current and future operations include, but are not limited to:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, in exchange for, or to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item, or service for which payment may be made, in whole or in part, under any U.S. federal healthcare programs, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers, among others, on the other. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the U.S. federal civil and criminal false claims laws, such as the False Claims Act, or FCA, which imposes significant penalties and can be enforced by private citizens through civil qui tam actions, and prohibits individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the U.S. federal government, false, fictitious or fraudulent claims for payment of federal funds, and knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. For example, pharmaceutical companies have been prosecuted under the FCA in connection with their alleged off-label promotion of drugs, purportedly concealing price concessions in the pricing information submitted to the government for government price reporting purposes, and allegedly providing free product to customers with the expectation that the customers would bill federal health care programs for the product. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA;
- the federal civil monetary penalties laws, which impose civil fines for, among other things, the offering or transfer of remuneration to a Medicare or state healthcare program beneficiary if the person knows or should know it is likely to influence the beneficiary's selection of a particular provider, practitioner, or supplier of services reimbursable by Medicare or a state healthcare program, unless an exception applies;
- HIPAA, which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended HITECH, and its implementing regulations, which imposes privacy, security and breach reporting obligations, including mandatory contractual terms, with respect to safeguarding the privacy and security of individually identifiable health information upon covered entities subject to the rule, such as health plans, healthcare clearinghouses and healthcare providers and their respective business associates and independent contractors that perform certain services for them that involve the use or disclosure of individually identifiable

health information on their behalf. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions;

- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- federal price reporting laws, which require manufacturers to calculate and report complex pricing metrics to government programs, where such reported prices may be used in the calculation of reimbursement and/or discounts on approved products;
- state law equivalents of each of the above federal laws, such as state anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers, and may be broader in scope than their federal equivalents;
- federal transparency requirements detailing interactions with and payments to healthcare providers, such as the federal reporting requirements under the Physician Payments Sunshine Act, which requires, among other things, certain manufacturers of drugs, devices, biologics and medical supplies reimbursed under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the HHS information related to payments and other transfers of value provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other health care professionals starting January 1, 2022, and teaching hospitals and physician ownership and investment interests, including such ownership and investment interests held by a physician's immediate family members. Failure to submit required information may result in civil monetary penalties;
- state laws that require pharmaceutical companies to implement compliance programs, comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or to track and report gifts, compensation and other remuneration provided to physicians and other health care providers and other potential referral sources, state laws that require drug manufacturers to file reports relating to pricing information and marketing expenditures, state and local laws requiring the registration of pharmaceutical sales representatives; and other state laws and regulations that govern the privacy and security of health information or personally identifiable information in certain circumstances, including state health information privacy and data breach notification laws which govern the collection, use, disclosure and protection of health-related and other personal information, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus requiring additional compliance efforts;
- the U.S. Foreign Corrupt Practices Act of 1977, as amended, which prohibits, among other things, U.S. companies and their employees and agents from authorizing, promising, offering, or providing, directly or indirectly, corrupt or improper payments or anything else of value to foreign government officials, employees of public international organizations and foreign government owned or affiliated entities, candidates for foreign political office, and foreign political parties or officials thereof; and
- similar healthcare and data protection laws in the European Union and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers and laws governing the privacy and security of certain protected information, such as the General Data Protection Regulation, or GDPR.

Ensuring that our business operations and current and future arrangements with third parties comply with applicable healthcare laws and regulations will likely be costly. It is possible that governmental authorities will conclude that our business practices, including, without limitation, our patient support and financial assistance programs, do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that apply to us, we may be subject to penalties, including civil, administrative and criminal penalties, damages, fines, the curtailment or restructuring of our operations, contractual damages, disgorgement, reputational harm, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, the exclusion from participation in federal and state healthcare programs and individual imprisonment, any of which could adversely affect our ability to market pitolisant, if approved, and adversely impact our financial results. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the applicable regulatory agencies or the courts, and their provisions are open to a variety of interpretations.

We face potential liability related to the privacy of health information we obtain from clinical trials sponsored by us.

Most healthcare providers, including research institutions from which we obtain patient health information, are subject to privacy and security regulations promulgated under HIPAA, as amended by the HITECH. We are not currently classified as a covered entity or business associate under HIPAA and thus are not directly subject to its requirements or penalties. However, any person may be prosecuted under HIPAA's criminal provisions either directly or under aiding-and-abetting or conspiracy principles. Consequently, depending on the facts and circumstances, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a HIPAA-covered healthcare provider or research institution that has not satisfied HIPAA's requirements for disclosure of individually identifiable health information. In addition, we may maintain sensitive personally identifiable information, including health information, that we receive throughout the clinical trial process, in the course of our research collaborations, and directly from individuals (or their healthcare providers) who enrol in our patient assistance programs. As such, we may be subject to state laws requiring notification of affected individuals and state regulators in the event of a breach of personal information, which is a broader class of information than the health information protected by HIPAA.

Furthermore, certain health privacy laws, data breach notification laws, consumer protection laws and genetic testing laws may apply directly to our operations and/or those of our collaborators and may impose restrictions on our collection, use and dissemination of individuals' health information. Patients about whom we or our collaborators obtain health information, as well as the providers who share this information with us, may have statutory or contractual rights that limit our ability to use and disclose the information. We may be required to expend significant capital and other resources to ensure ongoing compliance with applicable privacy and data security laws. Claims that we have violated individuals' privacy rights or breached our contractual obligations, even if we are not found liable, could be expensive and time consuming to defend and could result in adverse publicity that could harm our business.

If we or third-party CMOs, CROs or other contractors or consultants fail to comply with applicable federal, state or local regulatory requirements, we could be subject to a range of regulatory actions that could affect our or our contractors' ability to develop and commercialize our product candidates and

could harm or prevent sales of any affected products that we are able to commercialize, or could substantially increase the costs and expenses of developing, commercializing and marketing our products. Any threatened or actual government enforcement action could also generate adverse publicity and require that we devote substantial resources that could otherwise be used in other aspects of our business. Increasing use of social media could give rise to liability, breaches of data security or reputational damage.

Clinical practice guidelines and recommendations published by various organizations could have significant influence on the use of WAKIX.

Professional societies, practice management groups, private health and science foundations and organizations involved in various diseases from time to time may publish guidelines or recommendations to the healthcare and patient communities. The recommendations of these groups or organizations may relate to such matters as usage, dosage, route of administration and use of concomitant therapies. Recommendations or guidelines suggesting the reduced use of WAKIX or the use of competitive or alternative products as the standard of care to be followed by patients and healthcare providers could result in decreased use of WAKIX.

Product candidates we develop in the future may be classified as controlled substances, the making, use, sale, importation, exportation and distribution of which are subject to regulation by state, federal and foreign law enforcement and other regulatory agencies.

Product candidates we develop in the future may be classified as controlled substances, which are subject to state, federal and foreign laws and regulations regarding their manufacture, use, sale, importation, exportation and distribution. Controlled substances are regulated under the federal Controlled Substances Act of 1970, or CSA, and regulations of the DEA.

The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use and may not be marketed or sold in the United States. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances.

Various states also independently regulate controlled substances. Though state controlled substances laws often mirror federal law, because the states are separate jurisdictions, they may separately schedule drugs as well. While some states automatically schedule a drug when the DEA does so, in other states there must be rulemaking or a legislative action. State scheduling may delay commercial sale of any controlled substance drug product for which we obtain federal regulatory approval and adverse scheduling could impair the commercial attractiveness of such product. We or our collaborators must also obtain separate state registrations in order to be able to obtain, handle and distribute controlled substances for clinical trials or commercial sale, and failure to meet applicable regulatory requirements could lead to enforcement and sanctions from the states in addition to those from the DEA or otherwise arising under federal law.

For any of our products or product candidates classified as controlled substances, we and our suppliers, manufacturers, contractors, customers and distributors are required to obtain and maintain applicable registrations from state, federal and foreign law enforcement and regulatory agencies and comply with state, federal and foreign laws and regulations regarding the manufacture, use, sale, importation, exportation and distribution of controlled substances. There is a risk that DEA regulations may limit the supply of the compounds used in clinical trials for our product candidates, and, in the case of our approved products, the ability to produce and distribute our products in the volume needed to meet commercial demand.

Regulations associated with controlled substances govern manufacturing, labeling, packaging, testing, dispensing, production and procurement quotas, recordkeeping, reporting, handling, shipment and disposal. These regulations increase the personnel needs and the expense associated with development and commercialization of product candidates including controlled substances. The DEA, and some states, conduct periodic inspections of registered establishments that handle controlled substances. Failure to obtain and maintain required registrations or comply with any applicable regulations could delay or preclude us from developing and commercializing our product candidates containing controlled substances and subject us to enforcement action. The DEA may seek civil penalties, refuse to renew necessary registrations or initiate proceedings to revoke those registrations. In some circumstances, violations could lead to criminal proceedings. Because of their restrictive nature, these regulations could limit commercialization of any of our approved products or product candidates that are classified as controlled substances.

Enacted and future healthcare legislative changes may increase the difficulty and cost for us to obtain marketing approval for and commercialize our product candidates and affect the prices we may obtain.

In the United States, the European Union and other some foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any products for which we obtain marketing approval. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively the ACA, was enacted to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the healthcare industry, and impose additional healthcare policy reforms. The law has continued the downward pressure on pharmaceutical pricing, especially under the Medicare program, and increased the industry's regulatory burdens and operating costs. Among the provisions of the ACA of importance to the pharmaceutical industry and our potential product candidates are the following:

- an annual, non-deductible fee payable by any entity that manufactures or imports specified branded prescription drugs and biologic agents (other than those designated as orphan drugs), which is apportioned among these entities according to their market share in certain government healthcare programs;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program for branded and generic drugs;
- a methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts (increased to 70% pursuant to the Bipartisan Budget Act of 2018, effective as of January 1, 2019) off negotiated prices of applicable brand drugs to eligible beneficiaries under their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- establishment of a Center for Medicare Innovation at the CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Since its enactment, there have been numerous judicial, administrative, executive and legislative challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. Various portions of the ACA are currently undergoing challenges in the Fifth Circuit Court and the United States Supreme Court, the Trump Administration has issued various Executive Orders eliminating cost sharing subsidies and various provisions that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices, and Congress has introduced several pieces of legislation aimed at significantly revising or repealing the ACA. It is unclear whether the ACA will be overturned, repealed, replaced, or further amended. We cannot predict what affect further changes to the ACA would have on our business.

In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal and state legislation and regulations designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under government payor programs, and review 2020 relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. For example, in April of 2018, CMS published a final rule that would give states greater flexibility, starting in 2020, in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Further, Congress has indicated that it will continue to seek new legislative measures to control drug costs. For example, on September 25, 2019, the Senate Finance Committee introduced the Prescription Drug Pricing Reduction Action of 2019, a bill intended to reduce Medicare and Medicaid prescription drug prices. The proposed legislation would restructure the Part D benefit, modify payment methodologies for certain drugs, and impose an inflation cap on drug price increases. An even more restrictive bill, the Lower Drugs Costs Now Act of 2019 has passed out of the House and was delivered to the Senate on December 16, 2019. It would require HHS to directly negotiate drug prices with manufacturers. It is unclear whether either of these bills will make it through both chambers and be signed into law, and if either is enacted, what effect it would have on our business.

Additionally, in 2019, the Trump administration released a “Blueprint” to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The Trump administration’s budget proposal for fiscal year 2020 contains further drug price control measures that could be enacted during the 2020 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. HHS has also begun implementation of the Trump administration Blueprint, soliciting feedback on some of these measures and, immediately implementing others under its existing authority. For example, in May 2019, CMS issued a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2029 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several types of providers, including hospitals and cancer treatment centers, and increased the statute of limitations period in which the government may recover overpayments to providers from three to five years.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under government payor programs, and review 2020 relationship between pricing and manufacturer patient programs. While any proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. Furthermore, there has been increased interest by third party payors and governmental authorities in reference pricing systems and publication of discounts and list prices. These reforms could reduce the ultimate demand for our product candidates, once approved, or put pressure on our product pricing.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved. In markets outside of the United States and European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our current or any future product candidates we may develop may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs that we participate in, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We expect to participate in and have certain price reporting obligations to the Medicaid Drug Rebate Program. Under the Medicaid Drug Rebate Program, we would be required to pay a rebate to each state Medicaid program for our covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program as a condition of having federal funds being made available to the states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data we would have to report on a monthly and quarterly basis to the Centers for Medicare and Medicaid Services, or CMS, the federal agency that administers the Medicaid Drug Rebate Program. These data include, among other things, the average manufacturer price, or AMP, and, in the case of innovator products, the best price, or BP, for each drug which, in general, represents the lowest price available from the manufacturer to any entity in the U.S. in any pricing structure, calculated to include all sales and associated rebates, discounts and other price concessions. We are liable for errors associated with our submission of pricing data and for any overcharging of government payors. For example, failure to submit monthly/quarterly AMP and BP data on a timely basis could result in a civil monetary penalty for each day the submission is late beyond the due date. Failure to make necessary disclosures and/or to identify overpayments could result in allegations against us under the Federal False Claims Act and other laws and regulations. Any required refunds to the U.S. government or responding to a government investigation or enforcement action would be expensive and time consuming and could have a material adverse effect on our business, results of operations and financial condition.

Federal law requires that any company that participates in the Medicaid Drug Rebate Program also participate in the 340B program in order for federal funds to be available for the manufacturer's drugs under Medicaid and Medicare Part B. The 340B program requires participating manufacturers to agree to charge statutorily defined covered entities no more than the 340B "ceiling price" for the manufacturer's covered outpatient drugs. These 340B covered entities include a variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as hospitals that serve a disproportionate share of low-income patients. The ACA expanded the list of covered entities to include certain free-standing cancer hospitals, critical access hospitals, rural referral centers and sole community hospitals, but exempts "orphan drugs" from the ceiling price requirements for these covered entities. The 340B ceiling price is calculated using a statutory formula based on the average manufacturer price and rebate amount for the covered outpatient drug as calculated under the Medicaid Drug Rebate Program, and in general, products subject to Medicaid price reporting and rebate liability are also subject to the 340B ceiling price calculation and discount requirement. Any additional future changes to the definition of average manufacturer price and the Medicaid rebate amount under the ACA or other legislation or regulation could affect our 340B ceiling price calculations and negatively impact our results of operations commercializing pitolisant. In addition, legislation may be introduced that, if passed, would further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting.

In order to be eligible to have our products that we successfully commercialize paid for with federal funds under the Medicaid program and purchased by certain federal agencies and grantees, we also would have to participate in the U.S. Department of Veterans Affairs, or VA, Federal Supply Schedule, or FSS, pricing program. As part of this program, we would be obligated to make our products available for procurement on an FSS contract under which we must comply with standard government terms and conditions and charge a price that is no higher than the statutory Federal Ceiling Price, or FCP, to four federal agencies (VA, U.S. Department of Defense, or DOD, Public Health Service, and U.S. Coast Guard).

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws, and antimony laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic markets. We can face criminal liability and other serious consequences for violations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, the U.S. Foreign Corrupt Practices Act of 1977, as amended, the United States domestic bribery statute contained in 18 U.S.C. § 201, the United States Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors and other partners from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors and other partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences.

Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, which could prevent new products and services from being developed or commercialized in a timely manner, which could negatively affect our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel, accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the United States Securities and Exchange Commission, or SEC, have had to furlough critical FDA, SEC and other governmental employees and stop critical activities. Our business depends upon the ability of the FDA to accept and review our potential regulatory filings. If a prolonged government shutdown occurs, it could significantly affect the ability of the FDA to timely review and process our regulatory submissions, which harm our business. Similarly, a prolonged government shutdown could

prevent the timely review of any of our patent applications by the United States Patent and Trademark Office, or USPTO, which could delay the issuance of any U.S. patents to which we might otherwise be entitled. Further, upon completion of this offering and in our operations as a public company, future government shutdowns could affect our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

We will need to obtain FDA approval of any proposed product names, and any failure or delay associated with such approval may adversely affect our business.

Any proprietary name we intend to use for our product candidates will require approval from the FDA regardless of whether we have secured a formal trademark registration from the U.S. Patent and Trademark Office. The FDA reviews proposed product names, considering both the potential for the name to lead to medical errors due to confusion with other product names and whether the proposed name is overly fanciful, misleadingly implies unique effectiveness or composition, or contributes to overstatement of product efficacy, minimization of risk, broadening of product indications or unsubstantiated superiority. If the FDA objects to any of our proposed product names, we may be required to adopt an alternative name for our product candidates. If we adopt an alternative name, we would lose the benefit of our existing trademark applications for such product candidate, and may be required to expend significant additional resources in an effort to identify a suitable product name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. We may be unable to build a successful brand identity for a new trademark in a timely manner or at all, which would limit our ability to commercialize our product candidates.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely, and will continue to rely, on a combination of patents, trademarks and confidentiality agreements with employees, consultants, collaborators, advisors and other third parties to protect the intellectual property related to our current and future product candidates. Our success depends in large part on our licensor's ability to obtain and maintain patent protection in the United States with respect to WAKIX and our ability to obtain and maintain patent protection in the United States and any other relevant foreign jurisdictions with respect to any future product candidates that we develop. We seek to ensure that our current and future licensors obtain appropriate patent protection to all product candidates that we license from them. The patent prosecution process is expensive and time-consuming, and we and our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

Our patent portfolio comprises four U.S. patents exclusively licensed to us from Bioprojet. One U.S. patent has claims directed to a crystalline form of pitolisant and, methods for preparing the crystalline form of pitolisant which is expected to expire in February 2029 without taking into consideration any possible patent term extension. A second U.S. patent has claims directed to methods of treating excessive daytime sleepiness by administering pitolisant, which is expected to expire in September 2029 without taking into consideration any possible patent term extension. With all applicable patent term adjustments available and granted to us, the term of the last-to-expire pitolisant-related patent in our portfolio extends to September 2029.

The patents that we in-license now or the patents and patent applications that we own or in-license in the future may not have patentable claims that protect our current and future product candidates in the relevant jurisdictions where we intend to commercialize such products. There is no assurance that we and our licensor are aware of all potentially relevant prior art relating to future patent

applications. As such, the patent examiner may find prior art that can prevent a patent from issuing from a pending patent application. During the patent examination process, we or our licensor may be required to narrow the pending claims to overcome prior art, a process that may limit the scope of patent protection. Even if patents do successfully issue based on our future patent applications, and even if the issued patents cover our current and future product candidates, including their compositions formulation, method of manufacture, and method of use, third parties may challenge our issued patents' validity, enforceability or scope, which may result in such patents being narrowed, invalidated or held unenforceable. Any successful opposition to these patents or any other patents owned by or licensed to us in the future could deprive us of rights necessary for the successful commercialization of any of our current or future product candidates, if approved. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

If the patent applications we may own or in-license in the future with respect to our current and future product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for any of our current or future product candidates, it could dissuade other companies from collaborating with us to develop future product candidates, and threaten our ability to commercialize our current and future product candidates. Notably, pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Any such outcome could have an adverse effect on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does. Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act made a number of significant changes to United States patent laws. These include provisions that affect the way patent applications are prosecuted and challenged at the U.S. Patent and Trademark Office, or the USPTO, and may also affect patent litigation. The USPTO has developed and continues to develop new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it remains unclear what impact the Leahy-Smith Act, subsequent rulemaking, and judicial interpretation of the Leahy-Smith Act and regulations will have on the operation of our business. However, the Leahy- Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have an adverse effect on our business and financial condition. Moreover, future changes

to the patent laws of the United States and foreign jurisdictions may adversely affect the term, scope, validity and enforceability of our or our licensor's patent rights. For example, a new bill ("Terminating the Extension of Rights Misappropriated Act, or TERM Act, H.R. 3199) percolating through the United States Congress aims to reduce the term of certain drug patents in order to ease generic entry and increase competition.

The inventorship and ownership rights for patents that we in-license or may own or in-license in the future may be challenged by third parties. Such challenges could result in loss of exclusive rights to such patents, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or require us to obtain a license from such third parties on commercially reasonable terms to secure exclusive rights. If any such challenges to inventorship or ownership were asserted, there is no assurance that a court would find in our favor or that, if we choose to seek a license, such license would be available to us on acceptable terms or at all.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in pre- and post-issuance opposition, derivation, re-examination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications, whether owned or in-licensed now or in the future, is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our licensed patents may be challenged in the courts or patent offices in the United States. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after the filing of the earliest non-provisional application to which the patent claims priority. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. We may be required to disclaim a portion of patent term in order to overcome double patenting rejections from the patent office, thus potentially shortening our exclusivity period. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we fail to comply with our obligations under any license, collaboration or other agreements, we may be required to pay damages and could lose intellectual property rights that are necessary for developing and protecting our current and future product candidates.

We have licensed certain intellectual property rights covering pitolisant from Bioprojet, and we may license intellectual property rights from others in the future. If, for any reason, our license agreement with Bioprojet or any future licensor is terminated or we otherwise lose the rights associated with a license, it could adversely affect our business. Our license agreement with Bioprojet imposes, and any future collaboration agreements or license agreements we enter into are likely to impose various development, commercialization, funding, milestone, royalty, diligence, sublicensing,

insurance, patent prosecution and enforcement or other obligations on us. If we breach any material obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and the licensor may have the right to terminate the license, which could result in us being unable to develop, manufacture and sell products that are covered by the licensed technology, or having to negotiate new or reinstated licenses on less favorable terms, or enable a competitor to gain access to the licensed technology.

If we do not obtain protection under the Hatch-Waxman Amendments by extending the patent term for our current and future product candidates, our business may be harmed.

Our commercial success will largely depend on our licensor's ability to obtain and maintain patent and other intellectual property in the United States for pitolisant, and our target indications, and our ability to maintain obtain and maintain patent and other intellectual property in the United States for any product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting product candidates might expire before or shortly after such candidates begin to be commercialized. We expect to seek extensions of patent terms in the United States.

Depending upon the timing, duration and specifics of FDA marketing approval of our current and future product candidates, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years beyond the normal expiration of the patent as compensation for patent term lost during drug development and the FDA regulatory review process, which is limited to the approved indication (or any additional indications approved during the period of extension). This extension is limited to only one patent that covers the approved product, the approved use of the product, or a method of manufacturing the product. However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. We may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time-period or the scope of patent protection afforded could be less than we request.

If we or our licensor are unable to extend the expiration date of our or their existing patents or obtain new patents with longer expiry dates, as applicable, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data to obtain approval of competing products following our patent expiration and launch their product earlier than might otherwise be the case.

The validity, scope and enforceability of any patents listed in the Orange Book that cover our current and future product candidates can be challenged by third parties.

One or more third parties may challenge the current patents, or future patents within our portfolio, which could result in the invalidation of, or render unenforceable, some or all of the relevant patent claims or a finding of non-infringement. For example, if a third party files an Abbreviated New Drug Application, or ANDA, for a generic drug containing pitolisant, and relies in whole or in part on studies conducted by or for us, the third party will be required to certify to the FDA that either: (1) there is no patent information listed in the FDA's Orange Book with respect to our NDA for the applicable approved product candidate; (2) the patents listed in the Orange Book have expired; (3) the listed patents have not expired, but will expire on a particular date and approval is sought after patent expiration; or (4) the listed patents are invalid or will not be infringed by the manufacture, use or sale of

the third party's generic drug. A certification that the new drug will not infringe the Orange Book-listed patents for the applicable approved product candidate, or that such patents are invalid, is called a paragraph IV certification. If the third party submits a paragraph IV certification to the FDA, a notice of the paragraph IV certification must also be sent to us once the third party's ANDA is accepted for filing by the FDA. We may then initiate a lawsuit to defend the patents identified in the notice. The filing of a patent infringement lawsuit within 45 days of receipt of the notice automatically prevents the FDA from approving the third party's ANDA until the earliest of 30 months or the date on which the patent expires, the lawsuit is settled, or the court reaches a decision in the infringement lawsuit in favor of the third party. If we do not file a patent infringement lawsuit within the required 45-day period, the third party's ANDA will not be subject to the 30-month stay of FDA approval.

Moreover, a third party may challenge the current patents, or future patents within our portfolio, which could result in the invalidation of some or all of the patents that might otherwise be eligible for listing in the Orange Book for one of our products. If a third party successfully challenges all of the patents that might otherwise be eligible for listing in the Orange Book for one of our products, we will not be entitled to the 30-month stay of FDA approval upon the filing of an ANDA for a generic drug containing, for example, pitolisant, and relies in whole or in part on studies conducted by or for us.

Litigation or other proceedings to enforce or defend intellectual property rights are often very complex in nature, may be very expensive and time-consuming, may divert our management's attention from our core business, and may result in unfavorable results that could limit our ability to prevent third parties from competing with our current and future product candidates.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for noncompliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and other foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign national or international patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of patent rights include, but are not limited to, failure to timely file national and regional stage patent applications based on our international patent application, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain patents and patent applications, whether owned or in-licensed now or in the future, covering any of our current or future product candidates, our competitors might be able to enter the market, which would have an adverse effect on our business.

We may need to acquire or license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent rights that are important or necessary to the development of our current and future product candidates. It may be necessary for us to use the patented or proprietary technology of one or more third parties to commercialize our current and future product candidates. If we are unable to acquire such intellectual property outright, or obtain licenses to such intellectual property from such third parties when needed or on commercially reasonable terms, our ability to commercialize our current and future product candidates, if approved, would likely be delayed.

The risks described elsewhere pertaining to our intellectual property rights also apply to the intellectual property rights that we in-license, and any failure by us or our licensors to obtain, maintain, defend and enforce these rights could have an adverse effect on our business. In some cases we may not have control over the prosecution, maintenance or enforcement of the patents that we license, and may not have sufficient ability to provide input into the patent prosecution, maintenance and defense process with respect to such patents, and our licensors may fail to take the steps that we believe are necessary or desirable in order to obtain, maintain, defend and enforce the licensed patents.

Third-party claims or litigation alleging infringement of patents or other proprietary rights, or seeking to invalidate patents or other proprietary rights, may delay or prevent the development and commercialization of any of our current or future product candidates.

Our commercial success depends in part on our avoiding infringement and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the pharmaceutical and biotechnology industries, including patent infringement lawsuits, interferences, derivation and administrative law proceedings, inter partes review and post-grant review before the USPTO, as well as oppositions and similar processes in foreign jurisdictions. Numerous United States and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are or may in the future be developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our product candidates or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties.

Third parties may assert that we are infringing their patents or employing their proprietary technology without authorization.

There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our current and future product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our current and future product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our current and future product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patent was to be held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patent may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our current and future product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation

expense and would be a substantial diversion of employee resources from our business. In the event of a successful infringement or other intellectual property claim against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our affected products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our current and future product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our current and future product candidates, which could harm our business significantly. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We cannot provide any assurances that third-party patents do not exist which might be enforced against our current and future product candidates, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties or other forms of compensation to third parties.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our products.

We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our current product candidate in any jurisdiction.

It is possible that we and our current and future licensors will fail to identify patentable aspects of research and development output before it is too late to obtain patent protection. The patent applications that we may own or in-license in the future may fail to result in issued patents with claims that cover our current and future product candidates. We and our current and future licensors may also inadvertently make statements to regulatory agencies during the regulatory approval process that may be inconsistent with positions that have been taken during prosecution of the patent applications, which may result in such patents being narrowed, invalidated or held unenforceable.

The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively affect our ability to market our products. We may incorrectly determine that our products are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect, and our failure to identify and correctly interpret relevant patents may negatively affect our ability to develop and market our products.

We may become involved in lawsuits to protect or enforce our patents, the patents of our licensors or our other intellectual property rights, which could be expensive, time consuming and unsuccessful.

Competitors may infringe or otherwise violate the patents of our licensor or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims,

which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that an asserted patent is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that the asserted patent does not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of asserted patents at risk of being invalidated or interpreted narrowly and could put a related patent application at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte re-examinations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we may license in the future, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could harm our business.

We may not be able to detect or prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Our business could be harmed if in litigation the prevailing party does not offer us a license on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common shares.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may conclude that even if a third party is infringing our in-licensed patents, any patents that may be issued as a result of our future patent applications, or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our shareholders. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution.

Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

The United States has recently enacted and implemented wide-ranging patent reform legislation. The U.S. Supreme Court and the U.S. Court of Appeals for the Federal Circuit have issued numerous precedential opinions in recent years narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have licensed or that we might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

The U.S. federal government retains certain rights in inventions produced with its financial assistance under the Bayh-Dole Act. The federal government retains a “nonexclusive, non-transferable, irrevocable, paid-up license” for its own benefit. The Bayh-Dole Act also provides federal agencies with “march-in rights”. March-in rights allow the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a “nonexclusive, partially exclusive, or exclusive license” to a “responsible applicant or applicants.” If the patent owner refuses to do so, the government may grant the license itself.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other third parties.

We employ individuals who were previously employed at other biotechnology or pharmaceutical companies. Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, collaborators and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees’ former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our future patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, results of operations and financial condition.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we or our licensors fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we and our licensors are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Any trademarks we have obtained or may obtain may be infringed or be successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish any of our current and future product candidates that are approved for marketing from the products of our competitors. For example, we are marketing pitolisant for the treatment of adult patients with EDS in adult patients with narcolepsy under the brand name WAKIX, which we have licensed from Bioprojet. We may design or create new trademarks and apply to register them, our trademark applications may not be approved in the United States or any relevant foreign jurisdiction. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks and we may not have adequate resources to enforce our trademarks. If we attempt to enforce our trademarks and assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Risks Related to Being a Public Company

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to compliance with our public company responsibilities and corporate governance practices.

As a public company, and particularly after we are no longer an “emerging growth company,” we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, or the Sarbanes Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of and other applicable securities rules and regulations impose various requirements on public companies. Our management and other personnel will need to devote a substantial amount of time to compliance with these requirements. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. If, notwithstanding our efforts to comply with new or changing laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us, and our business may be harmed. Further, failure to comply with these laws, regulations and standards may make it more difficult and more expensive for us to obtain directors’ and officers’ liability insurance, which could make it more difficult for us to attract and retain qualified members to serve on our board of directors or committees or as members of senior management. We cannot predict or estimate the amount of additional costs we will incur as a public company or the timing of such costs.

As a result of becoming a public company, we will be obligated to develop and maintain proper and effective internal controls over financial reporting and any failure to maintain the adequacy of these internal controls may adversely affect investor confidence in our company and, as a result, the value of our common shares.

We will be required, pursuant to Section 404 of the Sarbanes Oxley Act, or Section 404, to furnish a report by management on, among other things, the effectiveness of our internal controls over

financial reporting for the fiscal year beginning January 1, . This assessment will need to include disclosure of any material weaknesses identified by our management in our internal controls over financial reporting. Our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting until our first annual report required to be filed with the SEC following the date we are no longer an emerging growth company, as defined in the JOBS Act. At such time as we are required to obtain auditor attestation, if we then have a material weakness, we would receive an adverse opinion regarding our internal control over financial reporting from our independent registered public accounting firm. We will be required to disclose significant changes made in our internal controls procedures on a quarterly basis.

We are beginning the costly and challenging process of compiling the system and processing documentation necessary to perform the evaluation needed to comply with Section 404, and we may not be able to complete our evaluation, testing and any required remediation in a timely fashion. Our compliance with Section 404 will require that we incur substantial legal, accounting and other compliance expense and expend significant management efforts. We currently do not have an internal audit group, and we will need to hire additional accounting and finance staff and consultants with appropriate public company experience and technical accounting knowledge and compile the system and process documentation necessary to perform the evaluation needed to comply with Section 404.

During the evaluation and testing process of our internal controls, if we identify one or more material weaknesses in our internal controls over financial reporting, we will be unable to assert that our internal controls over financial reporting are effective. We cannot assure you that there will not be material weaknesses or significant deficiencies in our internal controls over financial reporting in the future. Any failure to maintain effective internal controls over financial reporting could severely inhibit our ability to accurately report our financial condition or results of operations. If we are unable to conclude that our internal controls over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal controls over financial reporting, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common shares could decline, and we could be subject to sanctions or investigations by , the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal controls over financial reporting, or to implement or maintain other effective control systems required of public companies, could also negatively impact our ability to access to the capital markets.

In addition, effective disclosure controls and procedures enable us to make timely and accurate disclosure of financial and non-financial information that we are required to disclose. As a public company, if our disclosure controls and procedures are ineffective, we may be unable to report our financial results or make other disclosures accurately on a timely basis, which could cause our reported financial results or other disclosures to be materially misstated and result in the loss of investor confidence and cause the market price of our common shares to decline. If we were to subsequently elect instead to comply with these public company effective dates, such election would be irrevocable pursuant to the JOBS Act.

We are an emerging growth company and we cannot be certain if the reduced disclosure requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an “emerging growth company” as defined in the JOBS Act. Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to use this exemption from new or revised accounting standards and, therefore, we will not be subject to the same new or revised accounting standards as other public companies that have not made this election.

For as long as we continue to be an emerging growth company, we also intend to take advantage of certain other exemptions from various reporting requirements that are applicable to other public companies including, but not limited to, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a non-binding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We will remain an emerging growth company until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenue of $1.07 billion or more; (ii) the last day of the fiscal year following the fifth anniversary of the date of the closing of this offering; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three fiscal years; or (iv) the date on which we are deemed to be a “large accelerated filer” under the rules of the SEC.

Our management team has limited experience managing a public company.

Our chief executive officer does not have experience managing a public company, interacting with public company investors or complying with the increasingly complex laws pertaining to public companies. Our management team, as a whole, may not successfully or efficiently manage the transition to being a public company subject to significant regulatory oversight and reporting obligations under the federal securities laws and the continuous scrutiny of securities analysts and investors. These new obligations and constituents will require significant attention from our senior management, particularly from our chief executive officer, and could divert their attention away from the day-to-day management of our business, which could adversely affect our revenue, business, results of operations and financial condition.

Risks Related to This Offering and Ownership of Our Common Stock

If you purchase shares of our common stock in this offering, you will incur immediate and substantial dilution.

The offering price of our common stock is substantially higher than the net tangible book value per share of our common stock, which on a pro forma basis was $ per share of our common stock as of , 2019. Based on the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, you will experience immediate dilution of $ per share, representing the difference between our pro forma net tangible book value per share after giving effect to this offering and the assumed initial public offering price. This means that you will pay a higher price per share than the amount of our total tangible assets, less our total liabilities, divided by the number of shares of common stock outstanding. Furthermore, if the underwriters exercise their over-allotment option or our previously issued options, warrant and other rights to acquire common stock at prices below the assumed initial public offering price are exercised, you will experience further dilution. In addition, you may also experience additional dilution if options or other rights to purchase our common stock that are outstanding or that we may issue in the future are exercised or converted or we issue additional shares of our common stock at prices lower than our net tangible book value at such time. See “Dilution.”

No public market for our common stock currently exists, and an active trading market may not develop or be sustained following this offering.

Prior to this offering, there has been no public market for our common stock. Although we have applied to have our common stock listed on the , an active trading market may not develop

following the closing of this offering or, if developed, may not be sustained. The lack of an active market may impair your ability to sell your shares at the time you wish to sell them or at a price that you consider reasonable. The lack of an active market may also reduce the fair market value of your shares. An inactive market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration. The initial public offering price was determined by negotiations between us and the underwriters and may not be indicative of the future prices of our common stock.

Our share price may be volatile, and you may be unable to sell your shares at or above the offering price.

The market price of our common stock is likely to be volatile and could be subject to wide fluctuations in response to many risk factors listed in this section, and others beyond our control, including:

- the success of existing or new competitive products or technologies;
- regulatory actions with respect to pitolisant or any other potential product candidates or our competitors' products and product candidates;
- actual or anticipated fluctuations in our financial condition and operating results, including fluctuations in our quarterly and annual results;
- announcements of innovations by us or our competitors;
- overall conditions in our industry and the markets in which we operate;
- market conditions or trends in the biotechnology industry or in the economy as a whole;
- addition or loss of significant healthcare providers or other developments with respect to significant healthcare providers;
- changes in laws or regulations applicable to pitolisant or any other potential product candidates;
- actual or anticipated changes in our growth rate relative to our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;
- additions or departures of key personnel;
- competition from existing products or new products that may emerge;
- issuance of new or updated research or reports by securities analysts;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- disputes or other developments related to proprietary rights, including patents, and our ability to obtain intellectual property protection for our products;
- security breaches;
- litigation matters;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us or our stockholders;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;

- the expiration of contractual lock-up agreements with our executive officers, directors and stockholders; and
- general economic and market conditions.

Furthermore, the stock markets have experienced price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many companies. These fluctuations often have been unrelated or disproportionate to the operating performance of those companies. These broad market and industry fluctuations, as well as general economic, political and market conditions such as recessions, interest rate changes or international currency fluctuations, may negatively affect the market price of our common stock. If the market price of our common stock after this offering does not exceed the initial public offering price, you may not realize any return on your investment in us and may lose some or all of your investment. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities litigation. This risk is especially relevant for biopharmaceutical companies, which have experienced significant stock price volatility in recent years. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Our directors, officers and principal stockholders beneficially own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of , 2020, our directors, officers, five percent or greater stockholders, and their respective affiliates beneficially owned in the aggregate approximately % of our outstanding voting stock and, upon the completion of this offering, that same group will beneficially own in the aggregate approximately % of our outstanding voting stock (assuming no exercise of the underwriters' option to purchase additional shares). As a result, these stockholders have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, and approval of any merger, sale of assets or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Future sales of our common stock in the public market could cause our share price to fall.

Sales of a substantial number of shares of our common stock in the public market after this offering, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. Based on shares of common stock outstanding as of , 2020, the conversion of all of our preferred stock immediately prior to the closing of this offering into shares of common stock and the payment of an accrued dividend to holders of our convertible preferred stock upon the closing of this offering in the aggregate amount of shares of our common stock, upon the closing of this offering, we will have shares of common stock outstanding.

All of the common stock sold in this offering will be freely tradable without restrictions or further registration under the Securities Act of 1933, as amended, or the Securities Act, except for any shares held by our affiliates as defined in Rule 144 under the Securities Act. The remaining shares of common stock outstanding after this offering will be restricted as a result of securities laws, lock-up agreements or other contractual restrictions that restrict transfers for at least 180 days after the date of this prospectus, subject to certain extensions. See also the section of this prospectus captioned "Shares Eligible For Future Sale."

The underwriters may, in their sole discretion, release all or some portion of the shares subject to lock-up agreements with the underwriters prior to expiration of the lock-up period. For more information regarding the lock-up agreements with the underwriters, see the section of this prospectus captioned "Underwriting."

The holders of shares of common stock, or %, based on shares outstanding on an as-converted basis as of , 2020, the conversion of all of our preferred stock immediately prior to the closing of this offering into shares of common stock and the payment of an accrued dividend to holders of our convertible preferred stock upon the closing of this offering in the aggregate amount of shares of our common stock, will be entitled to rights with respect to registration of such shares under the Securities Act pursuant to a registration rights agreement between such holders and us. See "Description of Capital Stock—Registration Rights" below. If such holders, by exercising their registration rights, sell a large number of shares, they could adversely affect the market price for our common stock. We intend to file a registration statement on Form S-8 under the Securities Act to register the shares subject to outstanding stock options under the Equity Incentive Plan as of the date of this prospectus and shares of common stock for issuance under our 2020 Plan. The 2020 Plan will provide for automatic increases in the shares reserved for grant or issuance under the plan which could result in additional dilution to our stockholders. Once we register the shares under these plans, they can be freely sold in the public market upon issuance and vesting, subject to a 180-day lock-up period and other restrictions provided under the terms of the applicable plan and/or the award agreements entered into with participants.

Our management has broad discretion in the use of the net proceeds from this offering and may not use the net proceeds effectively.

Our management will have broad discretion in the application of the net proceeds of this offering. We cannot specify with certainty the uses to which we will apply these net proceeds. The failure by our management to apply these funds effectively could adversely affect our ability to continue maintaining and expanding our business.

If securities or industry analysts do not publish research or publish unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline. Moreover, if our operating results do not meet the expectations of the investor community, one or more of the analysts who cover our company may change their recommendations regarding our company, and our stock price could decline.

Our quarterly operating results may fluctuate significantly.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expenses related to our development programs;
- addition or termination of clinical trials;
- any intellectual property infringement lawsuit in which we may become involved;
- regulatory developments affecting pitolisant;
- our execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements;

- the achievement and timing of milestone payments under our existing collaboration and license agreements; and
- the level of underlying demand for WAKIX and customers' buying patterns.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially.

Future sales and issuances of our common stock or rights to purchase our common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause the stock price of our common stock to decline.

We may issue additional securities following the closing of this offering. In the future, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. We also expect to issue common stock to employees, consultants and directors pursuant to our equity incentive plans. If we sell common stock, convertible securities or other equity securities in subsequent transactions, or common stock is issued pursuant to equity incentive plans, investors may be materially diluted. New investors in such subsequent transactions could gain rights, preferences and privileges senior to those of holders of our common stock.

We have never paid dividends on our common stock and we do not intend to pay dividends for the foreseeable future. Consequently, any gains from an investment in our common stock will likely depend on whether the price of our common stock increases.

We have never declared or paid any dividends on our common stock and do not intend to pay any dividends in the foreseeable future. We anticipate that we will retain all of our future earnings for use in the operation of our business and for general corporate purposes. Any determination to pay dividends in the future will be at the discretion of our board of directors. Accordingly, investors must rely on sales of their common stock after price appreciation, which may never occur, as the only way to realize any future gains on their investments. Furthermore, we are party to a Credit Agreement with OrbiMed that contains negative covenants that limit our ability to pay dividends. For more information, see the section of this prospectus captioned "Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources."

Our charter documents and Delaware law could prevent a takeover that stockholders consider favorable and could also reduce the market price of our stock.

Our amended and restated certificate of incorporation and our amended and restated bylaws will contain provisions that could delay or prevent a change in control of our company. These provisions could also make it more difficult for stockholders to elect directors and take other corporate actions. These provisions include:

- providing for a classified board of directors with staggered, three-year terms;
- authorizing our board of directors to issue preferred stock with voting or other rights or preferences that could discourage a takeover attempt or delay changes in control;
- prohibiting cumulative voting in the election of directors;
- providing that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;

- prohibiting the adoption, amendment or repeal of our amended and restated bylaws or the repeal of the provisions of our amended and restated certificate of incorporation regarding the election and removal of directors without the required approval of at least 66.67% of the shares entitled to vote at an election of directors;
- prohibiting stockholder action by written consent;
- limiting the persons who may call special meetings of stockholders; and
- requiring advance notification of stockholder nominations and proposals.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, which is responsible for appointing the members of our management.

In addition, we are subject to the anti-takeover provisions contained in Section 203 of the Delaware General Corporation Law, or the DGCL. Under Section 203 of the DGCL, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other exceptions, the board of directors has approved the transaction.

These and other provisions in our amended and restated certificate of incorporation and our amended and restated bylaws and under Delaware law could discourage potential takeover attempts, reduce the price investors might be willing to pay in the future for shares of our common stock and result in the market price of our common stock being lower than it would be without these provisions. For more information, see the section of this prospectus captioned “Description of Capital Stock—Anti-Takeover Provisions.”

Our amended and restated certificate of incorporation that will become effective immediately prior to the closing of this offering provides that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation that will become effective immediately prior to the closing of this offering provides that the Court of Chancery of the State of Delaware is the exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by, or other wrongdoing by, any of our current or former directors, officers, employees or our stockholders;
- any action asserting a claim against us arising under the DGCL, our amended and restated certificate of incorporation, or our amended and restated bylaws (as either may be amended from time to time) or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

By becoming a stockholder in our Company, you will be deemed to have notice of and have consented to the provisions of our amended and restated certificate of incorporation related to choice of forum. This exclusive forum provision may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to

find the exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful third-party claims against us and may reduce the amount of money available to us.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that we will indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law.

In addition, as permitted by Section 145 of the DGCL, our amended and restated bylaws and our indemnification agreements that we have entered into with our directors and officers provide that:

- we will indemnify our directors and officers for serving us in those capacities or for serving other business enterprises at our request, to the fullest extent permitted by Delaware law. Delaware law provides that a corporation may indemnify such person if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the registrant and, with respect to any criminal proceeding, had no reasonable cause to believe such person's conduct was unlawful;
- we may, in our discretion, indemnify employees and agents in those circumstances where indemnification is permitted by applicable law;
- we are required to advance expenses, as incurred, to our directors and officers in connection with defending a proceeding, except that such directors or officers shall undertake to repay such advances if it is ultimately determined that such person is not entitled to indemnification;
- we will not be obligated pursuant to our amended and restated bylaws to indemnify a person with respect to proceedings initiated by that person against us or our other indemnitees, except with respect to proceedings authorized by our board of directors or brought to enforce a right to indemnification;
- the rights conferred in our amended and restated bylaws are not exclusive, and we are authorized to enter into indemnification agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons; and
- we may not retroactively amend our amended and restated bylaw provisions to reduce our indemnification obligations to directors, officers, employees and agents.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus includes forward-looking statements within the meaning of the federal securities laws. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends affecting the operating results and financial condition of our business. Forward-looking statements should not be read as a guarantee of future performance or results, and will not necessarily be accurate indications of the times at, or by, which such performance or results will be achieved. Forward-looking statements are based on information available at the time those statements are made and/or management's good faith belief as of that time with respect to future events, and are subject to risks and uncertainties that could cause actual performance or results to differ materially from those expressed in or suggested by the forward-looking statements. Important factors that could cause such differences include, but are not limited to, statements about:

- our commercialization efforts and strategy for WAKIX;
- the rate and degree of market acceptance and clinical utility of WAKIX, pitolisant in additional indications, if approved, and any other product candidates we may develop or acquire, if approved;
- our research and development plans, including our plans to explore the therapeutic potential of pitolisant in additional indications;
- our ongoing and planned clinical trials;
- our ability to expand the scope of our license agreement with Bioprojet;
- the availability of favorable insurance coverage and reimbursement for WAKIX;
- the timing of and our ability to obtain regulatory approvals for pitolisant for other indications as well as any other product candidates;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- our ability to identify additional products or product candidates with significant commercial potential that are consistent with our commercial objectives;
- our commercialization, marketing and manufacturing capabilities and strategy;
- significant competition in our industry;
- our intellectual property position;
- loss or retirement of key members of management;
- failure to successfully execute our growth strategy, including any delays in our planned future growth;
- our failure to maintain effective internal controls; and
- the impact of government laws and regulations.

In this prospectus, the words "believe," "may," "will," "estimate," "continue," "anticipate," "intend," "expect," "predict," "potential" and similar expressions, as they relate to our company, our business and our management, are intended to identify forward-looking statements. In light of these risks and uncertainties, the forward-looking events and circumstances discussed in this prospectus may not occur and actual results could differ materially from those anticipated or implied in the forward-looking statements.

Forward-looking statements speak only as of the date of this prospectus. You should not put undue reliance on any forward-looking statements. We assume no obligation to update forward-looking

statements to reflect actual results, changes in assumptions or changes in other factors affecting forward-looking information, except to the extent required by applicable laws. If we update one or more forward-looking statements, no inference should be drawn that we will make additional updates with respect to those or other forward-looking statements.

Unless otherwise indicated, information contained in this prospectus concerning our industry, including industry statistics and forecasts, competitive position and the markets in which we operate is based on information from independent industry and research organizations, other third-party sources and management estimates. Management estimates are derived from publicly available information released by independent industry analysts and other third-party sources, as well as data from our internal research, and are based on assumptions made by us upon reviewing such data, and our experience in, and knowledge of, such industry and markets, which we believe to be reasonable. In addition, projections, forecasts, assumptions and estimates of the future performance of the industry in which we operate and our future performance are necessarily subject to uncertainty and risk due to a variety of factors, including those described in “Risk Factors” and “Cautionary Note Regarding Forward-Looking Statements.” These and other factors could cause results to differ materially from those expressed and forecasts in the estimates made by the independent parties and by us.

Unless expressly stated, we obtained industry, business, market and other data from the reports, publications and other materials and sources listed below. In some cases, we do not expressly refer to the sources from which this data is derived. In that regard, when we refer to one or more sources of this type of data in any paragraph, you should assume that other data of this type appearing in the same paragraph is derived from the same sources unless otherwise expressly stated or the context otherwise requires.

- U.S. Food and Drug Administration, The Voice of the Patient – Narcolepsy (“Voice of the Patient”), June 2014
- Versta Research, Know Narcolepsy Survey (“Know Narcolepsy”), October 2018 (conducted by Versta Research on our behalf, and in collaboration with Narcolepsy Network, and respondents included 200 U.S. adults with narcolepsy, 1,203 U.S. adults without narcolepsy, and 251 physicians currently in clinical practice who have treated patients with narcolepsy in the last two years)

You should read this prospectus and the documents that we reference in this prospectus and have filed with the Securities and Exchange Commission, or SEC, as exhibits to the registration statement of which this prospectus is a part with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

USE OF PROCEEDS

We estimate, based upon an assumed initial public offering price of $ per share (which is the midpoint of the price range set forth on the cover page of this prospectus), that we will receive net proceeds from this offering of approximately $ million (or $ million if the underwriters exercise their option to purchase additional shares of common stock in full), after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

We currently estimate that we will use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately $ million to fund the clinical development of additional indications for pitolisant in PWS, DM1 and pediatric narcolepsy through ; and
- the remainder for working capital, business development opportunities, potential milestone payments to Bioprojet and general corporate purposes, including to support the continued commercialization of WAKIX in the United States.

Our expected use of proceeds from this offering represents our current intentions based on our present plans and business condition. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the proceeds to be received upon the completion of this offering or the actual amounts that we will spend on the uses set forth above. We may also use a portion of the proceeds to in-license, acquire or invest in additional businesses, technologies, products or assets. Although we have no specific agreements, commitments or understandings with respect to any in-licensing activity or acquisition, we evaluate these opportunities and engage in related discussions with other companies from time to time.

The amount and timing of our actual expenditures will depend on numerous factors, including the results of our research and development efforts, the timing and outcome of any ongoing or future preclinical studies or clinical trials, and the timing and outcome of regulatory submissions. As a result, our management will have broad discretion over the use of the proceeds from this offering.

Pending the use of the proceeds from this offering, we may invest the proceeds in interest- bearing, investment-grade securities, certificates of deposit or government securities.

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share (which is the midpoint of the price range set forth on the cover page of this prospectus) would increase (decrease) the net proceeds to us from this offering by approximately $ million, assuming the number of shares offered, as set forth on the cover page of this prospectus, remains the same, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each 1,000,000 share increase (decrease) in the number of shares offered in this offering would increase (decrease) the net proceeds to us from this offering by approximately $ million, assuming that the price per share for the offering remains at $ (which is the midpoint of the price range set forth on the cover page of this prospectus), and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

CAPITALIZATION

The following table sets forth the cash and capitalization as of December 31, 2019, as follows:

- on an actual basis;
- on a pro forma basis to give effect to (i) the conversion of all outstanding shares of our convertible preferred stock into shares of our common stock; (ii) the payment of an accrued dividend to holders of our convertible preferred stock in the aggregate amount of shares of our common stock and (iii) the effectiveness of our amended and restated certificate of incorporation, in each case immediately prior to the closing of this offering; and
- on a pro forma as adjusted basis to give effect to the pro forma adjustments described in the preceding clause and to reflect the issuance and sale by us of shares of common stock in this offering at an assumed initial public offering price of $ per share (which is the midpoint of the range set forth on the cover page of this prospectus), after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The pro forma as adjusted information below is illustrative only, and our capitalization following the completion of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. You should read this information in conjunction with our consolidated financial statements and the related notes included elsewhere in this prospectus and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section and other financial information contained in this prospectus.

	As of December 31, 2019		
	Actual	Pro forma	Pro Forma As adjusted
	(in thousands, except share data)		
Cash and cash equivalents	$	$	$
Convertible preferred stock, par value $0.00001 per share; shares authorized, shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted			
Preferred stock, par value $0.00001 per share; no shares authorized, issued and outstanding, actual; shares authorized, no shares issued or outstanding, pro forma and pro forma as adjusted			
Common stock, par value $0.00001 per share; shares authorized, shares issued and outstanding, actual; shares authorized, shares issued and outstanding, pro forma; shares authorized, shares issued and outstanding, pro forma as adjusted			
Additional paid-in capital			
Accumulated deficit			
Total stockholders' (deficit) equity			
Total capitalization	$	$	$

Each $1.00 increase or decrease in the assumed initial public offering price of $ per share (which is the midpoint of the price range set forth on the cover page of this prospectus) would increase or decrease each of cash and cash equivalents, total stockholders' (deficit) equity and total capitalization on a pro forma as adjusted basis by approximately $ million, assuming the number of shares offered, as set forth on the cover page of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each 1,000,000 share increase or decrease in the number of shares offered in this offering would increase or decrease each of cash and cash equivalents, total stockholders' (deficit) equity and total capitalization on a pro forma as adjusted basis by approximately $ million, assuming that the price per share for the offering remains at $ (which is the midpoint of the price range set forth on the cover page of this prospectus), and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The information in the table above excludes:

- shares of common stock issuable upon exercise of outstanding stock options granted under our Equity Incentive Plan as of December 31, 2019, at a weighted average exercise price of $ per share;
- shares of common stock available for future issuance under our Equity Incentive Plan as of December 31, 2019; and
- shares of our common stock that will become available for future issuance under our 2020 Plan, which will become effective in connection with the completion of this offering.

DIVIDEND POLICY

We currently intend to retain all available funds and any future earnings to fund the development, commercialization and growth of our business, and therefore we do not anticipate declaring or paying any cash dividends on any class of our common stock in the foreseeable future. Any future determination as to the declaration and payment of dividends, if any, will be at the discretion of our board of directors, subject to compliance with contractual restrictions and covenants in the agreements governing our current and future indebtedness. Any such determination will also depend upon our business prospects, results of operations, financial condition, cash requirements and availability and other factors that our board of directors may deem relevant. Our Credit Agreement with OrbiMed prohibits us from declaring and paying cash dividends.

The terms of our current certificate of incorporation provide that, upon the conversion of our Series A preferred stock, our Series B preferred stock and our Series C preferred stock into shares of our common stock upon the closing of this offering, each holder of our Series A preferred stock, our Series B preferred stock and our Series C preferred stock will receive a cumulative accrued dividend calculated at a rate per annum of 10% of the applicable issue price of such series of preferred stock, in each case, compounded annually, payable, at the determination of our board of directors, in either (i) shares of common stock or (ii) cash in an aggregate amount equal to the cumulative accrued dividend. We intend to pay the cumulative accrued dividend in shares of common stock. Assuming we pay the cumulative accrued dividend in shares of common stock, the cumulative accrued dividend will be issued to each holder of preferred stock as of immediately prior to the closing of this offering a number of shares of common stock equal to (x) the aggregate amount of the accrued dividend held by such holder and not previously paid as of immediately prior to the closing of this offering divided by (y) the actual price per share of common stock sold to the public in this offering. Based on the midpoint of the price range set forth on the cover page of this prospectus, we expect to issue (i) shares of our common stock for cumulative accrued dividends to holder of our Series A preferred stock, (ii) shares of our common stock for cumulative accrued dividends to holders of our Series B preferred stock and (iii) shares of our common stock to holders of our Series C preferred stock. Stock dividends will not be paid on any shares of our common stock purchased in this offering.

Accordingly, you may need to sell your shares of our common stock to realize a return on your investment, and you may not be able to sell your shares at or above the price you paid for them. See “Risk Factors—Risks Related to This Offering and Ownership of Our Common Stock—We have never paid dividends on our common stock and we do not intend to pay dividends for the foreseeable future. Consequently, any gains from an investment in our common stock will likely depend on whether the price of our common stock increases.”

DILUTION

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

Our historical net tangible book value (deficit) as of December 31, 2019 was , or $ per share of our common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities and preferred stock, which is not included within stockholders' equity (deficit). Historical net tangible book value (deficit) per share represents historical net tangible book value (deficit) divided by the shares of our common stock outstanding as of December 31, 2019.

Our pro forma net tangible book value (deficit) as of December 31, 2019 was $ million, or $ per share. Pro forma net tangible book value per share is determined by subtracting our total liabilities from the total book value of our tangible assets and dividing the difference by the number of shares of common stock deemed to be outstanding, after giving effect to (i) the conversion of all outstanding shares of our convertible preferred stock immediately prior to the closing of this offering in shares of common stock and (ii) the payment of an accrued dividend to holders of our convertible preferred stock in the aggregate amount of shares of our common stock which becomes due and payable to such holders upon the conversion of their convertible preferred stock upon the closing of this offering.

After giving further effect to our issuance and sale of shares of our common stock in this offering at an assumed initial public offering price of $ per share (which is the midpoint of the price range set forth on the cover page of this prospectus) and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of , would have been $ million, or $ per share of common stock. This amount represents an immediate increase in pro forma as adjusted net tangible book value of $ per share to our existing stockholders and an immediate dilution in pro forma as adjusted net tangible book value of $ per share to new investors purchasing shares of common stock in this offering. We determine dilution by subtracting the pro forma as adjusted net tangible book value per share after this offering from the amount of cash that a new investor paid for a share of common stock. The following table illustrates this dilution:

Assumed initial public offering price per share of common stock		$
Historical net tangible book value (deficit) per share as of December 31, 2019	$	
Increase per share attributable to the conversion of outstanding preferred stock and payment of accrued dividend		
Pro forma net tangible book value per share as of December 31, 2019 before this offering		
Increase in pro forma as adjusted net tangible book value per share attributable to investors in this offering		
Pro forma as adjusted net tangible book value per share after this offering		
Dilution per share to new common stock investors in this offering		$

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share (which is the midpoint of the price range listed on the cover page of this prospectus) would increase (decrease) the pro forma as adjusted net tangible book value per share after this offering by approximately $, and dilution in pro forma as adjusted net tangible book value per share to new investors by approximately $, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their option to purchase additional shares of our common stock in full, the pro forma as adjusted net tangible book value after the offering would be $ per share, the increase in pro forma as adjusted net tangible book value per share to existing stockholders would be $ per share and the dilution in pro forma as adjusted net tangible book value to new investors would be $ per share, in each case assuming an initial public offering price of $ per share, which is the midpoint of the price range listed on the cover page of this prospectus.

The following table summarizes, as of December 31, 2019, after giving effect to this offering, the number of shares of our common stock purchased from us, the total consideration paid, or to be paid, to us and the average price per share paid, or to be paid, by existing stockholders and by the new investors. The calculation below is based on an assumed initial public offering price of $ per share (which is the midpoint of the price range listed on the cover page of this prospectus) before deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

	Shares Purchased		Total Consideration		Average price per share
	Number	Percent	Amount	Percent	
Existing stockholders		%	$	%	$
New investors					
Total		**100%**	$	**100%**	**$**

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) the total consideration paid by new investors and the total consideration paid by all stockholders by $ million, assuming the number of shares offered by us remains the same and after deducting estimated underwriting discounts and commissions but before estimated offering expenses.

Except as otherwise indicated, the discussion and the tables above assume no exercise of the underwriters' option to purchase additional shares of our common stock and excludes:

- shares of common stock issuable upon exercise of outstanding stock options granted under our Equity Incentive Plan as of December 31, 2019, at a weighted average exercise price of $ per share;
- shares of common stock available for future issuance under our Equity Incentive Plan as of December 31, 2019; and
- shares of our common stock that will become available for future issuance under our 2020 Plan, which will become effective in connection with the completion of this offering.

To the extent any of these outstanding options are exercised, there will be further dilution to new investors. To the extent all of such outstanding options had been exercised as of , the pro forma as adjusted net tangible book value per share after this offering would be $, and total dilution per share to new investors would be $.

If the underwriters exercise their option to purchase additional shares of our common stock in full:

- the percentage of shares of our common stock held by the existing stockholders will decrease to approximately % of the total number of shares of our common stock outstanding after this offering; and
- the number of shares held by new investors will increase to , or approximately % of the total number of shares of our common stock outstanding after this offering.

SELECTED CONSOLIDATED FINANCIAL DATA

The following tables present our summary consolidated financial data. We have derived the summary consolidated statements of operations data for the year ended December 31, 2018 and 2019 and the summary consolidated balance sheet data as of December 31, 2018 and 2019 from our audited consolidated financial statements included elsewhere in this prospectus. You should read the following selected consolidated financial data in conjunction with the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements, related notes and other financial information included elsewhere in this prospectus. The selected consolidated financial data in this section is not intended to replace the consolidated financial statements and is qualified in its entirety by the consolidated financial statements, related notes and other financial information included elsewhere in this prospectus. Our historical results for any prior period are not necessarily indicative of our future results.

Consolidated Statement of Operations Data: *(U.S. dollars in thousands except share and per share data)*	**Year Ended December 31, 2018**	**Year Ended December 31, 2019**
Net product revenue	$ —	$
Cost of product sales	—	
Gross profit	—	
Operating expenses:		
Research and development	$ 12,372	$
Sales and marketing	16,861	
General and administrative	12,206	
Total operating expenses	41,439	
Operating loss	(41,439)	
Interest income (expense)	1,541	
Loss before taxes	(39,898)	
Income taxes	—	
Net loss and comprehensive loss	$ (39,898)	$
Accumulation of yield on preferred stock	(30,185)	
Net loss available to common stockholders	$ (70,083)	$
Loss per share:		
Loss per share, basic and diluted(1)	$ (0.96)	$
Weighted average number of common stock, basic and diluted	72,765,366	
Pro Forma net loss per share, basic and diluted (unaudited)(1)	—	$
Pro Forma weighted average shares of common stock outstanding, basic and diluted (unaudited)	—	

(1) See Note 12 to our financial statements for the year ended December 31, 2018 appearing at the end of this prospectus for further details on the calculation of basic and diluted net loss per share attributable to common stockholders.

Consolidated Balance Sheet Data: *(U.S. dollars in thousands except share and per share data)*	**Year Ended December 31, 2018**	**Year Ended December 31, 2019**
Cash and cash equivalents	$ 83,523	$
Working capital(1)	79,453	
Total assets	$ 89,282	$
Long-term debt, net of current portion	—	
Convertible preferred stock	324,201	
Total stockholders' (deficit) equity	(242,673)	

(1) We define working capital as current assets less current liabilities.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes thereto included elsewhere in this prospectus. This discussion contains forward-looking statements that involve risks and uncertainties. You should review the section titled "Risk Factors" and "Cautionary Note Regarding Forward-Looking Statements" for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a commercial-stage pharmaceutical company focused on developing and commercializing innovative therapies for patients with rare neurological disorders living with unmet medical needs. Our product, WAKIX (pitolisant), is a first-in-class molecule with a novel mechanism of action, or MOA, specifically designed to increase histamine signaling in the brain by binding to H_3 receptors. In August 2019, WAKIX was approved by the U.S. Food and Drug Administration, or the FDA, for the treatment of our lead indication, excessive daytime sleepiness, or EDS, in adult patients with narcolepsy, and its U.S. commercial launch was initiated in November 2019. WAKIX is the first-and-only approved product for patients with narcolepsy that is not scheduled as a controlled substance. We plan to expand the label for WAKIX in narcolepsy and expect to initiate a Phase 3 clinical trial in pediatric patients in pursuit of indications for both EDS and cataplexy, as well as to pursue pediatric exclusivity. In addition, we are evaluating our options regarding the approach to take with the FDA in pursuit of a cataplexy indication in adult patients with narcolepsy. We believe that pitolisant's ability to regulate histamine gives it the potential to provide therapeutic benefit in other rare neurological disorders that are mediated through H_3 receptors and histamine signaling. We are initially focusing on the treatment of EDS associated with Prader-Willi Syndrome, or PWS, and myotonic dystrophy type 1, or DM1. We intend to commence a Phase 2 clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS in the first half of 2020, with topline results expected in the second half of 2021. We are also planning to commence a Phase 2 clinical trial for DM1 in the second half of 2020, with topline results expected in the first half of 2022. Beyond these indications, we intend to further explore pitolisant in other rare neurological disorders in which fatigue and cognitive impairment are prominent symptoms with significant impact on daily functioning.

Pitolisant was developed by Bioprojet Société Civile de Recherche, or Bioprojet, and approved by the European Medicines Agency, or EMA, in 2016 for the treatment of narcolepsy in adult patients with or without cataplexy. We acquired an exclusive license to develop, manufacture and commercialize pitolisant in the United States pursuant to our license agreement with Bioprojet, or the Bioprojet License Agreement, in July 2017. See "—Strategic Agreements—License and Commercialization Agreement with Bioprojet" for further information regarding the Bioprojet License Agreement. Pitolisant was granted Orphan Drug Designation for the treatment of narcolepsy by the FDA in 2010. It received Breakthrough Therapy designation for the treatment of cataplexy in patients with narcolepsy and Fast Track status for the treatment of EDS and cataplexy in patients with narcolepsy in April 2018.

Our operating subsidiary, Harmony Biosciences, LLC, was formed in May 2017. We were formed in July 2017 as Harmony Biosciences II, LLC, a Delaware limited liability company, and we converted to a Delaware corporation named Harmony Biosciences II, Inc. in September 2017. In February 2020, we changed our name to Harmony Biosciences Holdings, Inc. Our operations to date have consisted of building and staffing our organization, acquiring the rights to pitolisant, raising capital, opening an Investigational New Drug, or IND, for pitolisant, initiating an Expanded Access Program, or EAP, for pitolisant for appropriate patients in the United States, preparing and submitting our pitolisant New

Drug Application, or NDA, gaining NDA approval for WAKIX for EDS in adult patients with narcolepsy, and launching and commercializing WAKIX in the United States. In addition, we have initiated or intend to initiate clinical development programs in PWS, DM1 and pediatric narcolepsy to pursue potential new indications. We have funded our operations through private placements of our convertible preferred stock as well as borrowings under a term loan agreement. We raised an aggregate of $295.0 million through offerings of our Series A and B convertible preferred stock in September 2017 and January 2018, respectively. In February 2019, we entered into a multi-draw term loan agreement with CRG Servicing LLC, or CRG, for an aggregate of $200.0 million, or the Loan Agreement of which $102.5 million was outstanding as of December 31, 2019. In August 2019, we raised an additional $50.0 million in gross proceeds from the sale of our Series C convertible preferred stock. On January 9, 2020, we entered into a credit agreement with OrbiMed Royalty & Credit Opportunities III, LP, or OrbiMed, for an aggregate of $200.0 million, or the Credit Agreement. We paid off all of our obligations under the Loan Agreement with proceeds from the Credit Agreement. As of January 2020, there was $200.0 million outstanding under the Credit Agreement.

We have generated limited revenue from product sales since inception. We expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution as well as significant expenses related to further clinical development programs with pitolisant for potential new indications. We have incurred significant operating losses since inception and expect to continue to incur significant operating losses for the foreseeable future. We recorded a net loss of $39.9 million and $ million for the years ended December 31, 2018 and 2019, respectively. As of December 31, 2018 and 2019, we had an accumulated deficit of $242.7 million and $ million, respectively.

As of December 31, 2019, our cash and cash equivalents were $ million. We believe that the expected revenue generated from sales of WAKIX, our existing cash and cash equivalents, together with the anticipated net proceeds from this offering, will enable us to fund our commercialization efforts, operating expenses, clinical trials, product development and capital requirements through the end of . See “—Liquidity and Capital Resources”.

We expect our expenses and operating losses to increase substantially as we continue to:

- commercialize WAKIX in the United States for the treatment of EDS in adult patients with narcolepsy;
- incur sales and marketing costs to support the commercialization of WAKIX and any additional product candidates;
- incur manufacturing costs for WAKIX and any additional product candidates;
- implement post-approval requirements related to WAKIX;
- actively pursue an indication for WAKIX for the treatment of cataplexy in adult patients with narcolepsy;
- acquire certain ex-U.S. rights for WAKIX from Bioprojet and subsequently seek foreign regulatory approvals for WAKIX in certain of those jurisdictions;
- conduct clinical trials in PWS and DM1;
- conduct a pediatric narcolepsy program in pursuit of an indication and extension of patent exclusivity;
- conduct additional clinical trials in pursuit of potential new indications for pitolisant;
- conduct earlier stage research and development activities for pitolisant;
- incur interest expenses in conjunction with our debt facility;

- seek regulatory approvals for pitolisant or any additional product candidates that successfully complete clinical development;
- hire additional personnel;
- invest in measures to protect and expand our intellectual property;
- acquire or in-license other assets and technologies; and
- incur additional costs associated with being a public company.

In addition, as we continue to commercialize pitolisant, we will be obligated to make certain milestone payments to the licensor. For example, previously, we made a milestone payment of $75.0 million plus an additional $2.0 million extension fee to Bioprojet in November 2019 and August 2019, respectively, for the approval of EDS in adult patients with narcolepsy. See “Business—Strategic Agreement—License and Commercialization Agreement with Bioprojet” elsewhere in this prospectus for further information regarding the Bioprojet License Agreement. Our net losses may fluctuate significantly quarterly or yearly, depending on the timing of milestone payments, clinical trials, research and development expenditures and commercialization expenses.

We may need to raise additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from sales of WAKIX, we expect to finance our operations through the sale of equity securities, debt financings or other capital resources, including potential collaborations with third parties or other strategic transactions. Adequate funding may not be available to us on acceptable terms, or at all. If we fail to raise capital or enter into such agreements as and when needed, we may have to significantly scale back or discontinue the development of pitolisant and commercialization of WAKIX, and/or one or more possible indications or delay our efforts to expand our product pipeline.

Financial Operations Overview

Revenue

We did not generate any revenue from inception until the fourth quarter of 2019. Our current product, WAKIX, was approved by the FDA for the treatment of EDS in adult patients with narcolepsy in August 2019 and became commercially available in November 2019.

Total revenue consists of net sales of WAKIX, which was commercially launched in November 2019. Net sales represent the gross sales of WAKIX less provisions for product sales discounts and allowances. At this time, these provisions include trade allowances, rebates, and discounts. Although we expect net sales to increase over time, the provisions for product sales discounts and allowances may fluctuate based on the mix of sales to different customer segments and/or changes in our accrual estimates. For further discussion of the components of Revenue see “—Critical Accounting Policies and Significant Judgments and Estimates.”

Cost of Product Sales

Cost of product sales includes manufacturing and distribution costs, the cost of the drug substance, FDA program fees, royalties due to third parties on net product sales, freight, shipping, handling, storage costs, and salaries of employees involved with production. We began capitalizing inventory upon FDA approval of WAKIX with a portion of inventory sold during the year produced prior to FDA approval and, therefore, expensed previously as research and development expense.

Research and Development Expenses

Our research and development expenses have primarily been limited to the license of the rights to pitolisant, the establishment of an Expanded Access Program, or EAP, to provide appropriate

patients with pitolisant at no cost as part of a clinical trial tracking safety, the preparation of the NDA, and the creation of a development program for new indications for pitolisant in patients with PWS, DM1 and pediatric narcolepsy. Research and development activities account for a significant portion of our operating expenses and these costs are expensed as incurred. Following the closing of this offering, we expect to significantly increase our research and development efforts as we continue to pursue an indication for the treatment of cataplexy in adult patients with narcolepsy, conduct clinical trials in patients with PWS, DM1 and pediatric narcolepsy, and continue to expand our product-candidate pipeline. Research and development expenses include:

- employee-related expenses, such as salaries, share-based compensation, benefits and travel expenses for our research and development personnel;
- direct third-party costs such as expenses incurred under agreements with contract research organizations, or CROs, and contract manufacturing organizations, or CMOs;
- manufacturing costs in connection with producing materials for use in conducting preclinical studies and clinical trials;
- other third-party expenses directly attributable to the development of our product candidates; and
- amortization expense for assets used in research and development activities.

We currently have one product, WAKIX, and do not currently track our internal research and development expenses on an indication-by-indication basis as they primarily relate to personnel, early research and consumable costs, which are deployed across multiple programs. A significant portion of our research and development costs are external costs, such as fees paid to consultants, central laboratories, contractors, CMOs and CROs in connection with our clinical development activities.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials, milestone payments, NDA process and pre-launch planning. We expect our research and development expenses to be significant over the next several years as we advance our current clinical development programs and prepare to seek regulatory approval for additional product candidates.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of any additional product candidates that we develop from our programs. We have begun to generate net cash inflows from sales of WAKIX; however we are unable to predict when if ever we would generate material cash inflows from WAKIX or other product candidates we develop, if at all. This is due to the numerous risks and uncertainties associated with developing product candidates, including uncertainty related to:

- the duration, costs and timing of clinical trials of our current and future indication expansion programs and new product candidates;
- successful completion of preclinical studies and clinical trials;
- sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials;
- receiving Bioprojet’s consent to pursue additional indications for pitolisant;
- acceptance of INDs for our planned clinical trials or future clinical trials;
- successful and timely enrollment and completion of clinical trials;
- successful data from our clinical program that supports an acceptable risk-benefit profile of our product candidates in the intended populations;

- receipt and maintenance of regulatory and marketing approvals from applicable regulatory authorities;
- establishing agreements with third-party manufacturers for clinical supply for our clinical trials and commercial manufacturing, if our product candidate is approved;
- entry into collaborations to further the development of our product candidates;
- obtaining and maintaining patent and trade secret protection or regulatory exclusivity for our product candidates; and
- successfully launching our product candidates and achieving commercial sales, if and when approved.

A change in the outcome of any of these variables with respect to the development of any of our programs or any product candidate we develop would significantly change the costs, timing and viability associated with the development of such program or product candidate.

Sales and Marketing Expenses

Our sales and marketing expenses have primarily been limited to the market development and launch activities of WAKIX for EDS in adult patients with narcolepsy. Market development and commercial launch activities account for a significant portion of the overall company operating expenses and are expensed as they are incurred. We expect our sales and marketing expenses to increase in the near- and mid-term to support our EDS in adult patient with narcolepsy indication and expand our portfolio with the anticipated growth from potential additional indications and assets. Sales and marketing expenses include:

- employee-related expenses, such as salaries, share-based compensation, benefits and travel expenses for our sales and marketing personnel;
- healthcare professional-related expenses, including marketing programs, healthcare professional promotional medical education, disease education, conference exhibits and market research;
- patient-related expenses, including patient awareness and education programs, disease awareness education, patient reimbursement programs, patient support services and market research;
- market access expenses, including payer education, and services to support the continued commercialization of WAKIX; and
- secondary data purchases (i.e. patient claims and prescription data), data warehouse development and data management.

In addition, these expenses include external costs such as website development, media placement fees, agency fees for patient, medical education and promotional expenses, market research and analysis secondary data expenses, conference fees, consulting fees and travel expenses.

General and Administrative Expenses

General and administrative expenses consist primarily of employee-related expenses, such as salaries, share-based compensation, benefits and travel expenses for our personnel in executive, legal, finance and accounting, human resources, and other administrative departments. General and administrative expenses also consist of office leases, interest expenses, and professional fees, including legal, tax and accounting and consulting fees.

We anticipate that our general and administrative expenses will increase in the future to support our continued commercialization efforts, ongoing and future potential research and development activities, and increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees paid to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with the requirements of and the Securities and Exchange Commission, or the SEC, insurance and investor relations costs. If any of our current or future indication expansion programs or new product candidates obtains U.S. regulatory approval, we expect that we would incur significantly increased expenses associated with building a sales and marketing team.

Paragon Agreements

We are party to a management services agreement, or the Management Services Agreement, with Paragon Biosciences, LLC, or Paragon, entered into on September 22, 2017, pursuant to which Paragon provides to us certain professional services. In exchange for services provided to us under the Management Services Agreement, we pay to Paragon a management fee of $0.3 million per each calendar month. This fee is reduced to $0.2 million per each calendar month following September 22, 2020. We intend to terminate the Management Services Agreement upon the consummation of this offering. Upon termination, we will owe Paragon a termination fee of $ million. See “Certain Relationships and Related Party Transactions—Related Party Agreements in Effect Prior to this Offering—Management and Other Agreements” for further information.

We are also party to a right of use agreement with Paragon whereby we have access to and the right to use certain office space leased by Paragon in Chicago, Illinois. For the year ended December 31, 2019, we paid a fee of $ million pursuant to this agreement.

Interest Income / Interest Expense

Interest income (expense), net consists primarily of interest expenses on outstanding line of credit balances and is offset by interest income earned on our cash balances.

Results of Operations

Comparison of the Years Ended December 31, 2018 and 2019

The following table sets forth our results of operations for the years ended December 31, 2018 and 2019.

	Year Ended December 31, 2018	Year Ended December 31, 2019	Change	
			Amount	%
		(*dollars in thousands*)		
Net product revenue	$ —	$	$	
Cost of product sales	—			
Gross profit	—			
Operating expenses:				
Research and development	$ 12,372	$	$	
Sales and marketing	16,861			
General and administrative	12,206			
Total operating expenses	41,439			
Other income (expense)	1,541			
Loss before provision for income taxes	(39,898)			
Provision for income taxes	—			
Net loss	$ (39,898)	$	$	

Net Product Revenue

Net product revenue was $ million for the year ended December 31, 2019, following the commercial launch of WAKIX on November 1, 2019.

Cost of Product Sales

Cost of product sales were $ million for the year ended December 31, 2019, following the commercial launch of WAKIX on November 1, 2019.

Research and Development Expenses

Research and development expenses were $ million for the year ended December 31, 2019, compared to $12.4 million for the year ended December 31, 2018, a of $ million, or %.

The in research and development expenses is attributable to .

Research and development expenses consisted of for the year ended December 31, 2019. Research and development expenses consisted of personnel expenses of $4.7 million, professional fees and services of $3.9 million, clinical supply purchases for our EAP of $1.9 million, tablet development expenses of $1.0 million and other expenses of $0.9 million for the year ended December 31, 2018.

Sales and Marketing Expenses

Sales and marketing expenses were $ million for the year ended December 31, 2019, compared to $16.9 million for the year ended December 31, 2018, a of $ million, or %.

The in sales and marketing expenses is attributable to .

Sales and marketing expenses consisted of for the year ended December 31, 2019. Sales and marketing expenses consisted of personnel expenses of $6.0 million, professional fees of $3.6 million, promotional and patient activities of $3.1 million and public relations, external communications, unbranded awareness, website development and digital marketing of $2.4 million and other expenses of $1.8 million for the year ended December 31, 2018.

General and Administrative Expenses

General and administrative expenses were $ million for the year ended December 31, 2019, compared to $12.2 million for the year ended December 31, 2018, a of $ million, or %.

The in general and administrative expenses is attributable to .

General and administrative expenses consisted of for the year ended December 31, 2019. General and administrative expenses consisted of personnel expenses of $4.9 million, a management fee to Paragon of $4.0 million, professional fees of $1.8 million, and rent, facility and other expenses of $1.5 million for the year ended December 31, 2018.

Interest Income (Expense), Net

Interest income was $ million for the year ended December 31, 2019, compared to $1.5 million for the year ended December 31, 2018, a of $ million, or %.

The increase in interest income (expense) is attributable to us entering into the Loan Agreement with CRG and the payment of interest expense thereunder.

Income Taxes

At December 31, 2018 and 2019, we had federal net operating loss, or NOL, carryforwards of $12.1 million and $ million, respectively, with no expiration. At December 31, 2018 and 2019 we had state NOL carryforwards of $4.6 million and $ million, respectively, with a 20-year expiration. In light of these considerations as well as uncertainty as to when we might generate taxable income, we have recorded a full valuation allowance of $ million as of December 31, 2019. The amount of the net deferred tax asset considered realizable could be adjusted in the future based on changes in positive and negative evidences subject to evaluation, including estimates of taxable income.

Liquidity and Capital Resources

Overview

To date, we have financed our operations primarily with proceeds from sales of our convertible preferred stock and borrowings under (i) our Loan Agreement with CRG and (ii) our Credit Agreement with OrbiMed. From our inception through December 31, 2019, we have received aggregate proceeds of $345.0 million from sales of our Series A convertible preferred stock, Series B convertible preferred stock and Series C convertible preferred stock. As of December 31, 2019, we had cash, cash equivalents and restricted cash of $ million and accumulated deficit of $ million. As of December 31, 2018 we had no outstanding debt and as of December 31, 2019, we had outstanding debt of $ million.

On February 28, 2019, we entered into the Loan Agreement with CRG for an aggregate of $200.0 million of which $102.5 million was outstanding at December 31, 2019. On January 9, 2020, we

entered into the Credit Agreement with OrbiMed for an aggregate of $200.0 million and paid off all of our obligations under the Loan Agreement. The Credit Agreement matures on January 9, 2026 and bears an interest rate of the greater of (a) LIBOR or (b) 2.00% per annum, plus 11.00% per annum. When the LIBOR rate is no longer used post-2021, the Prime Rate will be used in the determination of the interest rate. The Credit Agreement requires compliance with certain financial covenants, including minimum net revenue thresholds and cash balance requirements (which include maintaining minimum liquidity of $12.5 million), and financial reporting requirements. The Credit Agreement contains certain negative restrictive covenants that either limit our ability to, or require a mandatory prepayment in the event we, engage in new lines of business, incur additional indebtedness or liens, make certain investments, make certain payments, pay cash dividends, merge with other companies or consummate certain changes of control, acquire other companies, transfer or dispose of certain assets, liquidate or dissolve, amend certain material agreements, enter into sale and leaseback transactions, enter into various other specified transactions, and change our name, location, executive office or executive management without notice.

To date, we have drawn a total of $200.0 million under the Credit Agreement, all of which is outstanding.

We currently estimate that we will use the net proceeds from this offering to fund the clinical development of additional indications for pitolisant in PWS, DM1 and pediatric narcolepsy, and for working capital, business development opportunities, potential milestone payments to Bioprojet and general corporate purposes, including to support the continued commercialization of WAKIX in the United States. We may need additional funding to complete the clinical development of, seek regulatory approval for and commercially launch future potential indications for pitolisant.

Until such time as we generate substantial product revenue from sales of any of our current or future product candidates, we may finance our cash needs through a combination of equity offerings, debt financings and potential collaboration, license or development agreements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common shareholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. Adequate additional funding may not be available to us on acceptable terms, or at all. If we are unable to raise capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates, grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves or potentially discontinue operations.

Cash Flows

The following table sets forth a summary of our cash flows for the years ended December 31, 2018 and 2019:

	Year Ended December 31, 2018	Year Ended December 31, 2019
	(in thousands)	
Net cash (used in) operating activities	$ (38,799)	$
Net cash (used in) investing activities	(1,342)	
Net cash provided by financing activities	21,615	
Net increase/(decrease) in cash, cash equivalents and restricted cash	$ (18,526)	$

Operating Activities

For the year ended December 31, 2019, $ million of cash was used in operating activities, compared to $39.0 million for the year ended December 31, 2018, a of $ million, or %.

The in cash used in operating activities was primarily attributable to .

Cash used in operating activities consisted of in 2019. Cash used in operating activities consisted of sales and marketing, research and development and general and administrative expenditures in 2018.

Investing Activities

For the year ended December 31, 2019, $ million of cash was used in investing activities, compared to $1.3 million for the year ended December 31, 2018, a of $ million, or %.

The in cash used in investing activities is attributable to .

Cash used in investing activities consisted of in 2019. Cash used in investing activities consisted of the purchase of property and equipment for our new corporate headquarters in 2018.

Financing Activities

For the year ended December 31, 2019, cash provided by financing activities was $ million, compared to $22.0 million for the year ended December 31, 2018, a of $ million, or %.

The in cash provided by financing activities is attributable to .

Cash provided by financing activities consisted of in 2019. Cash provided by financing activities consisted of $25.0 million in proceeds from the issuance of our Series A and Series B Preferred Stock, offset by a $3.2 million repurchase of common stock from our former chief executive officer in 2018.

Outlook

Based on the expected net proceeds from this offering, our research and development plans and our timing expectations related to the development of our clinical programs to pursue indications for PWS, DM1 and pediatric narcolepsy, we believe that the expected revenue generated from sales of WAKIX, our existing cash and cash equivalents, together with the anticipated net proceeds from this

offering will enable us to fund our operating expenses, clinical development, sales and marketing, interest expense and capital expenditure requirements into . However, we have based this estimate on assumptions that may prove to be incorrect, and we could use our capital resources sooner than we expect.

The amount and timing of future funding requirements will depend on many factors, including, but not limited to:

- the success of our commercialization of WAKIX for EDS in adult patients with narcolepsy;
- the effect of competing technological and market developments;
- the cost and timing of manufacturing activities;
- the payment of licensing fees and potential milestone payments to Bioprojet;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA and other regulatory authorities;
- the potential expansion of our current development programs to seek new indications for pitolisant, potential new development programs for additional indications, and related general and administrative support;
- the initiation, progress, timing, and results of our clinical trials through all phases of development for pitolisant as a treatment for other indications and any other product candidates;
- the willingness of the FDA and other comparable regulatory authorities to accept our clinical trial designs, as well as data from our completed and planned clinical trials and preclinical studies and other work, as the basis for the review and approval of pitolisant for other potential indications or of any other product candidates;
- the cost of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights, in-licensed or otherwise;
- the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us for pitolisant or future product candidates;
- the cost of acquiring rights to other pharmaceutical products in the future to further develop and commercialize;
- the cost of general operating expenses;
- the cost of interest expense in conjunction with our debt facility;
- the cost of sales, marketing and distribution capabilities for WAKIX and the cost of establishing our sales and marketing our product candidates in regions where those product candidates are approved and where we choose to commercialize our products on our own; and
- the costs of operating as a public company.

Contractual Obligations and Commitments

As of December 31, 2019, our commitments consisted of operating leases for our corporate headquarters in Plymouth Meeting, Pennsylvania, for approximately 15,651 square feet, and office space in Chicago, Illinois. The following table summarizes our contractual obligations as of December 31, 2019.

	Payments Due by Period				
	Total	Less Than One Year	1–3 Years	3–5 Years	More Than Five Years
			(*in thousands*)		
Operating lease obligations	$	$	$	$	—

Under the Bioprojet License Agreement, we have obligations that are contingent upon future events such as our achievement of regulatory and commercial milestones and are required to make royalty and trademark payments in connection with the sale of products. In February 2019, we achieved one of our regulatory milestones, FDA file acceptance, and as a result, a milestone payment of $50.0 million was due to Bioprojet and was paid in February 2019. Further, upon achieving FDA approval for WAKIX for the treatment of EDS in adult patients with narcolepsy, we paid Bioprojet an FDA approval milestone payment of $75.0 million in November 2019 and an additional payment of $2.0 million in August 2019. As of December 31, 2019, we were unable to estimate the timing and likelihood of achieving the milestones or making future product sales and, therefore, any related payments are not included in the table above. See the section titled “Business—License Agreement with Bioprojet” for additional information regarding our license agreement with Bioprojet.

We enter into contracts in the normal course of business with clinical trial sites and clinical supply manufacturers and other services and products for operating purposes. These contracts generally provide for termination after a notice period, and, therefore, are cancelable contracts and not included in the table above.

Going Concern

The consolidated financial statements have been prepared as though we will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. We have incurred operating losses and negative cash flows from operations since inception. As of December 31, 2018 and 2019, we have an accumulated deficit of $242.7 million and $ million, respectively. Management expects to continue to incur operating losses and negative cash flows from operations for the foreseeable future. In addition, we are subject to potential milestone payments associated with a license agreement with Bioprojet, of between $40.0 million and $142.0 million. We have financed our operations to date with proceeds from the sale of preferred securities and drawing down on our Loan Agreement.

We will need to raise additional capital in order to continue to fund operations, including milestone obligations under the Bioprojet License Agreement. We believe we will be able to obtain additional capital through equity financings or other arrangements to fund operations; however, there can be no assurance that such additional financing, if available, can be obtained on acceptable terms. If we are unable to obtain such additional financing, future operations would need to be scaled back or discontinued.

Accordingly, these factors raise substantial doubt about our ability to continue as a going concern within one year after the date the consolidated financial statements are issued. The consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might be necessary should we be unable to continue as a going concern.

Off-Balance Sheet Arrangements

For the years ended December 31, 2018 and 2019, we did not have any off-balance sheet arrangements, as defined under SEC rules.

Critical Accounting Policies and Significant Judgments and Estimates

Our management’s discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America, or U.S. GAAP. The preparation of these financial

statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the dates of the balance sheets and the reported amounts of expenses during the reporting periods. In accordance with U.S. GAAP, we evaluate our estimates and judgments on an ongoing basis. Significant estimates include assumptions used in the determination of some of our costs incurred under our Services Agreement and which costs are charged to research and development and general and administrative expense. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

We define our critical accounting policies as those under U.S. GAAP that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. While our accounting policies are more fully described in Note 3 to our consolidated financial statements appearing elsewhere in this prospectus, we believe the following are the critical accounting policies used in the preparation of our consolidated financial statements that require significant estimates and judgments.

Revenue Recognition

Effective January 1, 2019, we adopted Accounting Standards Codification, or ASC, 606. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract and determine those that are performance obligations. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied. We have determined that the delivery of our product to our customer constitutes a single performance obligation as there are no other promises to deliver goods or services. Shipping and handling activities are considered to be fulfilment activities and are not considered to be a separate performance obligation. We have assessed the existence of a significant financing component in the agreements with our customers. The trade payment terms with our customers do not exceed one year and therefore, no amount of consideration has been allocated as a financing component. Taxes collected related to product sales are remitted to governmental authorities and are excluded from revenue.

Product Sales, Net

We began commercial sales of WAKIX in November 2019. We sell WAKIX to our customers (a limited number of specialty distributors) that, in turn, distribute WAKIX to patients.

We recognize revenue on sales of WAKIX when the customer obtains control of the product, which occurs at a point in time, typically upon delivery. Product revenues are recorded at the product's

wholesale acquisition costs, net of applicable reserves for variable consideration that are offered within contracts between us and our customers, payors, and other indirect customers relating to the sale of WAKIX. Components of variable consideration include government and commercial contracts, product returns, commercial co-payment assistance program transactions, and distribution service fees. These deductions, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as a current liability or reduction of receivables, based on the expected value method and a range of outcomes and are probability weighted in accordance with ASC 606.

The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. Our analyses contemplate the application of the constraint in accordance with ASC 606. For the twelve months ended December 31, 2019, we determined a material reversal of revenue would not likely occur in a future period for the rebate estimates detailed below and, therefore, the transaction price was not reduced further. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

Government Contracts

We have entered into contracts (i) to participate in the Medicaid Drug Rebate Program and the Medicare Part D program, and (ii) to sell to the U.S. Department of Veterans Affairs, 340b entities and other government agencies, or Government Payors, so that WAKIX will be eligible for purchase by, in partial or full reimbursement from, such Government Payors. These reserves are recorded in the same period the revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability, which is included in accounts payable or accrued expenses. For Medicare Part D, we estimate the number of patients in the prescription drug coverage gap for whom we will owe a payment under the Medicare Part D program.

We estimate the rebates that we will provide to Government Payors for those programs that require rebates. These rebate estimates are based upon (i) the government-mandated discounts applicable to government-funded programs, (ii) information obtained from its customers and (iii) information obtained from other third parties regarding the payor mix for WAKIX. The liability for these rebates consists of estimates of claims for the current year and estimated future claims that will be made for product shipments that have been recognized as revenue but remain in the distribution channel inventories at the end of each reporting period.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed and some require advanced payments. We make estimates of our accrued expenses of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses include fees paid to:

- CROs in connection with performing research services on our behalf and any clinical trials;
- investigative sites or other providers in connection with studies and any clinical trials;

- vendors in connections with the preparation of our NDA file, market and patient awareness programs, website development, market research and analysis and medical education;
- vendors related to product manufacturing, development and distribution of clinical supplies.

We base our expenses for services rendered on our estimates of the services received and efforts expended pursuant to quotes, contracts and communicating with our vendors. The financial terms of these agreement are subject to negotiation, vary from contract to contract and may result in uneven payments. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or amount of prepaid or accrued expenses accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low in any particular period. To date, we have not made any material adjustments to our prior estimates of accrued research and development expenses.

Stock-Based Compensation

We recognize stock-based compensation expense related to stock options granted to employees based on the estimated fair value of the awards on the date of grant. We estimate the grant date fair value, and the resulting stock-based compensation expense, for stock options that only have service vesting requirements or performance-based vesting requirements without market conditions using the Black-Scholes option-pricing model. The grant date fair value of the stock-based awards with service vesting requirements is generally recognized on a straight-line basis over the requisite service period, which is generally the vesting period of the respective awards. Determining the appropriate amount to expense for performance-based awards based on the achievement of stated goals requires judgment. The estimate of expense is revised periodically based on the probability of achieving the required performance targets and adjustments are made as appropriate. The cumulative impact of any revisions is reflected in the period of change. If any applicable financial performance goals are not met, no compensation cost is recognized and any previously recognized compensation cost is reversed.

We recognize stock-based compensation expense related to stock options granted to non-employees issued in exchange for services based on the estimated fair value of the awards on the date of grant. We estimate the grant date fair value, and the resulting share-based compensation expense, using the Black-Scholes option-pricing model; however, the fair value of the stock options granted to non-employees is remeasured each reporting period until the service is complete, and the resulting increase or decrease in value, if any, is recognized as expense or a reduction in previously recognized expense, respectively, during the period the related services are rendered.

The Black-Scholes option-pricing model requires the use of highly subjective assumptions, which determine the fair value of share-based awards. These assumptions include:

Expected term. Our expected term represents the period that our stock-based awards are expected to be outstanding and is determined using the simplified method (based on the mid-point between the vesting date and the end of the contractual term). For stock-based awards granted to non-employees, the expected term represents the contractual term of the award.

Common stock price. Our board of directors estimates the fair value of our common stock. Given the absence of a public trading market for our common stock, and in accordance with the American

Institute of Certified Public Accountants' Practice Guide, Valuation of Privately Held-Company Equity Securities Issued as Compensation, our board of directors exercises reasonable judgment and considers a number of objective and subjective factors to determine its best estimate of the fair value of our common stock, as further described below under "—Common Stock Valuations."

Expected volatility. Prior to this offering, we were a privately held company and did not have any trading history for our common stock and the expected volatility was estimated using weighted-average measures of implied volatility and the historical volatility of our peer group of companies for a period equal to the expected life of the stock options. Our peer group of publicly traded biopharmaceutical companies was chosen based on their similar size, stage in the life cycle or area of specialty.

Risk-free interest rate. The risk-free interest rate is based on the rates paid on securities issued by the U.S. Treasury with a term approximating the expected life of the stock options.

Expected dividend. We have never paid, and do not anticipate paying, cash dividends on our common stock. Therefore, the expected dividend yield was assumed to be zero.

The following table reflects the weighted average assumptions used to estimate the fair value of options granted during the periods presented.

	2019	2018
Dividend yield		0.00%
Expected volatility		112.00%
Risk-free interest rate		2.39%
Lack of marketability discount		43.00%
Expected term (years)		6.50

Common Stock Valuations

Historically, for all periods prior to this initial public offering, the fair values of the shares of common stock underlying our stock-based awards were determined on each grant date by our board of directors. Given the absence of a public trading market for our common stock, our board of directors exercised reasonable judgment and considered a number of objective and subjective factors to determine the best estimate of the fair value of our common stock, including our stage of development; our actual operating results and financial performance; the progress of our commercialization research and development efforts; conditions in the industry and economy in general; the rights, preferences and privileges of our convertible preferred stock relative to those of our common stock; the likelihood of achieving a liquidity event for the holders of our common stock, such as an initial public offering or a sale of our company, given prevailing market conditions; equity market conditions affecting comparable public companies; the lack of marketability of our common stock and the results of independent third party valuations. Our board of directors also took into consideration the valuations of our common stock that were prepared by an independent third-party valuation firm in accordance with the guidance provided by the American Institute of Certified Public Accountants 2013 Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation.

For our valuations performed prior to December 31, 2019, we used the Option Pricing Model Backsolve method to estimate the fair value of our common stock. In an option pricing method, or OPM, framework, the backsolve method for inferring the equity value implied by a recent financing transaction involves making assumptions for the expected time to liquidity, volatility and risk-free rate and then solving for the value of equity such that value for the most recent financing equals the amount paid. Furthermore, as of each of the valuation dates prior to December 31, 2019 and even being a late stage development company, the future liquidity events were difficult to forecast. We applied a discount for lack of marketability to account for a lack of access to an active public market.

Our common stock valuations prior to December 31, 2019 resulted in valuations, as of December 31, 2017, December 31, 2018, February 13, 2019, August 14, 2019 and December 31, 2019, of $0.40, $0.40, $0.50, $0.62 and $, respectively, per share. All historic option grants were made above such valuations at an exercise price of $1.00 per share.

After the closing of this offering, our board of directors will determine the fair value of each common share underlying share-based awards based on the closing price of our common shares as reported on the date of grant on the primary stock exchange on which our common stock is traded.

Based upon the initial public offering price of $ per common share, the aggregate intrinsic value of outstanding options to purchase our common shares as of , 2020 was $ million, all of which are related to unvested options.

Income Taxes

We provide for income taxes under the asset and liability method. Current income tax expense or benefit represents the amount of income taxes expected to be payable or refundable for the current year. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and the respective tax bases. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date.

When uncertain tax positions exist, we recognize the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2018 and 2019, we did not have any significant uncertain tax positions.

As of December 31, 2018, our total deferred tax assets were $59.0 million. Due to our lack of earnings history and uncertainties surrounding our ability to generate future taxable income, the net deferred tax assets have been fully offset by a valuation allowance. The deferred tax assets were primarily comprised of federal and state tax NOL carryforwards. We had a Series A preferred stock financing in September 2017, a Series B preferred stock financing in January 2018 and a Series C in August 2019. We have not completed a Section 382 study to assess whether an ownership change has occurred or whether there have been multiple ownership changes since our formation due to the complexity and cost associated with such a study and the fact that there may be additional such ownership changes in the future. Pursuant to Sections 382 and 383 of the Internal Revenue Code, annual use of our net operating loss and research and development tax credit carryforwards may be limited in the event a cumulative change in ownership of more than 50% occurs within a three-year period.

In the United States, on December 22, 2017, the Tax Cuts and Jobs Act, or the Tax Act, was signed into law. Substantially all of the provisions of the Tax Act are effective for taxable years beginning after December 31, 2017. The Tax Act includes significant changes to the Code, including amendments that significantly change the taxation of individuals and business entities. The Tax Act contains numerous provisions that might impact us, the most significant of which reduces the federal corporate statutory tax rate from 34% to 21%.

Recent Accounting Pronouncements

See Note 3 to our financial statements included elsewhere in this prospectus for more information.

The JOBS Act

We are an “emerging growth company”, or EGC, as defined in the Jumpstart Our Business Startups Act, or JOBS Act, of 2012. Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies.

We have elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date we (i) are no longer an EGC or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, our financial statements may not be comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates. If we were to subsequently elect instead to comply with these public company effective dates, such election would be irrevocable pursuant to the JOBS Act.

We will remain an EGC until the earliest of (i) the last day of our fiscal year (a) following the fifth anniversary of the completing of this offering, (b) in which we have total annual gross revenues of at least $1.07 billion or (ii) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th and (iii) the date on which we have issued more than $1.0 billion in non-convertible debt securities over a three-year period.

Quantitative and Qualitative Disclosures About Market Risk

Interest Rate Fluctuation Risk

We are exposed to market risk related to changes in interest rates. As of December 31, 2019, our cash and cash equivalents consisted of cash and money market accounts. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

As of December 31, 2019, we had $102.5 million in borrowings outstanding. In January 2020, we closed on the Credit Agreement with OrbiMed and drew down $200.0 million upon closing. The term loan bears interest at an interest rate of the greater of (a) LIBOR or (b) 2.00% per annum, plus 11.00% per annum. Based on the $200.0 million of principal outstanding as of January 2020, an immediate 10% change in the Prime Rate would not have a material impact on our debt-related obligations, financial position or results of operations.

Foreign Currency Fluctuation Risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with foreign vendors that are located in Europe. Our operations may be subject to fluctuations in foreign currency exchange rates in the future.

Inflation Fluctuation Risk

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations for the years ending December 31, 2018 and 2019.

BUSINESS

Overview

We are a commercial-stage pharmaceutical company focused on developing and commercializing innovative therapies for patients with rare neurological disorders living with unmet medical needs. Our product, WAKIX (pitolisant), is a first-in-class molecule with a novel mechanism of action, or MOA, specifically designed to increase histamine signaling in the brain by binding to H_3 receptors. In August 2019, WAKIX was approved by the U.S. Food and Drug Administration, or the FDA, for the treatment of our lead indication, excessive daytime sleepiness, or EDS, in adult patients with narcolepsy, and its U.S. commercial launch was initiated in November 2019. WAKIX is the first-and-only approved product for patients with narcolepsy that is not scheduled as a controlled substance. We plan to expand the label for WAKIX in narcolepsy and expect to initiate a Phase 3 clinical trial in pediatric patients in pursuit of indications for both EDS and cataplexy, as well as to pursue pediatric exclusivity. In addition, we are evaluating our options regarding the approach to take with the FDA in pursuit of a cataplexy indication in adult patients with narcolepsy. We believe that pitolisant's ability to regulate histamine gives it the potential to provide therapeutic benefit in other rare neurological disorders that are mediated through H_3 receptors and histamine signaling. We are initially focusing on the treatment of EDS associated with Prader-Willi Syndrome, or PWS, and myotonic dystrophy type 1, or DM1. We intend to commence a Phase 2 clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS in the first half of 2020, with topline results expected in the second half of 2021. We are also planning to commence a Phase 2 clinical trial for DM1 in the second half of 2020, with topline results expected in the first half of 2022. Beyond these indications, we intend to further explore pitolisant in other rare neurological disorders in which fatigue and cognitive impairment are prominent symptoms with significant impact on daily functioning.

Pitolisant was developed by Bioprojet Société Civile de Recherche, or Bioprojet, and approved by the European Medicines Agency, or EMA, in 2016 for the treatment of narcolepsy in adult patients with or without cataplexy. We acquired an exclusive license to develop, manufacture and commercialize pitolisant in the United States pursuant to our license agreement with Bioprojet, or the Bioprojet License Agreement, in July 2017. See "—Strategic Agreement—License and Commercialization Agreement with Bioprojet" for further information regarding the Bioprojet License Agreement. Pitolisant was granted Orphan Drug Designation for the treatment of narcolepsy by the FDA in 2010. It received Breakthrough Therapy designation from the FDA for the treatment of cataplexy in patients with narcolepsy and Fast Track status for the treatment of EDS and cataplexy in patients with narcolepsy in April 2018.

Narcolepsy Market Overview

Narcolepsy is a rare, chronic and debilitating neurologic disorder of sleep-wake state instability that is estimated to affect anywhere from 135,000 to 200,000 Americans, with fewer than 50% diagnosed. Narcolepsy is characterized by EDS, which is present in all patients with narcolepsy and is the primary reason why patients seek treatment. EDS is the inability to stay awake or alert throughout the day, including an irrepressible need for sleep, with lapses into drowsiness or sleep, which has a significant impact on a patient's ability to function. Additional symptoms of narcolepsy may include cataplexy (which is characterized by sudden and transient episodes of muscle weakness accompanied by full conscious awareness), hallucinations, sleep paralysis and disrupted nighttime sleep. In most patients, narcolepsy is caused by the loss of hypocretin, a neuropeptide in the brain that, along with histamine, works to support sleep-wake state stability. This disorder affects men and women equally, with typical symptom onset in adolescence or young adulthood; however, it can take up to a decade after onset of symptoms to be properly diagnosed. The U.S. narcolepsy market had an approximate

net sales value of $1.8 billion in 2019. The market is expected to continue to grow based on several factors, including, but not limited to, the introduction of new innovative therapies that offer novel mechanisms of action resulting in improved safety/tolerability profiles while delivering clinically meaningful efficacy, additional investment in education, increased rates of diagnosis, and population growth.

Prior to the approval of WAKIX, there were six approved medications to treat patients with narcolepsy, all of which are scheduled as controlled substances: Xyrem (sodium oxybate), Provigil (modafinil), Nuvigil (armodafinil), methylphenidate, amphetamine and Sunosi (solriamfetol). Other prescription drugs are used off-label for the treatment of either EDS or cataplexy in patients with narcolepsy, including stimulants and antidepressants. Some of the current therapies have significant side effects (such as increased heart rate and blood pressure) and boxed warnings due to the risk of respiratory depression, abuse and dependence. These therapies also have the potential for rebound and withdrawal symptoms. According to the 2007 American Academy of Sleep Medicine treatment guidelines, medications for narcolepsy, at best, provided only moderate improvement in narcolepsy symptoms and side effects may limit their use. The Voice of the Patient report from the FDA's patient-focused drug development initiative, published in 2014, concluded that, based on the overall benefit-risk assessment of current medications, there is a continued need for additional effective and tolerable treatment options for patients with narcolepsy. In a retrospective electronic chart review conducted on our behalf from June 2011 to December 2018, over 75% (73 out of 97 respondents) of patients with narcolepsy reported at least one residual symptom while on their current treatment. In a third party survey that we commissioned prior to the commercialization of WAKIX, of the 200 patients with narcolepsy who were surveyed, 93% (157 out of 169 respondents) expressed frustration with current treatment options, while 31 patients were not on treatment and, as such, did not provide a response to this question. The main drivers of patients' dissatisfaction were side effects and tolerability, loss of efficacy over time and concerns about abuse and dependence with current therapies. In 2019, two new therapies for narcolepsy, including WAKIX, were approved by the FDA, which represent the first new therapies for narcolepsy patients in the United States since 2007.

In market research sponsored by us prior to the commercial release of WAKIX, both patients and healthcare professionals, or HCPs, expressed frustration and dissatisfaction with then-existing therapies, reflecting current unmet medical needs. These unmet needs included, in order of importance, the availability of: (i) non-scheduled treatment options, (ii) more tolerable treatment regimens, (iii) more effective treatment options, (iv) novel MOAs beyond currently available therapies and (v) once-daily treatment options. Based on our market research, we believe the most significant unmet need identified was the availability of non-scheduled treatment options. Other than WAKIX, all drugs approved by the FDA for the treatment of narcolepsy, including stimulants, are scheduled as controlled substances by the DEA. Controlled substances have the potential for abuse, misuse, diversion. In addition, these products also have the potential for the development of tolerance and withdrawal symptoms. Despite their inherent drawbacks, due to the limited number of treatment options, stimulants have historically been a primary treatment for people with narcolepsy. In addition to having the potential for abuse, all of the treatments approved for narcolepsy, except WAKIX, require a Risk Evaluation and Mitigation Strategy, or REMS, program, which is required by the FDA for certain medications with serious safety concerns to help ensure the benefits of the medication outweigh its risks.

Our Solution

WAKIX (pitolisant) represents a novel approach to narcolepsy treatment. We believe that WAKIX offers a meaningfully differentiated product profile over current treatment options for the following reasons:

- ***First-in-class molecule with a novel MOA***. WAKIX is the only selective H_3 receptor antagonist/inverse agonist approved by the FDA for the treatment of EDS in adult patients with

narcolepsy and is the only narcolepsy treatment that works primarily through histamine, a major wake-promoting neurotransmitter. Pitolisant is thought to work by regulating histamine, such that it activates wake-promoting neurons and inhibits sleep promoting neurons, which helps to stabilize states of sleep and wakefulness. We believe that these novel characteristics differentiate it from other narcolepsy treatments.

- ***First-and-only non-scheduled treatment for narcolepsy.*** WAKIX is the first-and-only FDA-approved treatment for narcolepsy that is not scheduled as a controlled substance by the U.S. Drug Enforcement Administration, or the DEA. We believe one of the most significant unmet needs is the availability of non-scheduled treatment options. In a clinical trial, pitolisant demonstrated statistically significantly lower drug liking compared to phentermine (a Schedule IV stimulant), consistent with its lack of abuse potential.
- ***WAKIX is not a stimulant***. Stimulants are one of the most commonly prescribed treatments for patients with narcolepsy. Unlike stimulants, WAKIX has shown no evidence for the development of drug tolerance or withdrawal symptoms. Therefore, there is no need for patients to temporarily stop the medication to reset efficacy. In addition, unlike stimulants, WAKIX does not increase dopamine levels in the pleasure center of the brain, which contributes to its lack of abuse potential. According to the National Sleep Foundation, stimulants have the potential for abuse, so their use must be considered carefully by patients and HCPs. WAKIX gives patients and HCPs a new therapeutic option.
- ***WAKIX can be used as monotherapy or administered concomitantly with other narcolepsy treatments.*** Narcolepsy is a difficult disorder to manage and the majority of narcolepsy patients often require multiple medications to treat their symptoms. WAKIX was studied in combination with each of modafinil and sodium oxybate (two common treatments for narcolepsy) and demonstrated no effect on the pharmacokinetic, or PK, profile of either treatment, and neither treatment had a clinically relevant effect on the PK profile of WAKIX. We believe the ability of WAKIX to be taken as monotherapy or concomitantly with other narcolepsy medications affords HCPs the flexibility to better manage their patients with narcolepsy.
- ***WAKIX is a once-daily oral tablet administered in the morning upon wakening***. Patients have identified a need for treatment options that are easier to take and are dosed less frequently. We believe that once-daily dosing with WAKIX addresses this need and may help improve patient compliance with treatment.

Our Strategy

Our goal is to become a leading pharmaceutical company dedicated to developing and commercializing novel treatment options for patients with rare neurological disorders living with unmet medical needs, beginning with a focus on narcolepsy. The key elements of our strategy are to:

- ***Commercialize WAKIX in the United States***. We have assembled a team of approximately 150 professionals that possess comprehensive life sciences experience. We have also established a robust company infrastructure to execute on our core business and growth strategies. This team includes over 70 dedicated and experienced sales professionals who call on the approximately 8,000 HCPs who treat the majority of narcolepsy patients in the United States. In the fourth quarter of 2019, we launched WAKIX in the United States and the product became commercially available in early November 2019.
- ***Expand our Label in Narcolepsy***. Building upon an EDS indication in adult patients with narcolepsy, we plan to initiate a pediatric development program in the second half of 2020 with the goal of gaining a pediatric indication for both EDS and cataplexy as well as obtaining

pediatric exclusivity. In addition, we are evaluating our options regarding the approach to take with the FDA in pursuit of a cataplexy indication in adult patients with narcolepsy.

- ***Expand Into New Indications Beyond Narcolepsy***. We believe that pitolisant's novel MOA has therapeutic potential in several other rare neurological disorder patient populations. In the fourth quarter of 2019, we completed a Phase 1 PK clinical trial in pediatric patients with PWS, and initiated a long-term, open-label safety trial in these patients. We intend to commence a Phase 2 clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS in the first half of 2020. Topline results from this clinical trial are expected in the second half of 2021. For patients with DM1, we are planning to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with DM1 in a Phase 2 clinical trial targeted to commence second half of 2020. Topline results from this clinical trial are expected in the first half of 2022. We also plan to explore pitolisant's potential as a treatment of EDS and related symptoms in other rare neurologic disorders, including those in which fatigue and cognitive impairment are prominent symptoms with significant impact on daily functioning.
- ***Explore Expansion of our Product Portfolio***. We plan to explore obtaining additional licensing rights from Bioprojet to expand into certain international markets with WAKIX. As we continue our commercial growth and develop a global footprint, we will assess in-licensing or acquiring complementary rights, assets or product candidates that allow us to leverage our existing infrastructure and expand within our strategic areas of focus.

Early Launch Metrics

As of March 31, 2020, total prescriptions written were , which represents % of the approximately 44,000 diagnosed and treated narcolepsy patients in the United States within days of product availability. We have also secured formulary access for million covered lives, which represents % of our target covered lives. Of those patients who received a prescription, patients started treatment on WAKIX. For the three months ended March 31, 2020, net sales of WAKIX were $ million.

Our History and Leadership Team

Our operating subsidiary, Harmony Biosciences, LLC, was formed in May 2017. We were formed as a Delaware limited liability company in July 2017 and converted to a Delaware corporation in September 2017. We concurrently acquired the U.S. rights to develop and commercialize pitolisant from Bioprojet. In February 2020, we changed our name to Harmony Biosciences Holdings, Inc. Since inception, we have raised approximately $345 million in equity financing from healthcare investors including Paragon Biosciences, LLC, venBio Partners, Novo Holdings A/S, Valor Equity Partners, Vivo Capital and HBM Healthcare Investments, or their respective affiliates. We have assembled an experienced leadership team with a track record of developing and commercializing products to treat rare neurological disorders. We believe that the clinical development, regulatory, commercial and operational expertise of our executive and senior leadership team will be essential as we execute on our strategy of becoming a leading pharmaceutical company focused on developing and commercializing innovative therapies for the treatment of rare neurological disorders while delivering significant value to both patients and shareholders.

Our management team has held senior positions at leading pharmaceutical companies, including Cephalon, Inc., or Cephalon, Teva Pharmaceutical Industries Ltd., or Teva, Merck & Co., Inc., or Merck, Wyeth, LLC and ViroPharma Incorporated, or ViroPharma, among others, and possesses substantial experience and expertise in developing and commercializing products for rare neurological disorders, including narcolepsy and other sleep disorders.

John C. Jacobs, our President and Chief Executive Officer, has held a variety of senior leadership roles of increasing responsibility throughout his career including roles in marketing, commercial, operations and general management in both U.S. and global markets. Prior to Harmony, Mr. Jacobs held roles as General Manager of Teva's branded business in Canada and led North American Commercial Operations for Teva. Jeffrey Dierks, our Chief Commercial Officer, formerly Vice President of Marketing at Harmony Biosciences and Senior Director U.S. Pain Care and Sleep Disorders and Migraine Marketing at Teva, has over 20 years of commercial leadership experience with demonstrated success in leading product launches. Jeffrey Dayno, MD, our Chief Medical Officer, formerly Chief Medical Officer at Egalet Corporation, is a neurologist with 10 years of experience in clinical and academic medicine followed by over 20 years of experience in research and development leadership roles at Merck, Cephalon and ViroPharma.

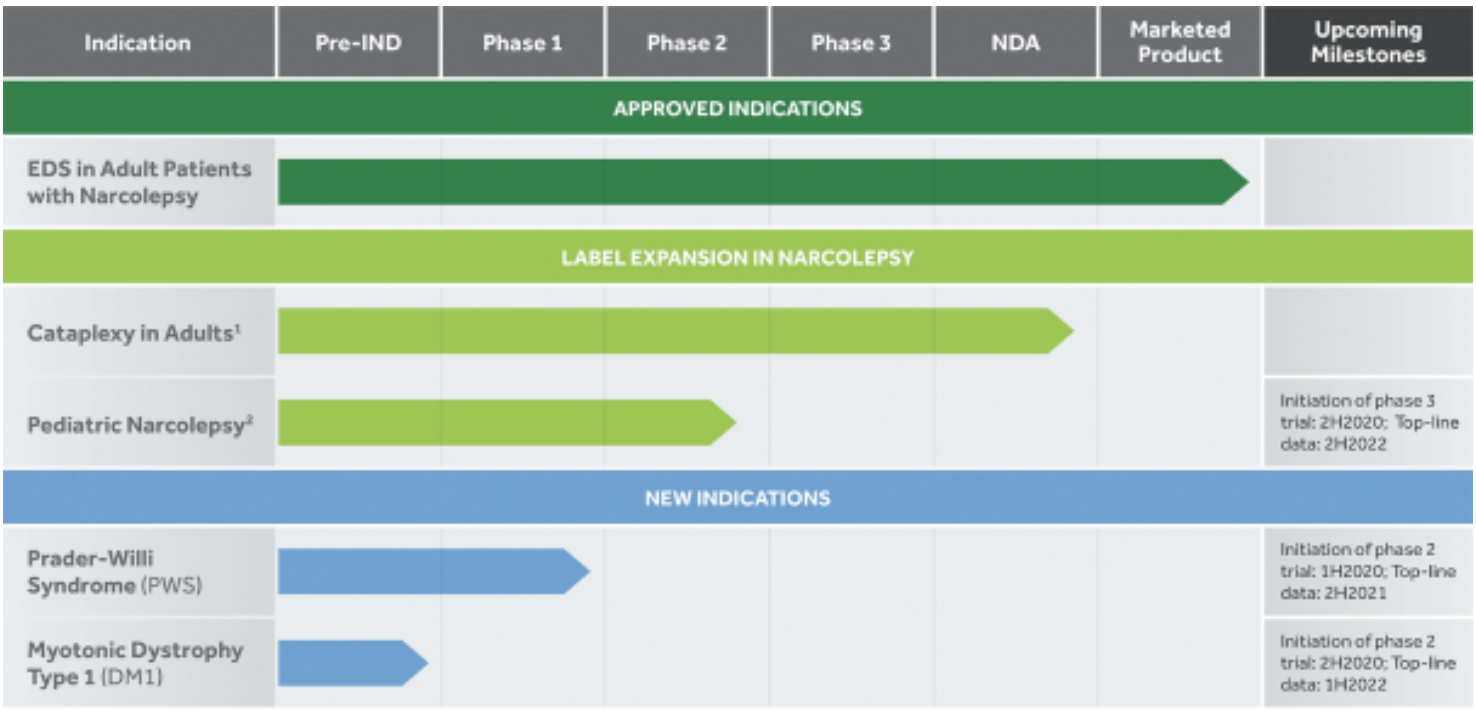
Overview of Development Pipeline

We are actively working on label expansion for WAKIX in narcolepsy, including a Phase 3 clinical trial in pediatric patients to support FDA approval for indications for both EDS and cataplexy in pediatric patients. We also intend to work with the FDA toward obtaining pediatric exclusivity for WAKIX. In addition, following the FDA's decision not to grant approval to WAKIX for the treatment of cataplexy in adult patients with narcolepsy, we are evaluating our options regarding the approach to take with the FDA in pursuit of this indication.

We believe that pitolisant's ability to regulate histamine gives it the potential to provide therapeutic benefit in other rare neurological disorders that are mediated through the H_3 receptor and histamine signaling. We plan to explore the potential benefit of pitolisant in additional rare neurological indications beyond narcolepsy, initially focusing on the treatment of EDS associated with PWS and DM1.

PWS is a rare genetic disorder caused by a loss of function of specific genes on chromosome 15 resulting in hypothalamic dysfunction. The hypothalamus controls both sleep-wake states and hunger-satiety. Therefore, two of the main symptoms in patients with PWS are EDS and insatiable hunger, or hyperphagia. Other consequences of PWS include low muscle tone, short stature, behavioral problems and cognitive impairment. It is estimated that approximately one in 12,000 to 15,000 people in the United States suffers from PWS. In a recent study, more than 90% of PWS patients surveyed reported EDS. We completed a Phase 1 PK clinical trial in pediatric patients with PWS in the fourth quarter of 2019, and initiated a long-term, open-label safety study in these patients. We intend to commence a Phase 2 clinical trial in patients with PWS in the first half of 2020. Topline results from this clinical trial are expected in the second half of 2021.

DM1 is a rare, multi-system genetic disease that affects the neuromuscular system as well as several other systems. It is inherited in an autosomal dominant pattern and the underlying cause of DM1 is a mutation in the myotonic dystrophy protein kinase, or DMPK, gene on chromosome 19. DM1 is the most common form of adult-onset muscular dystrophy and affects as many as 40,000 patients in the United States. EDS and fatigue are hallmark clinical characteristics in patients with DM1 and are referred to as the most frequent non-muscular symptoms in patients with DM1. Cognitive impairment is also a prominent symptom in patients with DM1 and all of these symptoms are thought to be mediated through H_3 receptors and histaminergic pathways located throughout the central nervous system, or CNS. We anticipate commencing a Phase 2 clinical trial in patients with DM1 in the second half of 2020. Topline results from this clinical trial are expected in the first half of 2022.



1. We received a complete response letter for treatment of cataplexy in adult patients with narcolepsy. We are currently evaluating our options regarding the approach to take with the FDA in pursuit of this indication.
2. Current trial being conducted by Bioprojet. We plan to commence a Phase 3 clinical trial in pediatric patients with narcolepsy in pursuit of pediatric indications for both EDS and cataplexy.

Beyond the target indications listed above, we intend to further explore pitolisant in other rare neurological disorders in which fatigue and cognitive impairment are prominent symptoms with significant impact on daily functioning.

Our Commercialization Strategy

We launched WAKIX into the narcolepsy market in November 2019 and are engaging with HCPs, patients and payors through the focused commercialization strategy outlined below to optimize adoption of WAKIX in the marketplace:

- ***HCP Awareness and Adoption***: To facilitate HCP awareness and adoption of WAKIX, we have deployed our dedicated, in-house, over 70-person sales team to educate a defined prescriber base of approximately 8,000 HCPs comprised of neurologists, pulmonologists, psychiatrists and high-prescribing primary care physicians who specialize in or focus on sleep disorders. We believe these HCPs diagnose and treat the majority of the narcolepsy patients in the United States. We began our commercial HCP outreach in August 2019 following FDA approval of WAKIX for the treatment of EDS in adult patients with narcolepsy.
- ***Patient Awareness***: It is estimated that narcolepsy affects anywhere from 135,000 to 200,000 Americans with fewer than 50% diagnosed. Of those living with narcolepsy in the United States, it is estimated that fewer than 45,000 are on narcolepsy medications, which we believe indicates a significant unmet medical need. To drive patient awareness of WAKIX and its differentiated product profile, we have been communicating with the narcolepsy patient community and providing them with educational materials and information on WAKIX.
- ***Payor Coverage***: Recognizing the importance of payor coverage, our field market access team has been engaging with national and regional payors over the past two years to educate them on the clinical data and value proposition of WAKIX. Through March 31, 2020, we have secured formulary access covering approximately million lives.

We believe the differentiating attributes of WAKIX that will facilitate awareness, adoption, and coverage include: (i) it is a first-in-class molecule with a novel MOA, (ii) it is the first-and-only non-scheduled treatment approved for narcolepsy, (iii) it is not a stimulant, (iv) it has broad clinical utility because it can be used as monotherapy or administered concomitantly with other narcolepsy treatments, and (v) it is a once-daily oral tablet administered in the morning upon wakening.

Clinical Development of WAKIX (pitolisant)

Overview

The strategy behind the clinical development of pitolisant is based on its MOA, which is thought to work by regulating histamine transmission. Pitolisant is a first-in-class molecule with a novel MOA, acting as a potent and highly selective antagonist/inverse agonist of the H_3 receptor. It activates histaminergic neurons in the brain, a neuronal system involved in the maintenance of wakefulness, attention, vigilance and cognition. Pitolisant binds to H_3 receptors on presynaptic neurons and blocks the normal negative feedback mechanism for histamine release, resulting in increased release of this wake-promoting neurotransmitter. It also functions as an inverse agonist, resulting in enhanced histamine synthesis and release from presynaptic neurons. Increased histamine available in the synapse binds to postsynaptic H_1 receptors, activating postsynaptic neurons, which stimulate wake-promoting brain regions and inhibit sleep-promoting regions of the brain.

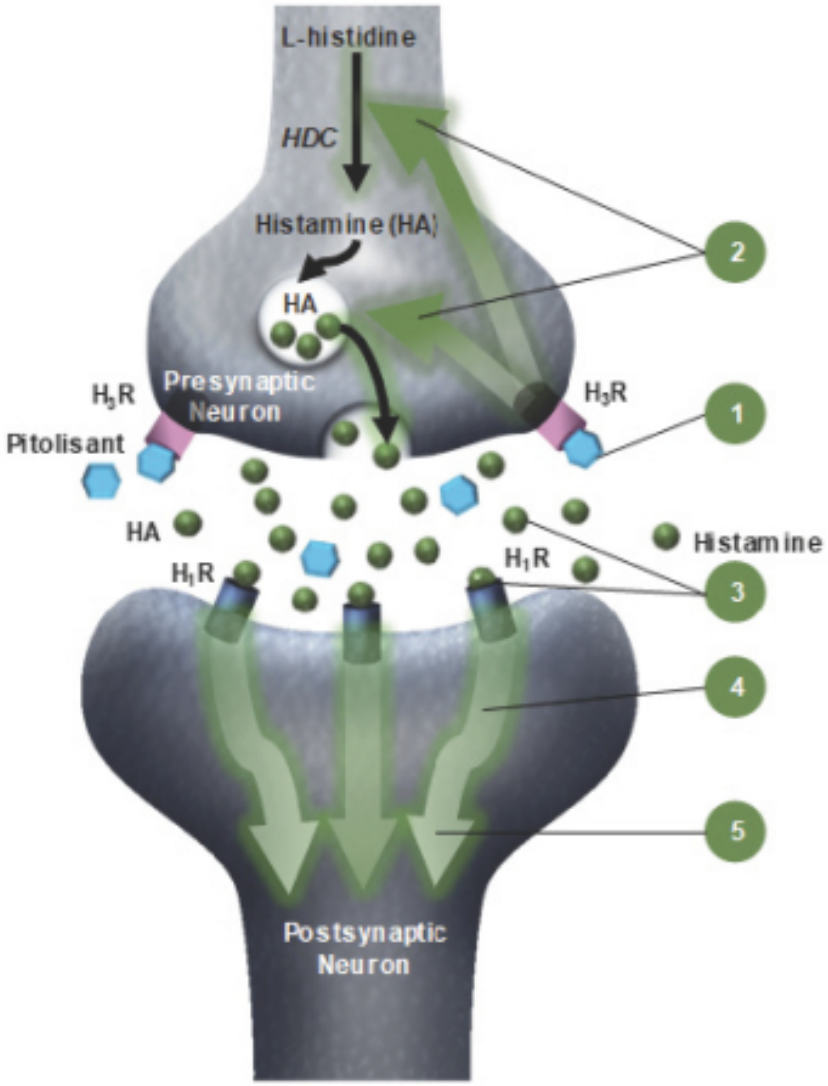
Pitolisant also stimulates the release of other wake-promoting neurotransmitters (dopamine, norepinephrine, serotonin and acetylcholine) via H_3 heteroreceptors within those neuronal systems. Importantly, pitolisant does not increase dopamine levels in the striatum, including the nucleus accumbens, which is the pleasure center of the brain where increase in dopamine levels is correlated with abuse potential. This feature of pitolisant's MOA, along with primarily working through the histaminergic system, are two of the aspects that differentiate pitolisant from all other currently approved treatments for narcolepsy.

WAKIX® (pitolisant) Mechanism of Action

Pitolisant is a histamine H_3-receptor antagonist / inverse agonist that enhances the activity of histaminergic neurons in the brain

1. Pitolisant binds to presynaptic H_3 autoreceptors, which blocks histamine binding to these receptors and increases histamine release from presynaptic neurons
2. Acting as an inverse agonist, pitolisant initiates increased histamine synthesis and release from vesicles into the synapse
3. This increased histamine in the synapse is then available to bind to excitatory postsynaptic H_1 receptors
4. Increased histamine binding at H_1 receptors results in an increase in neuronal firing of postsynaptic neurons
5. Increased firing of histamine neurons further activates wake-associated brain regions and further inhibits non-REM and REM sleep-associated brain regions

HA = Histamine
HDC = L-histidine decarboxylase
H_3R = Histamine 3 Receptor
H_1R = Histamine 1 Receptor



The safety profile of pitolisant is based on pooled safety data from 22 Phase 2/3 clinical trials conducted by Bioprojet, eight of which were in patients with narcolepsy and 14 of which were in other indications. These trials included a total of 1,513 unique patients, of whom 1,043 received pitolisant in double-blind placebo-controlled studies, and others received pitolisant in single-blind or open-label trials. Three successful pivotal trials in narcolepsy, HARMONY 1, HARMONY 1bis, and HARMONY CTP, were completed in Europe by Bioprojet and served as the foundation for the approval of pitolisant by the EMA in 2016 for the treatment of narcolepsy in adults with or without cataplexy. Pitolisant was evaluated in a long-term safety and tolerability trial, HARMONY 3, which further supported the results observed in HARMONY 1, HARMONY 1bis, and HARMONY CTP. The data from these trials were submitted, along with a human abuse potential, or HAP, trial, to the FDA as part of the NDA for WAKIX (pitolisant), which the FDA approved on August 14, 2019 for the treatment of EDS in adult patients with narcolepsy. The table below provides an overview of the trial designs from these five clinical trials.

	Trial Design	Number of Patients; % with Cataplexy	Maximum Dose; % at that Dose	Primary Endpoint	Results
Harmony 1	Randomized, double-blind, placebo & active-controlled trial; patients with narcolepsy +/- cataplexy; 8 weeks in duration	N = 95 80%	35.6 mg; 61%	Change in Epworth Sleepiness Scale (ESS) score	Pitolisant demonstrated a 3.1-point greater reduction in ESS score vs. placebo (p=0.022)
Harmony 1bis	Randomized, double-blind, placebo & active-controlled trial; patients with narcolepsy +/- cataplexy; 8 weeks in duration	N = 166 75%	17.8 mg; 76%	Change in ESS score	Pitolisant demonstrated a 2.2-point greater reduction in ESS score vs. placebo (p=0.030)
HARMONY CTP	Randomized, double-blind, placebo-controlled trial; patients with narcolepsy and cataplexy; 7 weeks in duration	N = 106 100%	35.6 mg 65%	Change in Weekly Rate of Cataplexy (WRC)	Pitolisant demonstrated a significant reduction in the WRC compared to placebo (75% vs. 38%; p<0.0001)
HARMONY 3	Long-term, open-label, real-world trial; ³1 year	N = 104 74%	35.6 mg 88%	Long-term safety	Safety / tolerability profile c/w that seen in the RCTs
Human Abuse Potential Study	Randomized, double-blind, active & placebo- controlled, 4-way crossover study	43 n/a	35.6 mg & 213.6 mg; Phentermine 60 mg (active control)	Maximum Drug Liking	Pitolisant demonstrated a statistically significant and clinically relevant reduction in drug liking compared to phentermine (p<0.0001)

RCTs = randomized controlled trials

Clinical Trial Highlights

The key findings from these clinical trials are as follows:

- Pitolisant showed a statistically significant improvement in EDS in adult patients with narcolepsy in HARMONY 1 and HARMONY 1bis compared to placebo. Specifically, the clinical trials demonstrated a statistically significant, and clinically relevant, improvement in EDS as measured by the Epworth Sleepiness Scale, or ESS, scores compared to placebo (p=0.022 in HARMONY 1 and p=0.030 in HARMONY 1bis), supported by statistically significant improvement on the Maintenance of Wakefulness Test, or MWT.

- Pitolisant demonstrated a statistically significant reduction in measures of cataplexy in adult patients with narcolepsy in HARMONY CTP as compared to placebo. Reduction in the weekly rate of cataplexy in patients on pitolisant was 75% compared to a 38% reduction in the placebo group ($p<0.0001$). This finding was supported by a significant reduction in cataplexy (a secondary endpoint) in the HARMONY 1 trial of 62% in the pitolisant group compared to 8% in the placebo group ($p=0.034$). However, the FDA stated that the cataplexy data from the HARMONY 1 trial in the NDA did not provide substantial evidence of effectiveness with respect to cataplexy because the statistical analysis plan did not prospectively control for Type 1 error of the secondary endpoints, and the subgroup of patients with cataplexy was not identified prospectively. As a result, the FDA issued a complete response letter, or CRL, with respect to the cataplexy indication.
- Pitolisant was generally well tolerated in clinical trials. In the placebo-controlled clinical trials conducted in patients with narcolepsy with or without cataplexy, the most common adverse reactions (occurring in ^{3}5% of patients and at twice the rate of placebo) with the use of pitolisant were insomnia (6%), nausea (6%), and anxiety (5%). In these trials, 6 of the 152 patients (3.9%) who received pitolisant and 4 of the 114 patients (3.5%) who received placebo discontinued because of an adverse event.
- In the HARMONY 3 trial, a favorable long-term safety/tolerability profile for pitolisant out to one year was demonstrated; safety findings were similar to those seen in the randomized controlled trials, with no new safety signals identified.
    - In this open-label, long-term real-world trial, improvement in EDS (as measured by a reduction in ESS scores) and reduction in cataplexy (as measured by reduction in mean daily cataplexy episodes) was maintained out to twelve months.
- In a clinical HAP trial, pitolisant demonstrated a statistically significantly lower maximum drug liking (primary endpoint), overall drug liking, and willingness to take drug again compared to phentermine (C-IV), with responses similar to placebo. No evidence of abuse potential based on clinical and preclinical data has been observed to date, and WAKIX was therefore approved without being scheduled as a controlled substance by the DEA.

HARMONY 1

Design

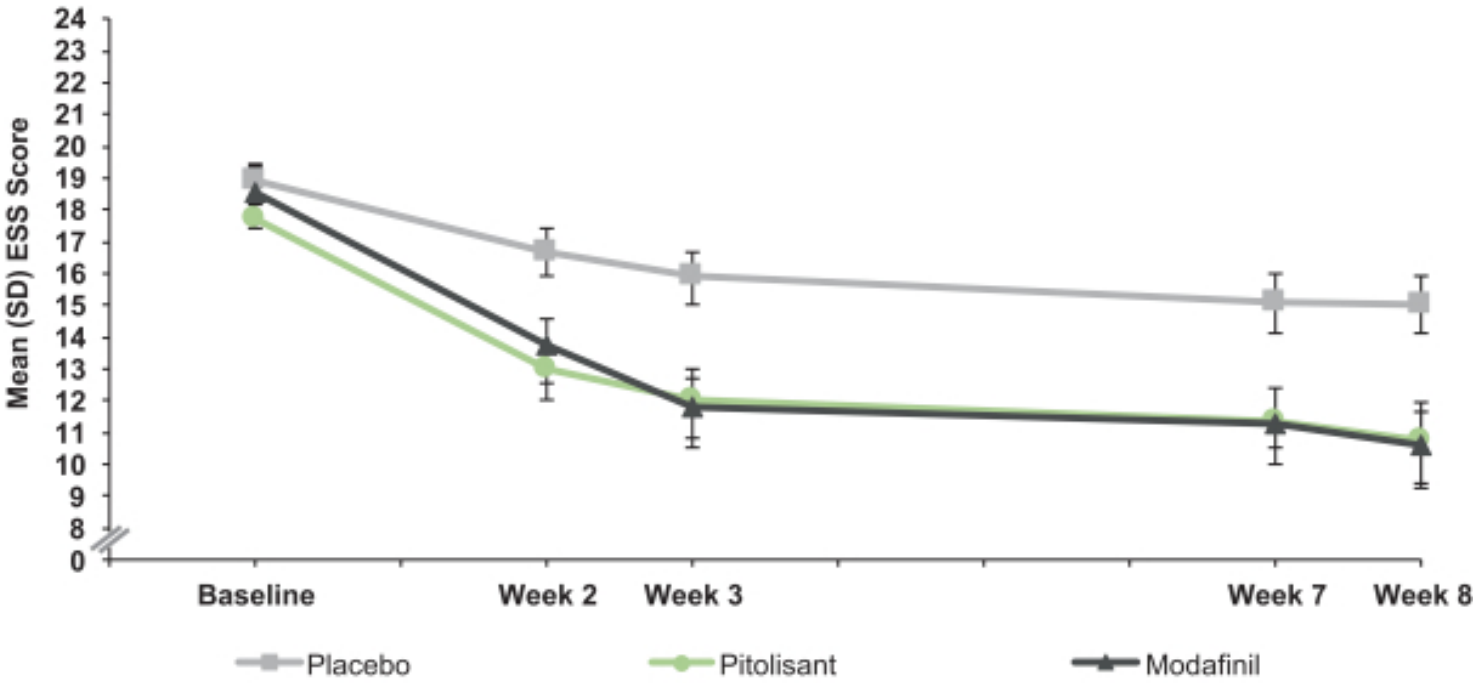
HARMONY 1 was a randomized, double-blind, placebo-controlled trial that evaluated the efficacy and safety of pitolisant in adult patients with narcolepsy on improvement in EDS over an eight-week period. The trial was conducted in the EU, and consequently was designed to include both a placebo arm and an active comparator, modafinil, which was used in doses up to 400 mg/day. HARMONY 1 consisted of 95 patients and had flexible dosing during the first three weeks of the trial, followed by five weeks of stable dosing. The maximum dose of pitolisant in this dose-to-effect trial was 35.6 mg and only 61% of the patients were titrated to this dose for the stable dosing period. Approximately 80% of the patients had a history of cataplexy.

The primary endpoint in the trial was the ESS score at final visit, adjusted for baseline, for pitolisant compared with placebo. ESS is a self-administered eight-item questionnaire scored 0 to 24 with lower scores corresponding to lower EDS. Secondary endpoints in HARMONY 1 included ESS responder rates, MWT (an objective measure of the ability to stay awake), the Sustained Attention to Response Task, or SART, reduction in cataplexy, Clinical Global Impression of Change, or CGI-C, for both EDS and cataplexy, the European Quality of Life Questionnaire, or the EQ-5D, and the Patient's Global Opinion on the Effect of Treatment Questionnaire. The main efficacy objective of the trial was to demonstrate superiority of pitolisant compared to placebo on the primary endpoint, while one of the secondary objectives was to explore the non-inferiority of pitolisant compared to modafinil on ESS score.

Efficacy Results

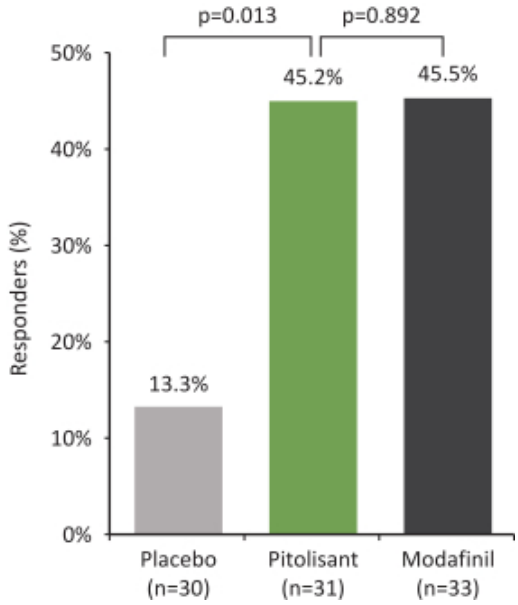
Pitolisant showed a significant reduction in mean ESS score at end of trial as compared to placebo (-6.0 versus -3.3) and between-group differences in ESS score were evident within the first two weeks of treatment. This resulted in a treatment effect (change in ESS score adjusted for baseline differences) of -3.1, which was statistically significant for pitolisant versus placebo (p=0.022). The final adjusted ESS score for modafinil was -6.9 and, based on this score, pitolisant was not found to be non-inferior to modafinil (mean difference of 0.09, p=0.932) and the trial therefore did not meet this secondary efficacy objective. We believe there are several factors that contributed to this finding. First, 73% of the patients on modafinil in this trial were titrated up to a dose of 400 mg/day (the recommended dose of modafinil in the FDA-approved U.S. Prescribing Information, or USPI, is 200 mg/day) while only 61% of the patients on pitolisant were titrated to the maximum pitolisant dose of 35.6 mg/day (which is the maximum approved dose in the USPI), such that a greater number of patients in the modafinil arm received the maximum effective dose than those in the pitolisant arm, raising the possibility that those subjects in the pitolisant arm could have seen greater treatment effect had they been dosed at the maximum dose available. Second, the margin of non-inferiority for the difference in the ESS scores pre-specified in the statistical analysis plan was narrow (2 points), meaning that the change in ESS score adjusted for baseline compared between pitolisant and modafinil had to have a lower 95% CI of no less than -2 points to declare pitolisant non-inferior to modafinil. The lower bound of the 95% CI of the analysis fell just outside this margin (-2.11). According to literature, however, a clinically relevant difference on the ESS ranges from 2–3 points, such that the non-inferiority margins pre-specified under the statistical analysis plan may have been too tight. Ultimately, however, the trial results comparing pitolisant and modafinil did not impact the FDA's findings that pitolisant was effective for improvement in EDS, and the FDA-approved label for WAKIX does not contain any data on modafinil.

Change in ESS Score Over Time

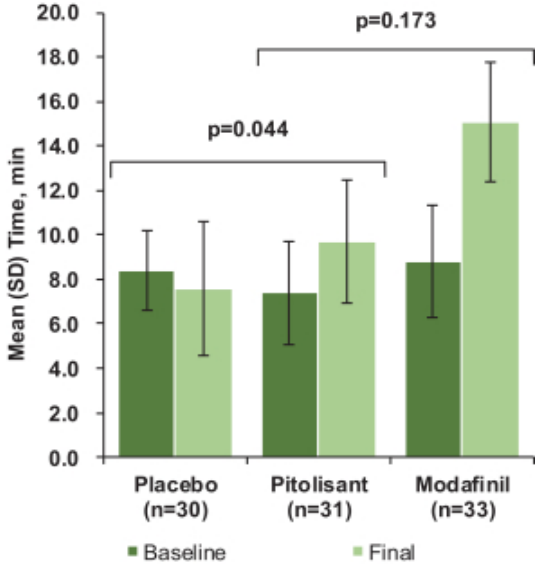


Regarding the secondary endpoints, ESS responder rates (a responder was defined as having a final ESS score £ 10) were significantly greater for those patients treated with pitolisant compared to those on placebo (45.2% vs. 13.3%, respectively; p=0.013). The responder rate for patients treated with modafinil was 45.5% and the difference compared to pitolisant was not statistically significant (p=0.892). On the MWT, pitolisant treatment improved performance when compared to placebo in a statistically significant manner (p=0.044), while improvement was not significantly different compared to modafinil (p=0.173).

Responders (Final ESS £ 10)



Change in MWT



With regard to other secondary endpoints, the overall pattern of response was that the findings for patients on both pitolisant and modafinil were superior to those on placebo while the responses were not statistically significantly different for pitolisant compared to modafinil. The SART Total Score (a measure of attention) was significantly higher in the pitolisant group as compared to placebo (p=0.041), and while not significantly different from the modafinil group (p=0.363), the scores were similar (9.1 and 8.9 for pitolisant and modafinil, respectively). The CGI-C for EDS showed improvement in 56% of patients on placebo, 73% of patients on pitolisant, and 86% of patients on modafinil. Regarding the daily cataplexy rates endpoint, patients treated with pitolisant experienced a 62% reduction in the daily rate of cataplexy compared to a reduction of 8% in those on placebo (p=0.034); the difference between modafinil (25%) and placebo was not statistically significant (p=0.396). Responses on the CGI-C for cataplexy were consistent with this outcome, with 29%, 45%, and 35% of patients who experienced cataplexy during the trial reporting an improvement in their cataplexy symptoms in the placebo, pitolisant, and modafinil groups, respectively. Lastly, the Patient's Global Opinion on the Effect of Treatment Questionnaire recorded positive responses in 56% of patients in the placebo group, 81% of patients in the pitolisant group, and 86% of patients in the modafinil group.

Safety Results

Pitolisant was generally well tolerated in HARMONY 1. Sixty patients experienced a treatment emergent adverse event, or TEAE, during the trial: 61% in the pitolisant group, 60% in the placebo group, and 70% in the modafinil group. The most commonly reported TEAE in the pitolisant treatment group was headache, reported by 35% of the patients, compared to 20% in the placebo group. Other frequently reported TEAEs in the pitolisant treatment group were insomnia, nausea and weight increase (each reported by two patients, or 6%). There were five serious adverse events during HARMONY 1 and none were considered treatment-related (two in the pitolisant group, two in the modafinil group, and one in the placebo group). There were no deaths during the trial and no significant changes in laboratory values or hemodynamic parameters (heart rate and blood pressure) from baseline to final visit in any group.

HARMONY 1bis

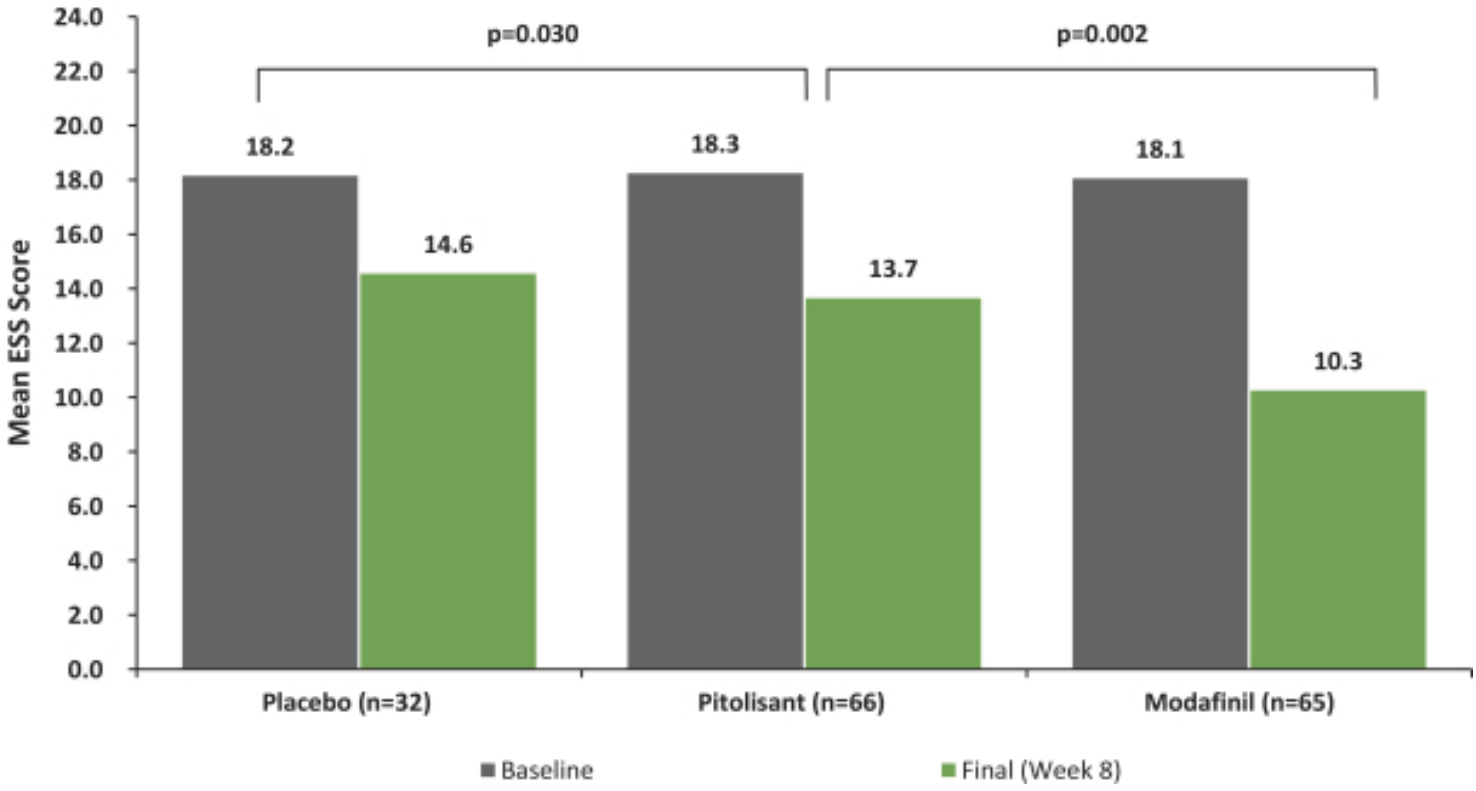
HARMONY 1bis was a randomized, double-blind, placebo-controlled trial that evaluated the efficacy and safety of pitolisant in adult patients with narcolepsy on improvement in EDS over an eight-week period. This trial was designed in accordance with recommendations from European regulators, and as such, contained both a placebo and active comparator arm. The active comparator was modafinil used in doses up to 400 mg/day. HARMONY 1bis enrolled 165 patients and had flexible dosing during the first three weeks of the trial, followed by five weeks of stable dosing. The maximum dose of pitolisant in this dose-to-effect trial was 17.8 mg and only 76% of the patients were titrated to this dose for the stable dosing period. 75% of the patients had a history of cataplexy.

The primary endpoint in the trial was the ESS score at final visit, adjusted for baseline, for pitolisant compared with placebo. Secondary endpoints included ESS responder rates, MWT, SART, reduction in cataplexy, CGI-C for both EDS and cataplexy, the EQ-5D, and the Patient's Global Opinion on the Effect of Treatment Questionnaire. The main efficacy objective of the trial was to demonstrate superiority of pitolisant compared to placebo on the primary endpoint, while one of the secondary objectives was to explore the non-inferiority of pitolisant compared to modafinil on ESS score.

Efficacy Results

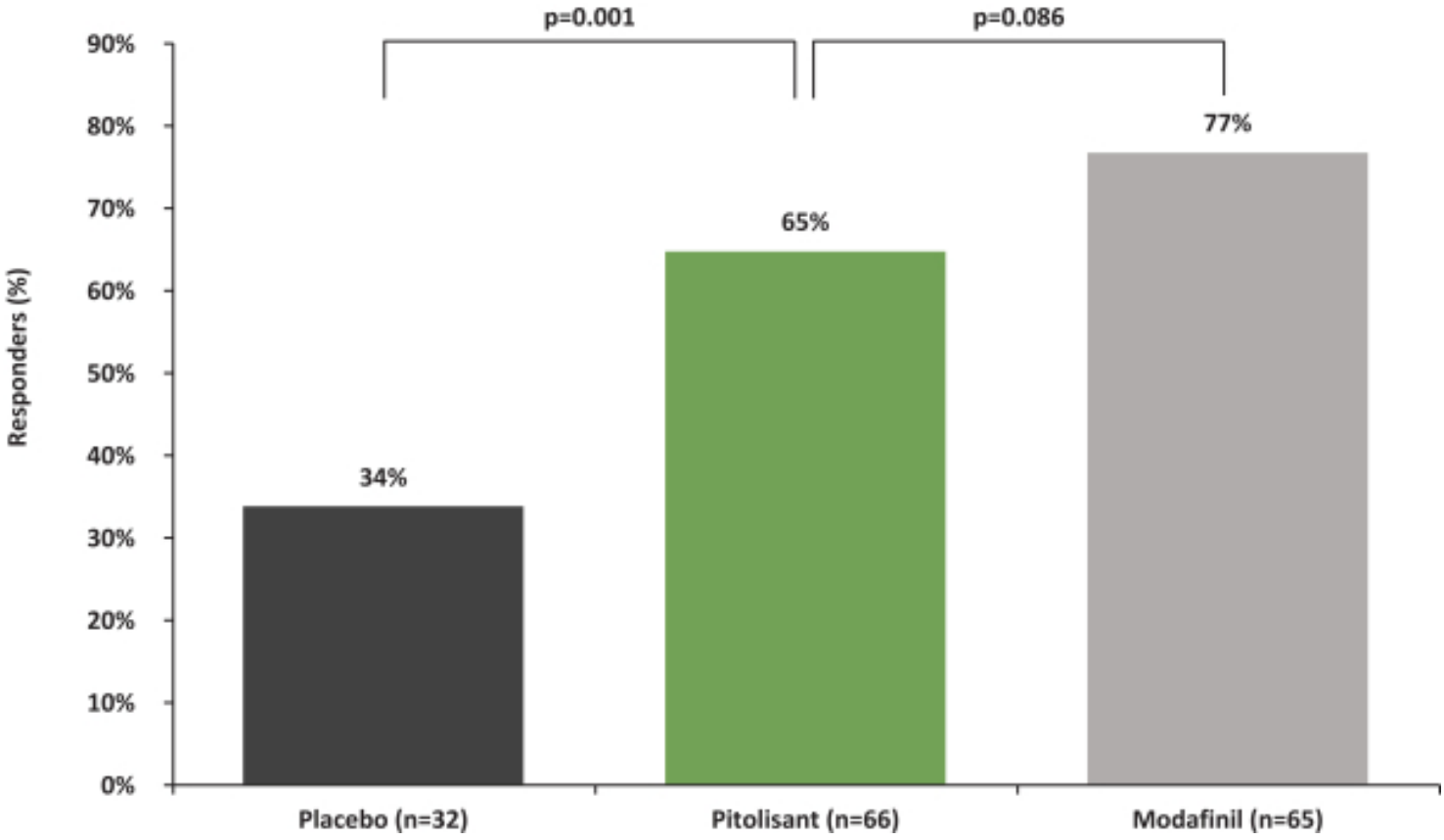
Pitolisant showed a significant reduction in mean ESS score at end of trial compared to placebo (-4.6 versus -3.6), with a statistically significant treatment effect (change in ESS score adjusted for baseline differences) of -2.2 versus placebo (p=0.030). The treatment effect between modafinil and pitolisant was -2.75 and, based on this score and the pre-specified statistical analysis plan, resulted in pitolisant not being non-inferior to modafinil. We believe the same factors that contributed to this result in HARMONY 1 also apply to HARMONY 1bis. In addition, in this trial, the maximum dose of pitolisant to which patients could be titrated (17.8 mg) was not the maximum labeled dose for pitolisant (which is 35.6 mg), and 24% of patients in this trial were on doses lower than 17.8 mg, which means that a substantial percentage of patients were on study drug at an amount less than the maximum approved dose in the USPI for pitolisant. In addition, modafinil was dosed up to 400 mg/day, while the recommended dose of modafinil in its USPI is 200 mg/day, which means that the respective doses of pitolisant and modafinil were not comparable.

Change in Mean ESS Score

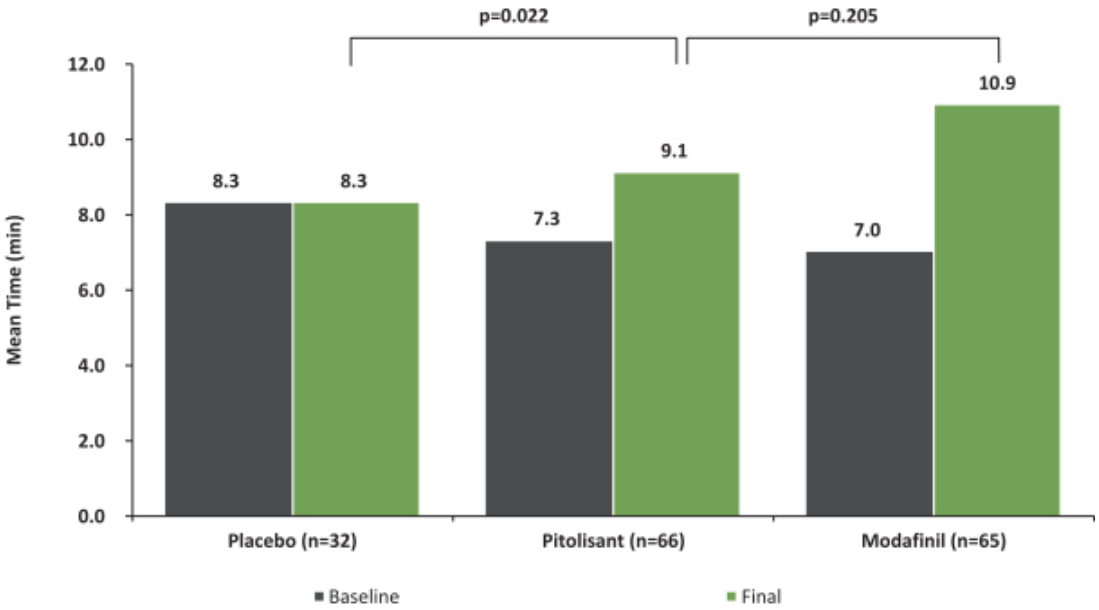


Regarding the secondary endpoints, ESS responder rates (a responder was defined as having a final ESS score £ 10 or change in ESS score ³ 3) were significantly greater for those patients treated with pitolisant compared to those on placebo (65% vs. 34%, respectively; p=0.001). The responder rate for patients treated with modafinil was 77% and the difference compared to pitolisant was not statistically significant (p=0.086). On the MWT, pitolisant treatment significantly improved performance when compared to placebo (p=0.022), while improvement was not significantly different compared to modafinil (p=0.294).

Responders (Final ESS £ 10 or D ESS ³ 3)



Change in MWT



With regard to other secondary endpoints, the overall pattern of response was that the findings for patients on both pitolisant and modafinil were superior to those on placebo while the responses were not significantly different for pitolisant and modafinil. The pitolisant group's SART Total Score was significantly improved compared to placebo (p=0.043), while not significantly different compared to modafinil (p=0.407). The CGI-C for EDS showed improvement in 37% of patients on placebo, 72% of patients on pitolisant, and 78% of patients on modafinil. Responses on the CGI-C for cataplexy showed improvement for 60% of patients treated with pitolisant compared to 54% of patients on modafinil and 36% of patients on placebo. However, the difference in the reduction in the daily rate of cataplexy between pitolisant (0.32) and placebo (0.31) was not statistically significant (p=0.873). Lastly, the findings on both the EQ-5D and the Patient's Global Opinion on the Effect of Treatment Questionnaire did not show any meaningful differences between the pitolisant and placebo treatment groups in the HARMONY 1bis trial (no statistical test was performed for the EQ-5D and the p-value for the Patient's Global Opinion on the Effect of Treatment Questionnaire was 0.070).

Safety Results

Pitolisant was generally well tolerated in HARMONY 1bis. Seventy-seven patients experienced a TEAE during the trial: 49% in the pitolisant group, 36% in the placebo group, and 49% in the modafinil group. The most commonly reported TEAEs in the pitolisant treatment group were headache (13%), dizziness (6%), vomiting (4.5%), insomnia (4.5%), and decreased appetite (4.5%). There were no serious adverse events in the pitolisant group and there was one serious adverse event during HARMONY 1bis in the modafinil treatment group, which was not treatment-related. There were no deaths during the trial and no significant changes in laboratory values or hemodynamic parameters (heart rate and blood pressure) from baseline to final visit.

HARMONY CTP

Design

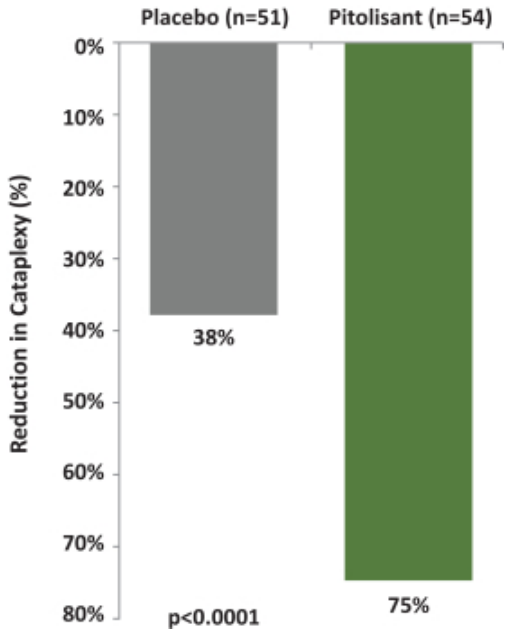
HARMONY CTP was a randomized, double-blind, placebo-controlled trial to evaluate the safety and efficacy of pitolisant on the reduction in cataplexy in adult patients with narcolepsy with frequent attacks of cataplexy over a seven-week period. HARMONY CTP consisted of 106 patients. The maximum dose of pitolisant in this dose-to-effect trial was 35.6 mg and only 65% of patients reached this dose during the stable dosing period. Both stimulants and wake-promoting agents were prohibited during the trial; only 11% of subjects were on stable doses of anti-cataplectic medications (7% in the pitolisant treatment group and 16% for placebo).

The primary endpoint in HARMONY CTP was the change in the weekly rate of cataplexy, or WRC, from baseline to the stable dosing period (Weeks 4–7). Secondary endpoints included proportion of patients with high cataplexy rate (WRC >15), CGI-C for cataplexy and EDS, mean change in ESS score and percentage of ESS responders, MWT, the EQ-5D, number of days with hallucinations (as recorded in the patient diaries), and Patient's Global Opinion on the Effect of Treatment Questionnaire.

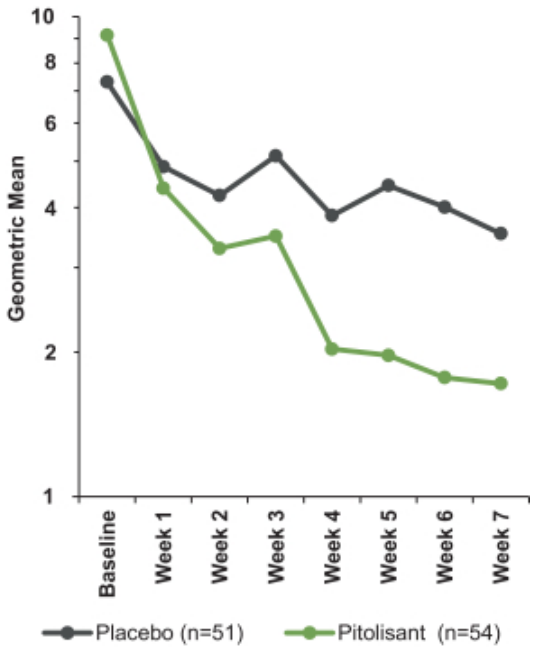
Efficacy Results

In HARMONY CTP, pitolisant resulted in a significantly greater reduction than placebo in the WRC from baseline to the stable dosing period (Weeks 4–7), with a 75% reduction in the pitolisant group compared to 38% on placebo (p<0.0001). Further, significantly fewer patients had WRC >15 at endpoint with pitolisant (6%) versus placebo (24%) (p=0.005). The clinical relevance of these findings was captured by the CGI-C related to cataplexy. Mean CGI-C score was 3.5±1.1 with placebo versus 2.6±1.1 with pitolisant. The mean reduction of the CGI-C score for pitolisant compared with placebo was -0.95 (95% CI (-1.36, -0.54); p<0.0001). Overall positive response rates on the CGI-C related to cataplexy were 33% on placebo and 67% on pitolisant.

Percent Reduction in WRC

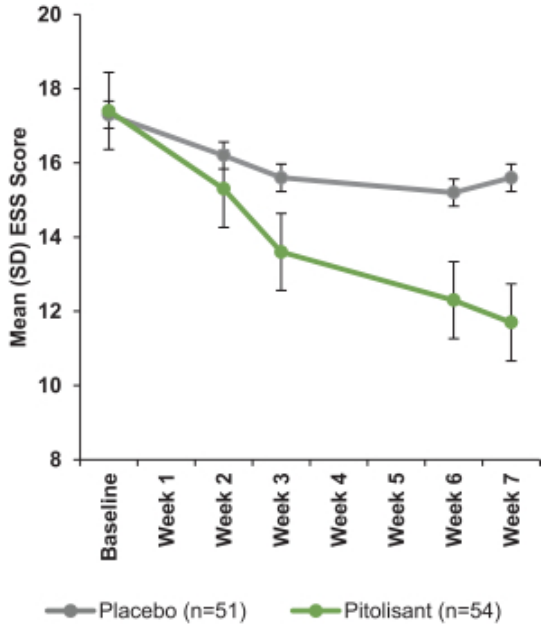


Reduction in WRC Over Time

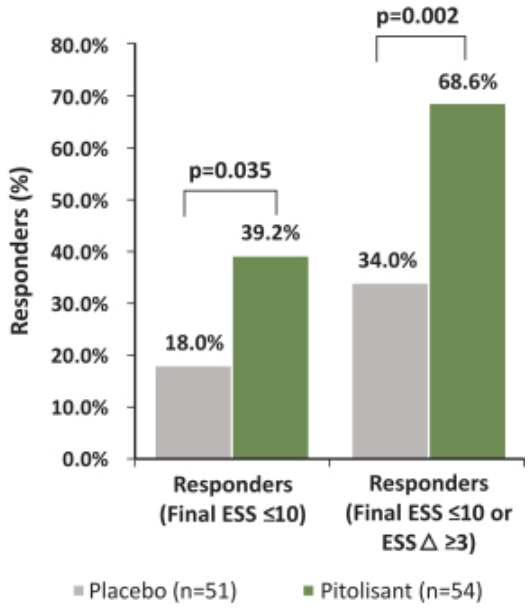


With regard to other secondary endpoints, pitolisant demonstrated a statistically significant reduction in mean ESS score from baseline to final visit at week seven as compared to placebo (-5.4 vs. -1.9; p=0.0001) and significantly higher ESS responder rates compared to placebo (p=0.035 for Type 1 ESS responders rate and p=0.002 for Type 2 ESS responders rate; see graph below). On the CGI-C related to EDS, the mean score was 3.7 with placebo versus 2.6 with pitolisant, with a mean reduction of -0.99 (p<0.0001). Overall positive response rates on the CGI-C related to EDS were 24% on placebo and 69% on pitolisant.

Change in ESS Score Over Time



ESS Responder Rate



With regard to other secondary endpoints, pitolisant showed a statistically significant improvement on the MWT from baseline to end of trial compared to placebo. Baseline geometric means on the MWT were 4.3 minutes and 3.7 minutes for placebo and pitolisant, respectively, with final MWT values of 4.6 minutes and 7.1 minutes for placebo and pitolisant, respectively; the improvement in MWT was 78% higher with pitolisant compared to placebo (p=0.003). On the Patient's Global Opinion on the Effect of Treatment Questionnaire, overall improvement was reported in 26% of patients on placebo compared to 54% on pitolisant (p=0.001).

Safety Results

Pitolisant was generally well tolerated in HARMONY CTP. Thirty-five patients experienced a TEAE during the trial: 35% in the pitolisant group and 31% in the placebo group. The most commonly reported AE in the pitolisant group in HARMONY CTP was headache, which 9% of the group reported, compared to 10% for the placebo group. Other frequently reported AEs in the pitolisant group were irritability, anxiety and nausea (each reported by 3 patients, or 6%). There were no deaths or serious adverse events during HARMONY CTP and no significant changes in laboratory values or hemodynamic parameters (heart rate and blood pressure) from baseline to final trial visit in either group.

HARMONY 3

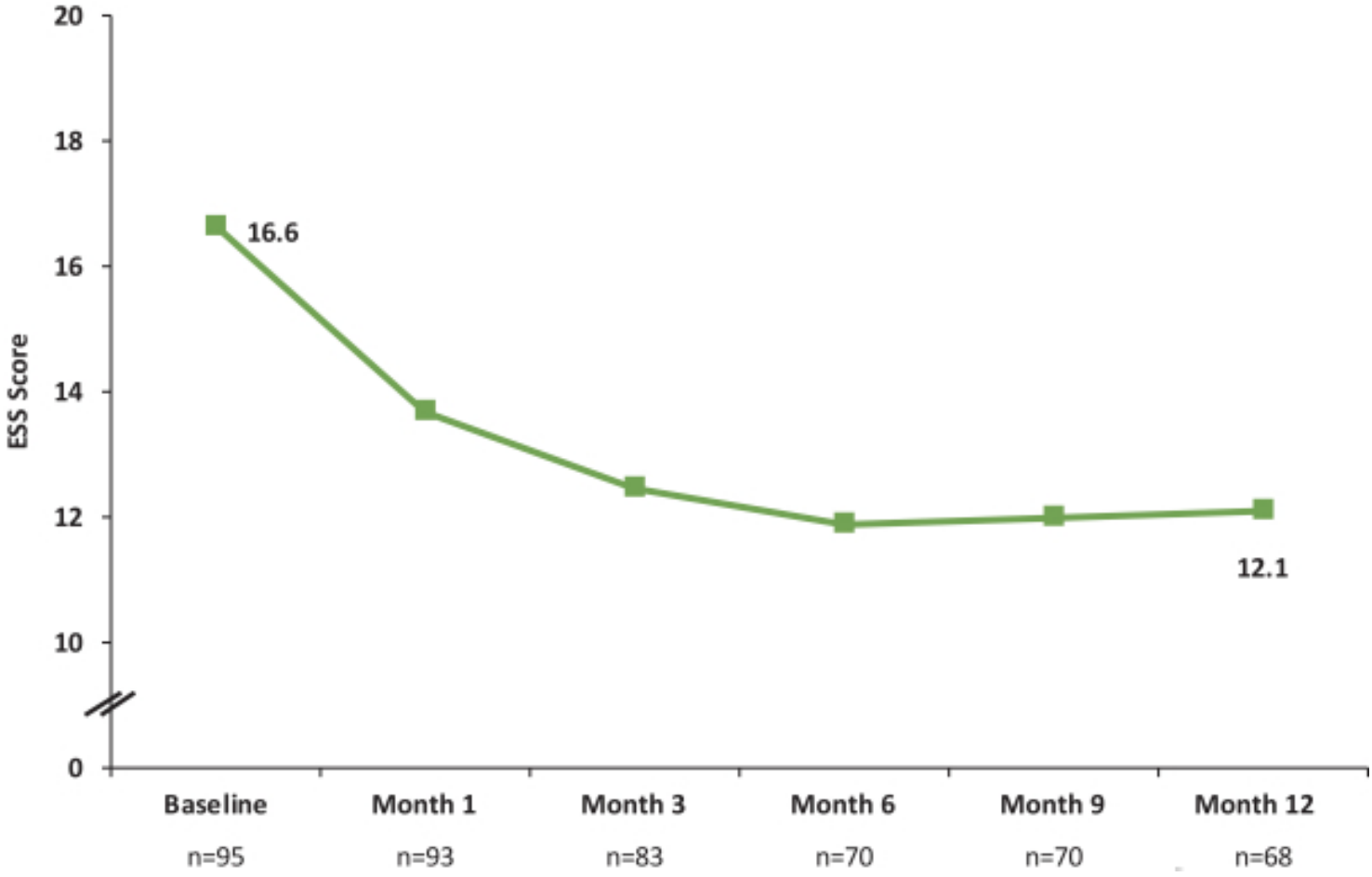
Design

HARMONY 3 was an open-label, real-world trial to assess the long-term safety and tolerability of pitolisant in the treatment of EDS in adult patients with narcolepsy, with or without cataplexy, over a one-year period (with a 5-year extension at the trial sites in France). HARMONY 3 enrolled 104 patients, 102 of whom were treated with pitolisant, and 68 completed out to one year. In HARMONY 3, 75% of patients had a history of cataplexy and 76% of patients who completed out to one year were on the maximum dose of pitolisant of 35.6 mg. For the 5-year extension phase at the trial sites in France, 50 patients were eligible to continue, of which 48 patients elected to do so and 14 of them completed out to 5 years.

Efficacy Results

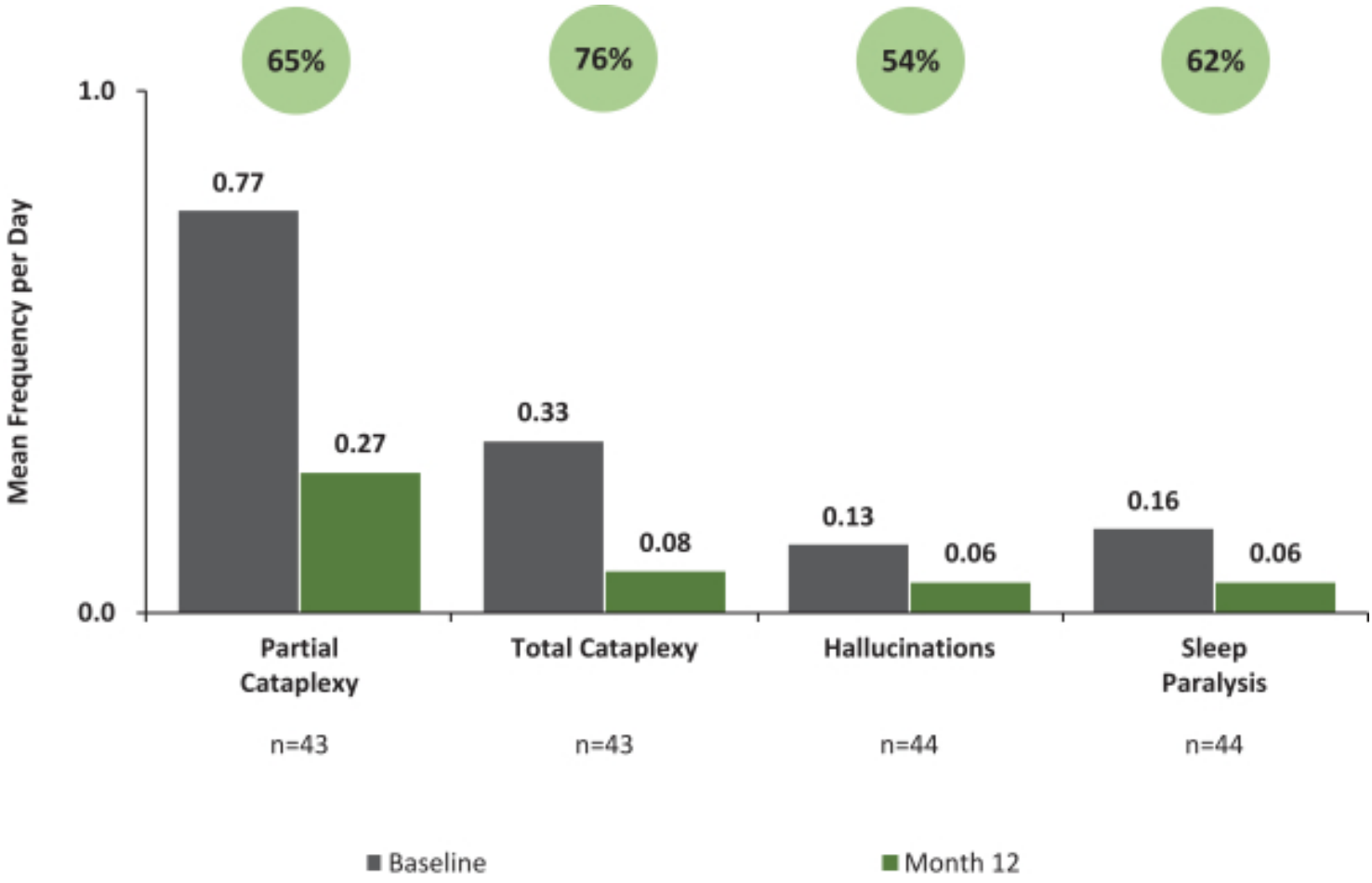
In the 68 patients with data at baseline and at 12 months in HARMONY 3, pitolisant reduced the mean ESS score by -4.63 over this period. The magnitude of the decrease in ESS score was larger in the subgroup of patients (n=86) who were not on pitolisant at trial entry (-5.25) as compared to the subgroup of patients (n=16) who came into the trial on pitolisant from the French Compassionate Use Program (-2.63).

ESS Score Over Time



In HARMONY 3, pitolisant also demonstrated a reduction in cataplexy and other symptoms of REM intrusion into wakefulness from baseline to month 12, showing a reduction of 65% to 76% in partial or total cataplexy attacks, respectively, out to one year. Reductions of more than 50% were also seen for other symptoms of REM dysfunction, such as hallucinations and sleep paralysis.

Reduction in Cataplexy and Other Symptoms of REM Sleep Intrusion into Wakefulness with Pitolisant



Safety Results

Pitolisant was generally well tolerated in HARMONY 3. AEs observed with long-term pitolisant treatment were consistent with those observed in short-term randomized, controlled trials such as HARMONY 1, HARMONY 1bis, and HARMONY CTP. Fifty-eight of the 102 treated patients (57%) reported an aggregate of 168 TEAEs in HARMONY 3, the most common of which are shown in the table below. During the one-year trial, there were no deaths and seven patients reported 10 serious adverse events, nine of which were deemed by the investigator to be unrelated to pitolisant, and one miscarriage which was considered possibly related. No clinically significant changes in laboratory parameters, vital signs or electrocardiogram parameters were recorded over the course of the trial.

Adverse Events (Incidence ≥3%), n (%)	Total Population (N=102)
Any adverse event	58 (56.9)
Headache	12 (11.8)
Insomnia	9 (8.8)
Weight increased	8 (7.8)
Anxiety	7 (6.9)
Depression	5 (4.9)
Nausea	5 (4.9)
Irritability	4 (3.9)
Vomiting	4 (3.9)
Vertigo	4 (3.9)

Clinical HAP Trial

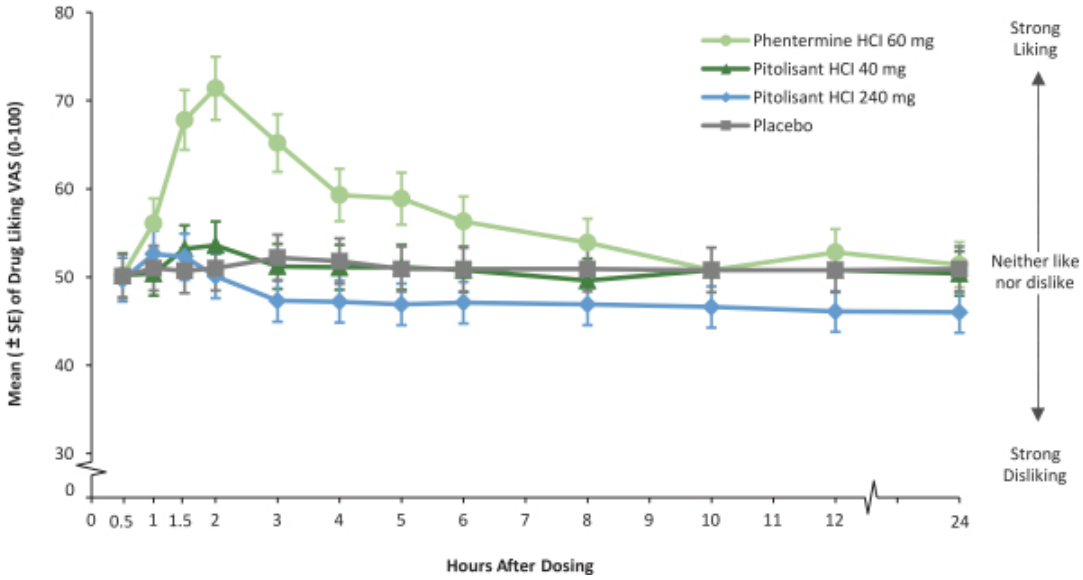
Design

A clinical HAP trial was conducted to evaluate the human abuse potential of pitolisant. In this trial, nondependent, recreational stimulant users able to distinguish phentermine hydrochloride (HCl; 60 mg), a CIV stimulant, from placebo in a drug discrimination test were randomized in a 4-period, double-blind, crossover design to receive single doses of pitolisant 35.6 mg (therapeutic dose), pitolisant 213.6 mg (supra-therapeutic dose), phentermine HCl 60 mg, and placebo. The primary endpoint was maximum effect (E_{max}) on the 100-point Drug Liking (at the moment) visual analog scale.

Results

A total of 43 subjects were enrolled and 38 completed the trial. Mean Drug Liking E_{max} was significantly greater for phentermine (78.7) versus pitolisant 35.6 mg (57.3; p<0.0001) and pitolisant 213.6 mg (59.0; p<0.0001). Drug Liking E_{max} was similar for pitolisant (both doses) and placebo (56.1) (p<0.001 for 35.6 mg versus placebo, and p=0.003 for 213.6 mg versus placebo).

Maximum Drug Liking



Similarly, for key secondary measures of Overall Drug Liking and willingness to Take Drug Again, mean E_{max} scores were significantly greater for phentermine (77.4 for Overall Drug Liking and 78.7 for Take Drug Again) versus pitolisant 213.6 mg (49.3 and 44.5) and 35.6 mg (52.7 and 49.4) ($p < 0.0001$ for each comparison for both doses of pitolisant).

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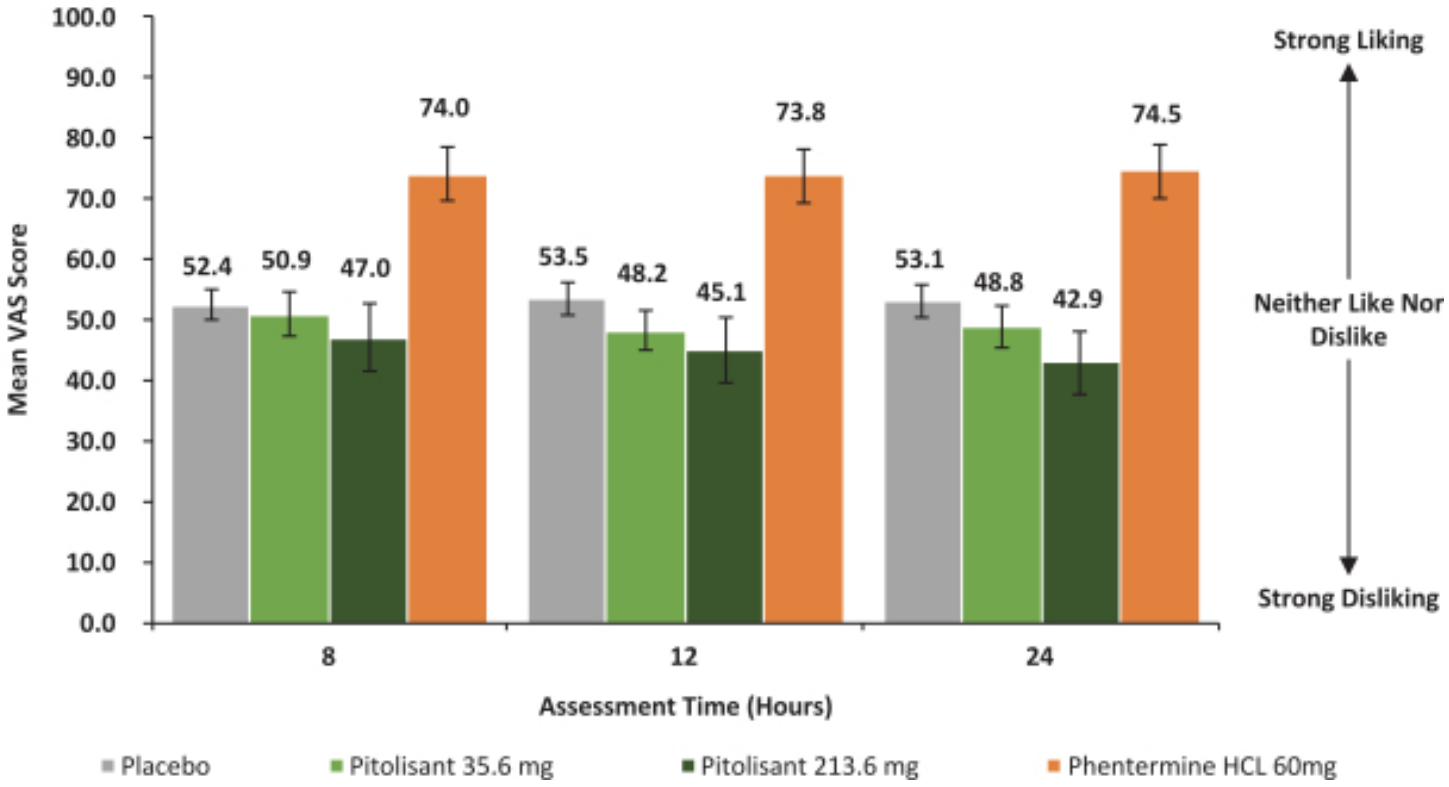
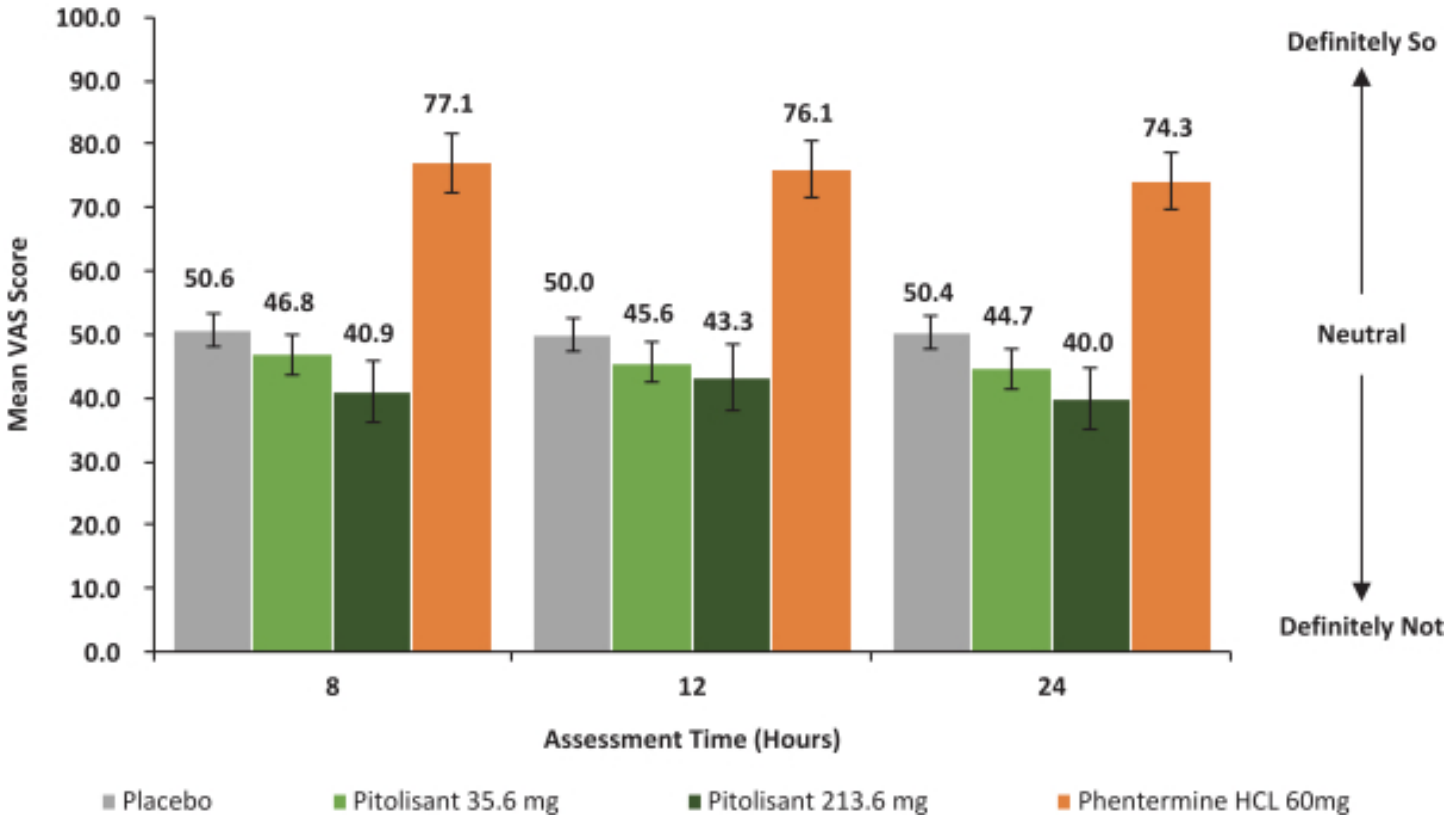
Overall Drug Liking



Take Drug Again

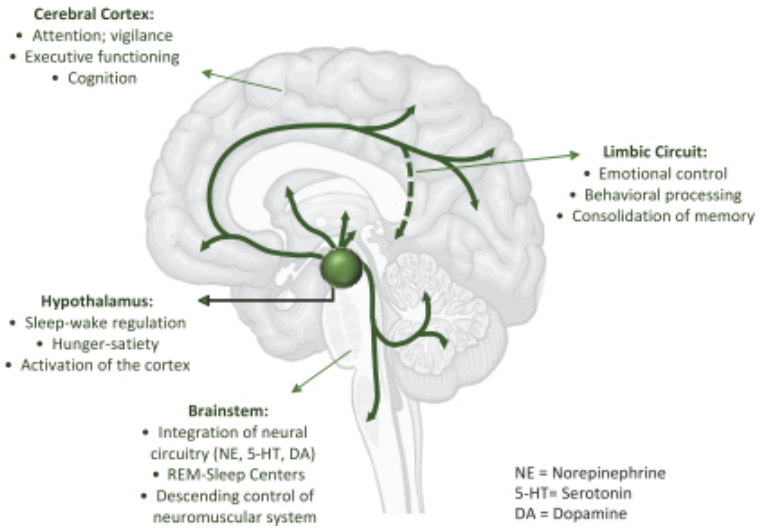


In summary, in the clinical HAP trial, pitolisant demonstrated a statistically significant and clinically relevant reduction in drug liking compared with phentermine as well as an overall response profile similar to placebo. Based on these clinical data, along with data from preclinical abuse liability studies, the evidence pointed to a low risk of abuse for pitolisant, which supported the approval of WAKIX without being scheduled as a controlled substance by the DEA.

Potential New Indications for Pitolisant

We are actively working on label expansion for WAKIX in narcolepsy, including indications for both EDS and cataplexy in pediatric patients. We also intend to work with the FDA toward gaining pediatric exclusivity for WAKIX. In addition, we are evaluating our options regarding the approach to take with the FDA in pursuit of a cataplexy indication in adult patients with narcolepsy. We believe that pitolisant's ability to regulate histamine and histaminergic signaling gives it the potential to provide therapeutic benefit in other disorders that are mediated through the H_3 receptor and histamine signaling. Histamine plays an important role in normal physiologic functioning beyond wakefulness in the areas of attention, vigilance, behavior and cognition. The presence of H_3 receptors in the hypothalamus, brainstem and cerebral cortex account for different functions, which could provide an opportunity for pitolisant to treat symptoms other than EDS in different disorders. In addition, H_3 receptors are located mainly in the CNS as opposed to other parts of the body outside the CNS. This fact, along with pitolisant being highly selective for the H_3 receptor (as opposed to H_1 receptors, H_2 receptors and H_4 receptors), is the reason, we believe, for pitolisant's unique MOA and why it works very differently than anti-histamines (peripheral H_1 receptor blockers) or anti-ulcer medications (H_2 receptor blockers).

- Role of histamine in normal physiologic functioning beyond wake promotion (e.g. attention, vigilance, behavior, cognition)
- Location of H_3 receptors in hypothalamus, brainstem, and cerebral cortex account for different functions (and potential symptoms in different disorders)
- Limited H_3 receptor populations outside the CNS



Our initial plan is to seek new indications in patient populations that have symptom overlap with narcolepsy, such as EDS. The initial clinical targets will focus on rare neurological disorders consistent with our overall strategy. We completed a Phase 1 PK clinical trial in pediatric patients with PWS in the fourth quarter of 2019 and initiated a long-term, open-label safety trial in these patients. We intend to commence a Phase 2 clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS in the first half of 2020 and anticipate topline results from this trial in the second half of 2021. We also anticipate commencing a Phase 2 clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with DM1 in the second half of 2020 with topline results anticipated in the first half of 2022. While conducting clinical programs to evaluate these indications, other clinical endpoints beyond EDS will be evaluated as secondary or exploratory endpoints, such as behavioral symptoms, vigilance, fatigue and cognition, to broaden the investigation of pitolisant with the hope of generating pilot data to help inform the next phase of our clinical development strategy.

Label Expansion in Narcolepsy

Cataplexy Indication

The NDA submission for WAKIX initially sought approval for the treatment of both EDS and cataplexy in adult patients with narcolepsy. Our application requesting approval for a cataplexy indication was based on our cataplexy results from HARMONY 1 and HARMONY CTP. The FDA approved WAKIX for the treatment of EDS in adult patients with narcolepsy but issued a CRL for the cataplexy indication, and therefore did not approve WAKIX for the treatment of cataplexy in adult patients with narcolepsy. The FDA determined that, although we had submitted one positive clinical trial for cataplexy, the NDA submission did not provide substantial evidence of effectiveness regarding cataplexy. Among other concerns, the FDA did not consider HARMONY 1 as an adequate and well-controlled trial for the cataplexy endpoint. The FDA found that cataplexy was a secondary endpoint in HARMONY 1, and there was no prospective plan to control the Type 1 error rate for secondary endpoints in this trial. The FDA also noted that the subgroup of interest (patients with cataplexy) was defined post hoc based on event(s) that occurred post-randomization. With regard to HARMONY CTP, the FDA considered it a positive trial, but the FDA commented that its design had certain weaknesses that do not render it the type of trial that could, on its own, provide sufficient evidence of effectiveness to support approval of the cataplexy indication, and the FDA generally requires two adequate and well-controlled clinical studies to support approval. The FDA therefore recommended that we conduct a second trial substantiating the results of HARMONY CTP in order to obtain approval for the cataplexy indication. We are evaluating our options regarding the approach to take with the FDA in order to obtain an indication for WAKIX for the treatment of cataplexy in adult patients with narcolepsy utilizing the data originally submitted in the NDA.

Pediatric Narcolepsy

Approximately 6% of diagnosed narcolepsy patients (approximately 4,500 patients) are under 18 years old. Symptoms often have a more profound effect in children, resulting in reduced function and greater psychological impact. Until the fourth quarter of 2018, no treatments were approved for pediatric narcolepsy, at which time Xyrem received an expanded indication for the treatment of cataplexy and EDS in patients seven years of age or older with narcolepsy. Bioprojet is conducting a Phase 2 clinical trial in pediatric patients with narcolepsy ages six to up to 18 years old with results expected in the first half of 2020. We plan to engage the FDA in the first of half of 2020 to discuss a pediatric development program, with the goal of pursuing a pediatric indication for both EDS and cataplexy, and pediatric exclusivity for WAKIX. This will require us to request the FDA to issue a Pediatric Written Request, which we would then need to follow in pursuit of pediatric exclusivity. We intend to commence a pivotal Phase 3 trial in pediatric patients in the second half of 2020, pending feedback from the FDA on the proposed trial design. Our current plan is to evaluate approximately 90 to 100 pediatric patients, ages six to up to 18, to assess the safety and efficacy of pitolisant in pediatric narcolepsy patients on improvement in both EDS and reduction in weekly rates of cataplexy.

Develop Pitolisant in New Patient Populations in Pursuit of Additional Indications

Prader-Willi Syndrome

PWS is a rare genetic disorder caused by a loss of function of specific genes on chromosome 15 resulting in hypothalamic dysfunction and decreased levels of hypocretin in some patients with PWS. The hypothalamus controls both sleep-wake states and hunger-satiety; therefore, two of the main symptoms in patients with PWS are EDS and hyperphagia. Other features include low muscle tone, short stature, behavioral problems and cognitive impairment. It is estimated that approximately one in 12,000 to 15,000 people in the United States suffers from PWS. In a recent study, more than 90% of PWS patients surveyed reported EDS. We completed a Phase 1 PK clinical trial in pediatric patients

with PWS in the fourth quarter of 2019, and initiated a long-term, open-label safety trial in these patients. We intend to commence a Phase 2 clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS in the first half of 2020 and anticipate topline results from this trial in the second half of 2021.

PWS poses a heavy burden for both patients and caregivers and there are few therapeutic options available. Current development programs are focused on hyperphagia, with no other programs focusing on EDS or cognitive function. We believe there is a compelling opportunity to impact the EDS component of this disorder as well as other symptoms, such as behavioral issues and cognitive function, for which the mechanism of action of pitolisant could be effective. We have collaborated with the Foundation for Prader-Willi Research, or the FPWR, to advance our clinical program and underscore our commitment to this patient population. We are members of the FPWR Clinical Trials Consortium and are working with members of its Scientific Advisory Board to gain their insights for our development program. Progress to date includes (i) the opening of an IND for PWS on October 28, 2019, (ii) the completion of a Phase 1 PK trial in patients with PWS in the fourth quarter of 2019, with patients actively rolling over into an open-label, long-term safety trial, (iii) the submission of a Phase 2 clinical protocol synopsis to the FDA for their review and comment, and (iv) plans underway to initiate a Phase 2 trial in the first half of 2020.

The proposed Phase 2 clinical trial is a randomized, double-blind, placebo-controlled trial to assess the safety and efficacy of pitolisant in patients with PWS ages 6 to 65. An estimated 60 to 70 patients will be enrolled at approximately 10 sites across the United States. Patients will be randomized to low-dose pitolisant, high-dose pitolisant or placebo in a 1:1:1 treatment ratio and titrated over three weeks up to their randomized dose, followed by eight weeks of stable dosing. The primary trial objective is to assess for improvement in EDS as measured by the Multiple Sleep Latency Test. Secondary endpoints include several behavioral symptom scales as well as specific measures of cognitive function using validated computer-based assessments. Clinician global impression of disease severity, caregiver global impression of EDS severity, and overall caregiver burden will be measured. Exploratory endpoints include the effect of pitolisant on hyperphagia and measurements of ghrelin levels. Patients who complete the trial will be eligible to participate in an open-label extension phase to assess the long-term safety and effectiveness of pitolisant in patients with PWS, which will run throughout the duration of the PWS development program.

Myotonic Dystrophy Type 1

DM1 is a rare, multi-system genetic disease that affects the neuromuscular system as well as several other systems. It is inherited in an autosomal dominant pattern and the underlying cause of DM1 is a mutation in the DMPK gene on chromosome 19. The prevalence of DM1 ranges from 2.1 to 14.3 per 100,000 people worldwide, with a prevalence in the United States of approximately 40,000 patients. The primary dysfunction is in the neuromuscular system, with hallmark symptoms of myotonia and progressive muscle weakness. Among the non-muscular symptoms of the disorder, EDS is the most common, with fatigue and cognitive dysfunction also pervasive. Together these non-muscular symptoms are viewed by patients and family members as prominent, debilitating features of the disorder that contribute to the overall disease burden and reduced independence of those afflicted. DM1 is another condition in which hypocretin levels have been identified as being below the normal range in some patients with this disorder. The lack of approved treatments for this disorder results in an unmet medical need for this patient population.

The therapeutic application of pitolisant may provide benefits across the key symptoms of EDS and fatigue which are often among the chief complaints of patients with DM1. In a survey of 451 DM1 patients, daytime sleepiness and fatigue were second only to muscle weakness in symptom prevalence and impact. Our clinical program will be designed to demonstrate effect on measures of

EDS and fatigue, as well as assess performance related to cognitive function, such as attention, vigilance and working memory. Progress to date includes working with KOLs to develop the scientific rationale for the investigation of pitolisant in patients with DM1, development of a draft Phase 2 clinical protocol synopsis, and submission of a pre-IND meeting request to the FDA in January 2020. A pre-IND meeting has been granted and scheduled for March 2020, during which we will discuss the clinical development program for DM1 with the FDA. Based on the outcome of this meeting, we plan to initiate a Phase 2 clinical trial in the second half of 2020, subject to receiving authorization to proceed from the FDA under an IND we plan to submit.

The proposed Phase 2 clinical trial is a randomized, double-blind, placebo-controlled trial to assess the safety and efficacy of pitolisant in patients with DM1 ages 18 to 65. An estimated 90 patients will be enrolled at approximately 10 sites across the United States. Patients will be randomized to low-dose pitolisant, high-dose pitolisant, or placebo in a 1:1:1 treatment ratio and titrated over three weeks up to their randomized dose, followed by eight weeks of stable dosing. The primary trial objective is to assess for improvement in EDS as measured by the ESS and other DM1 disease-specific scales. Secondary endpoints include assessments of fatigue as well as specific measures of cognitive function using validated computer-based assessments. Clinician and patient global impression of disease severity using the CGI-S and PGI-S, respectively, will be measured as well as patient assessments of overall disease burden. Plasma samples will be collected to generate pharmacokinetic data and a PK/PD analysis will be performed. Patients who complete the trial will be eligible to participate in an open-label extension phase to assess the long-term safety and effectiveness of pitolisant in patients with DM1, which will run throughout the duration of the DM1 development program.

Other Potential Indications

The next phase of clinical development for pitolisant will be guided by the signals generated from the clinical trials described above and other potential trials in PWS and DM1. If we observe favorable results in these trials on the symptoms of fatigue and cognitive dysfunction, we plan to investigate pitolisant in other rare neurological patient populations in which these symptoms are a prominent part of the disease process resulting in significant impact on daily functioning.

Manufacturing and Supply

We have secured a commercial drug supply to support the launch of WAKIX in the United States. Although we do not currently own or operate facilities for product manufacturing, storage and distribution, or testing, we have contracted directly with third parties for each of these functions.

Manufacturing is subject to extensive regulation that imposes various procedural and documentation requirements that govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance, and more. Our systems and our contractors are required to be in compliance with these regulations, and compliance is assessed regularly through monitoring of performance and a formal audit program.

Our current supply chains for WAKIX involve several manufacturers that specialize in specific operations of the manufacturing process, specifically, intermediate and starting material manufacturing, drug substance manufacturing, and drug product manufacturing labeling and secondary packaging, and distribution services:

- Interor S.A. manufactures our BF4 and BF6 intermediate and starting material used in the active pharmaceutical ingredient, or API.

- Corden Pharma Chenôve SAS, a full-service contract development and manufacturing organization, or CDMO, manufactures our API.
- Patheon UK Limited, a CDMO owned by Thermo Fisher Scientific Inc., manufactures our finished product tablets and fills them into unlabeled bottles.
- Carton Service, Inc., dba Pharma Packaging Solutions, handles our labeling and secondary packaging.
- Integrated Commercialization Solutions, LLC (ICS), a division of AmerisourceBergen Corporation, is our third-party logistics provider.
- Inmar Rx Solutions, Inc., an advanced technology and data analytics company, specializes in reverse distribution of our product and manages our pharmaceutical returns and product recall, if needed.

Competition

Our industry is highly competitive and subject to rapid and significant change as research provides a deeper understanding of rare neurological disorders, including narcolepsy, and as new therapies are developed. We face potential competition from multiple sources, including large pharmaceutical, biotechnology and specialty pharmaceutical companies. The key competitive factors affecting the success of WAKIX, and any other product candidates that we develop, if approved, are likely to be efficacy, safety, convenience, price, the level of generic competition and the availability of reimbursement from government and other third-party payors.

WAKIX competes with currently FDA-approved products for the treatment of EDS in adult patients with narcolepsy, all of which are controlled substances. Jazz Pharmaceuticals' Xyrem (sodium oxybate) is the only FDA-approved product for the treatment of EDS and cataplexy in adult patients with narcolepsy and, in October 2018, received FDA approval for an expanded indication in patients seven years and older for the treatment of cataplexy and EDS. Provigil and Nuvigil, which are WPAs, and stimulants such as methylphenidate and amphetamine, are approved for the treatment of EDS in narcolepsy. Anti-depressants and certain other agents are sometimes used off-label for the treatment of cataplexy in narcolepsy. Jazz Pharmaceuticals' Sunosi (solriamfetol) was approved by the FDA in March 2019 and launched in July 2019. Sunosi (solriamfetol) is a Schedule IV controlled substance and is indicated to improve wakefulness in adult patients with EDS associated with narcolepsy or obstructive sleep apnea. It is not indicated for cataplexy in patients with narcolepsy. Additionally, Jazz Pharmaceuticals is currently working on a lower/low sodium formulation of Xyrem, with an expected approval in the second half of 2020 or beyond and Avadel Pharmaceuticals is working on a once-per-night version of Xyrem (sodium oxybate), with approval expected in 2021 or beyond.

Strategic Agreement

License and Commercialization Agreement with Bioprojet

On July 28, 2017, we and Bioprojet entered into a license and commercialization agreement, or the Bioprojet License Agreement. Bioprojet granted to us an exclusive, sublicensable license to commercialize, in the United States and its territories, commonwealths, and protectorates, including Puerto Rico, a product containing pitolisant currently known as WAKIX for narcolepsy, obstructive sleep apnea, idiopathic hypersomnia, Parkinson's disease, and any other indication agreed upon by the parties (which currently include PWS and DM1), or the field, as well as rights to related patent rights, know-how, trademarks, trade dress, regulatory filings and approvals, or the Bioprojet Assets. Bioprojet also granted us a co-exclusive (with Bioprojet), sublicensable license to Bioprojet Assets to

clinically develop and register the pitolisant product in the field in the United States. Bioprojet retains the right to manufacture the product in the United States, and to develop outside the United States and commercialize other products that contain pitolisant as an active ingredient anywhere in the world. Bioprojet also granted us an exclusive license to use certain trademarks and trade names in connection with the commercialization of the product under the Bioprojet License Agreement.

Under the Bioprojet License Agreement, Bioprojet is responsible for conducting all preclinical and clinical studies necessary for achieving and maintaining regulatory approval in the United States for narcolepsy and cataplexy indications, including all costs and expenses. We are responsible for all other costs associated with other development and regulatory activities, unless Bioprojet otherwise agrees to participate in funding such activities. Bioprojet is responsible for filing, with our participation, the initial new drug application for the product with the FDA and is required to transfer such application to us upon approval by the FDA.

Upon approval by the FDA, we were required under the Bioprojet License Agreement to promptly launch the product and use commercially reasonable efforts to commercialize the approved products in the United States in the field for each approved indication. In addition, we are required to deploy a number of sales representatives and spend an amount of expenditure, each as agreed upon in a commercialization plan.

Under the Bioprojet License Agreement, Bioprojet has the right and authority to prepare, file, prosecute and maintain all Bioprojet patents on a worldwide basis at its own cost. Bioprojet shall keep us informed of the course of prosecution and other proceedings in the United States. We have the first right to enforce the licensed patent rights with respect to any infringing products in the United States. If we do not bring an action to enforce such patents against infringing activities that involve such infringing products, Bioprojet has the right to bring such action.

We paid Bioprojet an initial license fee of $150.0 million, a milestone payment of $50.0 million upon FDA acceptance of the NDA in February 2019, and a milestone payment of $75.0 million plus an addition $2.0 million fee for approval of the NDA in November 2019. We are subject to two further milestone payments: (i) a milestone payment of $40.0 million upon the attainment of aggregate net sales of WAKIX in the United States of $500.0 million subsequent to the date of NDA approval by the FDA and (ii) a milestone payment of $102.0 million if we receive NDA approval from the FDA for a cataplexy indication, which amount includes a $2.0 million extension fee. We agreed to pay royalties on the product at tiered royalty rates of 13 to 24% based on annual total net sales during the period commencing on first commercial sale of the product and ending on the latest of 10 years from first commercial sale of the product, expiration of all regulatory exclusivity, or expiration of the last Bioprojet patent covering the product. Such royalty payments are subject to reductions based on royalties paid to any third party in order for us to commercialize the product. We also agreed to pay royalties in consideration for a trademark license at a rate of 3% of net sales for 20 years after first commercial sale of the product. We further agreed to pay minimum royalties during the third through tenth year of the Bioprojet License Agreement if the product is approved for narcolepsy to the extent such minimum royalties exceed the royalties payable as described above, which minimum amounts were calculated based on sales materially below our sales forecast.

The Bioprojet License Agreement will continue until the expiration of the obligation to pay royalties with respect to the product. We and Bioprojet may each terminate the Bioprojet License Agreement for a material breach by the other party that remains uncured for 90 days. Bioprojet may terminate the Bioprojet License Agreement in its entirety if we or our sublicensees challenge the licensed patents. In addition, we and Bioprojet have the right to terminate the Bioprojet License Agreement upon the other party's insolvency.

Intellectual Property

Intellectual property, including patents, trade secrets, trademarks and copyrights, is important to our business. Our commercial success depends in part on our ability to obtain and maintain proprietary intellectual property protection for our WAKIX product and potential future pitolisant-based products, as well as for future product candidates and novel discoveries, product development technologies, and know-how. Our commercial success also depends in part on our ability to operate without infringing on the proprietary rights of others and to prevent others from infringing our proprietary rights. Our policy is to develop and maintain protection of our proprietary position by, among other methods, licensing or filing U.S. and foreign patents and applications relating to our technology, inventions, and improvements that are important to the development and implementation of our business.

Our patent portfolio comprises four U.S. patents exclusively licensed to us from Bioprojet. One U.S. patent has claims directed to a crystalline form of pitolisant and, methods for preparing the crystalline form of pitolisant which is expected to expire in February 2029 without taking into consideration any possible patent term extension. A second U.S. patent has claims directed to methods of treating excessive daytime sleepiness by administering pitolisant, which is expected to expire in September 2029 without taking into consideration any possible patent term extension. With all applicable patent term adjustments available and granted to us, the term of the last-to-expire pitolisant-related patent in our portfolio extends to September 2029. We may receive additional patent term based on the patent term extension described below.

The term of individual patents in our portfolio depends upon the legal term of patents in the countries in which they are obtained. In the United States, the patent term is 20 years from the earliest date of filing a non-provisional patent application. The term of a U.S. patent may be eligible for patent term adjustment, which permits patent term restoration as compensation for delays incurred at the USPTO during the patent prosecution process. In addition, for patents that cover an FDA-approved drug, the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration of the patent. While the length of the patent term extension is related to the length of time the drug is under regulatory review, patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, and only one patent per approved drug may be extended under the Hatch-Waxman Act. We have applied for patent term extension on two patents covering pitolisant, only one of which will receive patent term extension, if at all. There is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment of whether such extension should be granted, and if granted, the length of such extension.

The patent positions of companies like ours are generally uncertain and involve complex legal and factual questions. Changes in either the patent laws or their interpretation in the United States may diminish our ability to protect our technology or product candidates and could affect the value of such intellectual property. In particular, our ability to stop third parties from making, using, selling, offering to sell or importing products that infringe our intellectual property will depend in part on our success in enforcing patent claims that cover our technology, inventions and improvements. We cannot guarantee that patents will be granted with respect to any patent applications we may file in the future, nor can we be sure that any patents that may be granted to us in the future will be commercially useful in protecting our products, the methods of use or manufacture of those products. Moreover, issued patents do not guarantee the right to practice our technology in relation to the commercialization of our products. Issued patents only allow us to block potential competitors from practicing the claimed inventions of the issued patents.

Further, patents and other intellectual property rights in the pharmaceutical and biotechnology space are evolving and involve many risks and uncertainties. For example, third parties may have

blocking patents that could be used to prevent us from commercializing our product candidates and practicing our proprietary technology, and our issued patents may be challenged, invalidated or circumvented, which could limit our ability to stop competitors from marketing related products or could limit the term of patent protection that otherwise may exist for our product candidates. In addition, the scope of the rights granted under any issued patents may not provide us with protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may independently develop similar technologies that are outside the scope of the rights granted under any issued patents. For these reasons, we may face competition with respect to our product candidates. Moreover, because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that, before any particular product candidate can be commercialized, any patent protection for such product may expire or remain in force for only a short period following commercialization, thereby reducing the commercial advantage the patent provides.

We and/or our licensor also rely on protections under trade secret laws, and seek to protect and maintain the confidentiality of proprietary information to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. Our trade secrets include, for example, certain program specific synthesis, formulations, patient selection strategies and certain aspects of our research. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us, and for employees and consultants to enter into invention assignment agreements with us. These agreements provide that all confidential information developed or made known to the individual during the course of the individual's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. Where applicable, the agreements provide that all inventions to which the individual contributed as an inventor shall be assigned to us, and as such, will become our property. There can be no assurance, however, that these agreements will provide meaningful protection or adequate remedies for our trade secrets in the event of unauthorized use or disclosure of such information.

Further, we have in-licensed from Bioprojet the registered trademark product name "WAKIX" in the United States. We also have registered trademark protection in the United States for "KNOW NARCOLEPSY" and trademark applications pending with the U.S. Patent and Trademark Office for "REM AT THE WRONG TIME" and "NON-REM AT THE WRONG TIME" as well as our brand and logo "HB," "HB HARMONY BIOSCIENCES" and "HARMONY BIOSCIENCES."

Government Regulation

The FDA and comparable regulatory authorities in state and local jurisdictions and in other countries impose substantial and burdensome requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs, such as those we are developing. These agencies and other federal, state and local entities regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of our product candidates.

U.S. Drug Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or

after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending NDAs, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;
- submission to the FDA of an Investigational New Drug application, or IND, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practice, or GCP, requirements to establish the safety and efficacy of the proposed drug product for each indication;
- submission to the FDA of an NDA;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practice, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; and
- FDA review and approval of the NDA, including consideration of the views of any FDA advisory committee, prior to commercial marketing or sale of the drug in the United States; and
- Compliance with any post-approval requirements, including the potential requirement to implement a REMS program or to conduct a post-approval study.

Preclinical Studies

Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as animal studies to assess potential safety and efficacy. An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data and any available clinical data or literature, among other things, to the FDA as part of an IND. Some preclinical testing may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Clinical holds also may be imposed by the FDA at any time before or during clinical trials due to safety concerns about on-going or proposed clinical trials or non-compliance with specific FDA requirements, and the trials may not begin or continue until the FDA notifies the sponsor that the hold has been lifted. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical Trials

Clinical trials involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include the

requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution. Information about certain clinical trials must be submitted within specific timeframes to the NIH for public dissemination on their www.clinicaltrials.gov website.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase 1: The drug is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.
- Phase 2: The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- Phase 3: The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

The FDA or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. In addition, some clinical trials are overseen by an independent group of qualified experts organized by the sponsor, known as a data safety monitoring board or committee. Depending on its charter, this group may determine whether a trial may move forward at designated check points based on access to certain data from the trial.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug. In addition, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life. There are also requirements governing the reporting of ongoing clinical trials and completed trial results to public registries.

Marketing Approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. Under the Prescription Drug User Fee Act, or PDUFA, guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA for a new molecular entity to review and act on the submission. This review

typically takes twelve months from the date the NDA is submitted to FDA because the FDA has approximately two months to make a "filing" decision. Specifically, the FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety and quality.

The FDA also may require submission of a REMS to ensure that the benefits of the drug outweigh its risks. The REMS could include medication guides, physician communication plans, assessment plans, and/or elements to assure safe use, such as restricted distribution methods, patient registries, or other risk minimization tools.

The FDA may refer an application for a novel drug to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP requirements.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a complete response letter. A complete response letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for FDA to reconsider the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

The Pediatric Research Equity Act, or PREA, requires a sponsor to conduct pediatric clinical trials for most drugs, for a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration. Under PREA, original NDAs and supplements must contain a pediatric

assessment unless the sponsor has received a deferral or waiver. The required assessment must evaluate the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The sponsor or FDA may request a deferral of pediatric clinical trials for some or all of the pediatric subpopulations. A deferral may be granted for several reasons, including a finding that the drug is ready for approval for use in adults before pediatric clinical trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric clinical trials begin. The FDA must send a non-compliance letter to any sponsor that fails to submit the required assessment, keep a deferral current or fails to submit a request for approval of a pediatric formulation.

FDA Expedited Development and Review Programs

The FDA has various programs, including fast track designation, accelerated approval priority review, and breakthrough therapy designation, which are intended to expedite or simplify the process for the development and FDA review of drugs that are intended for the treatment of serious or life threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drugs to patients earlier than under standard FDA review procedures.

To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a product is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. The FDA will determine that a product will fill an unmet medical need if it will provide a therapy where none exists or provide a therapy that may be potentially superior to existing therapy based on efficacy or safety factors. The FDA may review sections of the NDA for a fast track product on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

The FDA may give a priority review designation to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. A priority review means that the goal for the FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. Under the new PDUFA agreement, these six and ten month review periods are measured from the “filing” date rather than the receipt date for NDAs for new molecular entities, which typically adds approximately two months to the timeline for review and decision from the date of submission. Products that are eligible for fast track designation may also be considered appropriate to receive a priority review.

In addition, products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be eligible for accelerated approval and may be approved on the basis of adequate and well-controlled clinical trials establishing that the drug product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require a sponsor of a drug receiving accelerated approval to perform post-marketing studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical endpoint, and the drug may be subject to accelerated withdrawal procedures.

Moreover, under the provisions of the Food and Drug Administration Safety and Innovation Act, or FDASIA, passed in July 2012, a sponsor can request designation of a product candidate as a

"breakthrough therapy." A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs designated as breakthrough therapies are also eligible for accelerated approval. The FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy. The designation includes all of the benefits of a fast track designation. The breakthrough therapy designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same drug if relevant criteria are met.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. Furthermore, fast track designation, priority review, and breakthrough therapy designation do not change the standards for approval, but may expedite the development or approval process.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;

- safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warning or other safety information about the product;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Marketing Exclusivity

Market exclusivity provisions under the FDCA can delay the submission or the approval of certain marketing applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not approve or even accept for review an abbreviated new drug application, or ANDA, or an NDA submitted under Section 505(b)(2), or 505(b)(2) NDA, submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovative drug or for another indication, where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with the FDA by the innovator NDA holder.

The FDCA alternatively provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA, if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application. This three-year exclusivity covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for drugs containing the active agent for the original indication or condition of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of marketing exclusivity available in the United States. Pediatric exclusivity provides for an additional six months of marketing exclusivity attached to another period of exclusivity if a sponsor conducts clinical trials in children in response to a written request from the FDA. The issuance of a written request does not require the sponsor to undertake the described clinical trials.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, which is a disease or condition that (i) affects fewer than 200,000 individuals in the United States, or (ii) if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making the product available in the United States for the disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting an NDA. After the FDA grants an orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity during which the FDA may not approve any other applications to market the same therapeutic agent for the same indication, except in limited circumstances, such as a subsequent product's showing of clinical superiority over the product with orphan exclusivity or where the original applicant cannot produce sufficient quantities of product. Competitors, however, may receive approval of different therapeutic agents for the indication for which the orphan product has exclusivity or obtain approval for the same therapeutic agent for a different indication than that for which the orphan product has exclusivity. Among other benefits of an orphan drug designation are tax credits for certain research and a waiver of the user fee for the NDA.

Orphan product exclusivity could block the approval of one of our products for seven years if a competitor obtains approval for the same therapeutic agent for the same indication before we do, unless we are able to demonstrate that our product is clinically superior. If an orphan designated product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan exclusivity. Further, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

We received an orphan designation for pitolisant for the treatment of narcolepsy and, upon approval of WAKIX, we received orphan exclusivity until 2026.

DEA Regulation

The Controlled Substances Act of 1970, or CSA, which establishes registration, security, recordkeeping, reporting, storage, distribution and other requirements administered by the DEA. The DEA is concerned with the control of handlers of controlled substances, and with the equipment and raw materials used in their manufacture and packaging, in order to prevent loss and diversion into illicit channels of commerce.

The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use, and may not be marketed or sold in the United States. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances. The FDA did not recommend that the DEA schedule WAKIX as a controlled substance, and WAKIX is therefore not scheduled as a controlled substance by the DEA.

Annual registration is required for any facility that manufactures, distributes, dispenses, imports or exports any controlled substance. The registration is specific to the particular location, activity and

controlled substance schedule. For example, separate registrations are needed for import and manufacturing, and each registration will specify which schedules of controlled substances are authorized.

The DEA typically inspects a facility to review its security measures prior to issuing a registration. Security requirements vary by controlled substance schedule, with the most stringent requirements applying to Schedule I and Schedule II substances. Required security measures include background checks on employees and physical control of inventory through measures such as cages, surveillance cameras and inventory reconciliations. Records must be maintained for the handling of all controlled substances, and periodic reports made to the DEA, for example distribution reports for Schedule I and II controlled substances, Schedule III substances that are narcotics, and other designated substances. Reports must also be made for thefts or losses of any controlled substance, and to obtain authorization to destroy any controlled substance. In addition, special authorization and notification requirements apply to imports and exports.

In addition, a DEA quota system controls and limits the availability and production of controlled substances in Schedule I or II. Distributions of any Schedule I or II controlled substance must also be accompanied by special order forms, with copies provided to the DEA. The DEA may adjust aggregate production quotas and individual production and procurement quotas from time to time during the year, although the DEA has substantial discretion in whether or not to make such adjustments. To meet its responsibilities, the DEA conducts periodic inspections of registered establishments that handle controlled substances. Individual states also regulate controlled substances.

Other Healthcare Laws

In addition to FDA regulation of pharmaceutical products, pharmaceutical companies are subject to federal healthcare laws and regulations as well as regulation by the states and foreign jurisdictions in which they conduct their business that restrict business practices in the pharmaceutical industry. These laws may impact, among other things, our current and future business operations, including our clinical research activities, and proposed sales, marketing and education programs and constrain the business or financial arrangements and relationships with healthcare providers and other parties through which we market, sell and distribute our products for which we obtain marketing approval. These laws include U.S. federal and state anti-kickback and false claims laws, civil monetary penalties laws, consumer protection and transparency laws as well as similar foreign laws in the jurisdictions outside the U.S., including, without limitation, those laws described below.

The federal Anti-Kickback Statute prohibits, among other things, individuals or entities from knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, overtly or covertly, in cash or in kind to induce or in return for purchasing, leasing, ordering or arranging for or recommending the purchase, lease or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act and the civil monetary penalties statute.

The federal civil and criminal false claims laws, including the civil False Claims Act, prohibit, among other things, any individual or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. Manufacturers can be held liable under the False Claims Act even when they

do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims. The False Claims Act also permits a private individual acting as a “whistleblower” to bring actions on behalf of the federal government alleging violations of the False Claims Act and to share in any monetary recovery.

The federal Civil Monetary Penalties Law prohibits, among other things, the offering or transferring of remuneration to a Medicare or Medicaid beneficiary that the person knows or should know is likely to influence the beneficiary’s selection of a particular supplier of Medicare or Medicaid payable items or services. Federal government price reporting laws require manufacturers to calculate and report complex pricing metrics to government programs.

The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, created additional federal criminal statutes that prohibit, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate it in order to have committed a violation. The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or other transfers of value made to physicians and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by physicians and their immediate family members.

Similar state and local laws and regulations may also restrict business practices in the pharmaceutical industry, such as state anti-kickback and false claims laws, which may apply to business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, or by patients themselves; state laws that require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing information and marketing expenditures or which require tracking gifts and other remuneration and items of value provided to physicians, other healthcare providers and entities; and state and local laws that require the registration of pharmaceutical sales representatives.

Violation of any of such laws or any other governmental regulations that apply may result in significant criminal, civil and administrative penalties including damages, fines, imprisonment, disgorgement, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, disgorgement, exclusion from participation in government healthcare programs and the curtailment or restructuring of our operations. To the extent that any of our products are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, implementation of corporate compliance programs, reporting of payments or transfers of value to healthcare professionals, and additional data privacy and security requirements.

Data Privacy and Security Laws

Pharmaceutical companies may be subject to U.S. federal and state health information privacy, security and data breach notification laws, which may govern the collection, use, disclosure and protection of health-related and other personal information. In the U.S., HIPAA imposes privacy, security and breach reporting obligations with respect to individually identifiable health information upon "covered entities" (health plans, health care clearinghouses and certain health care providers), and their respective business associates, individuals or entities that create, received, maintain or transmit protected health information in connection with providing a service for or on behalf of a covered entity. HIPAA mandates the reporting of certain breaches of health information to HHS, affected individuals and if the breach is large enough, the media. Entities that are found to be in violation of HIPAA as the result of a breach of unsecured PHI, a complaint about privacy practices or an audit by the Department of Health and Human Services, or HHS, may be subject to significant civil, criminal and administrative fines and penalties and/or additional reporting and oversight obligations if required to enter into a resolution agreement and corrective action plan with HHS to settle allegations of HIPAA non-compliance. Even when HIPAA does not apply, according to the Federal Trade Commission or the FTC, failing to take appropriate steps to keep consumers' personal information secure constitutes unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act, or the FTCA, 15 U.S.C § 45(a). The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. Individually identifiable health information is considered sensitive data that merits stronger safeguards. The FTC's guidance for appropriately securing consumers' personal information is similar to what is required by the HIPAA Security Rule.

In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which may be more stringent, broader in scope or offer greater individual rights with respect to protected health information, or PHI, than HIPAA, many of which may differ from each other, thus, complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties and private litigation.

European Union member states, the United Kingdom, Switzerland and other jurisdictions have also adopted data protection laws and regulations, which impose significant compliance obligations. In the EEA and the United Kingdom, the collection and use of personal data, including clinical trial data, is governed by the provisions of the General Data Protection Regulation, or GDPR. The GDPR, together with national legislation, regulations and guidelines of the EU member states and the United Kingdom governing the processing of personal data, impose strict obligations and restrictions on the ability to collect, analyze and transfer personal data, including health data from clinical trials and adverse event reporting. In particular, these obligations and restrictions concern the consent of the individuals to whom the personal data relates, the information provided to the individuals, the transfer of personal data out of the EEA or the United Kingdom, security breach notifications, security and confidentiality of the personal data and imposition of substantial potential fines for breaches of the data protection obligations. European data protection authorities may interpret the GDPR and national laws differently and impose additional requirements, which add to the complexity of processing personal data in or from the EEA or United Kingdom. Guidance on implementation and compliance practices are often updated or otherwise revised.

Coverage and Reimbursement

Sales of any pharmaceutical product depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement for such

product by third-party payors. In the United States, no uniform policy exists for coverage and reimbursement for pharmaceutical products among third-party payors. Therefore, decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. The process for determining whether a third-party payor will provide coverage for a product typically is separate from the process for setting the price of such product or for establishing the reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the FDA-approved products for a particular indication, or place products at certain formulary levels that result in lower reimbursement levels and higher cost-sharing obligation imposed on patients. One third-party payor's decision to cover a particular medical product or service does not ensure that other payors will also provide coverage for the medical product or service and the level of coverage and reimbursement can differ significantly from payor to payor. As a result, the coverage determination process will often require us to provide scientific and clinical support for the use of our products to each payor separately and can be a time-consuming process, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Additionally, a third-party payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved.

In international markets, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Pharmaceutical products may face competition from lower-priced products in foreign countries that have placed price controls on pharmaceutical products. Furthermore, there can be no assurance that a product will be considered medically reasonable and necessary for a specific indication, that a product will be considered cost-effective by third-party payors, that an adequate level of reimbursement will be established even if coverage is available or that the third-party payors' reimbursement policies will not adversely affect the ability to sell a product profitably.

Healthcare Reform

In the United States and certain foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system. In March 2010, the ACA was signed into law, which substantially changed the way healthcare is financed by both governmental and private insurers in the United States and significantly affected the pharmaceutical industry. The ACA contains a number of provisions, including those governing enrollment in federal healthcare programs, reimbursement adjustments and fraud and abuse changes. Additionally, the ACA increased the minimum level of Medicaid rebates payable by manufacturers of brand name drugs; required collection of rebates for drugs paid by Medicaid managed care organizations; required manufacturers to participate in a coverage gap discount program, under which they must agree to offer point-of-sale discounts (increased to 70 percent pursuant to the Bipartisan Budget Act of 2018, effective as of January 1, 2019) off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; imposed a non-deductible annual fee on pharmaceutical manufacturers or importers who sell certain "branded prescription drugs" to specified federal government programs, implemented a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted, or injected expanded the types of entities eligible for the 340B drug discount program; expanded eligibility criteria for Medicaid programs; creates a new Patient-Centered Outcomes

Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial, administrative, executive and Congressional legislative challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. Various portions of the ACA are currently undergoing constitutional challenges in the Fifth Circuit Court and the United States Supreme Court, the Trump Administration has issued various Executive Orders eliminating cost sharing subsidies and various provisions that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices, and Congress has introduced several pieces of legislation aimed at significantly revising or repealing the ACA. It is unclear whether the ACA will be overturned, repealed, replaced, or further amended, and we cannot predict what affect further changes to the ACA would have on our business.

Other legislative changes have been proposed and adopted since the ACA was enacted, including aggregate reductions of Medicare payments to providers of 2% per fiscal year and reduced payments to several types of Medicare providers. Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted legislation designed, among other things, to bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for pharmaceutical products. Further, the Trump Administration released a “Blueprint” to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The Trump administration’s budget proposal for fiscal year 2020 contains further drug price control measures that could be enacted during the 2020 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. HHS has begun implementation of the Trump administration Blueprint, soliciting feedback on some of these measures and, immediately implementing others under its existing authority. For example, in May 2019, CMS issued a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020.

Further, Congress has indicated that it will continue to seek new legislative measures to control drug costs. For example, on September 25, 2019, the Senate Finance Committee introduced the Prescription Drug Pricing Reduction Action of 2019, a bill intended to reduce Medicare and Medicaid prescription drug prices. The proposed legislation would restructure the Part D benefit, modify payment methodologies for certain drugs, and impose an inflation cap on drug price increases. An even more restrictive bill, the Lower Drugs Costs Now Act of 2019 has passed out of the House and was delivered to the Senate on December 16, 2019. If enacted as written, the Lower Drugs Costs Now Act would require HHS to directly negotiate drug prices with manufacturers. It is unclear whether either of these bills will make it through both chambers and be signed into law, and if either is enacted, what effect it would have on our business.

Individual states in the United States have also become increasingly active in implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures and, in some cases, mechanisms to encourage importation

from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine which drugs and suppliers will be included in their healthcare programs. Furthermore, there has been increased interest by third party payors and governmental authorities in reference pricing systems and publication of discounts and list prices.

Facilities

Our corporate headquarters are located 630 W. Germantown Pike, Suite 215, Plymouth Meeting, Pennsylvania, where we lease approximately 15,651 square feet of office space. Approximately 40 of our employees are located at our corporate headquarters. We also lease, pursuant to our Right of Use Agreement with Paragon, office space at 330 N. Wabash Ave, Suite 3500, Chicago, Illinois 60611, where eight of our employees are located.

Employees

As of December 31, 2019, we have approximately 150 employees, 100 of whom are dedicated to commercial functions, which includes sales, marketing, market access, commercial operations and insights, and 23 of whom are dedicated to research and development. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good. Also, pursuant to our Management Services Agreement with Paragon, at a given time up to six employees of Paragon assist us with regulatory, capital markets and legal transactional matters.

Legal Proceedings

From time to time, we may be subject to legal proceedings and claims in the ordinary course of business. The results of any current or future litigation cannot be predicted with certainty, and regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

MANAGEMENT

The following table provides information regarding our executive officers and members of our board of directors (ages as of the date of this prospectus):

Name	Age	Position(s)
Executive Officers		
John C. Jacobs	52	Chief Executive Officer, Director
Susan L. Drexler	50	Chief Financial Officer
Jeffrey Dayno, M.D.	62	Chief Medical Officer
Jeffrey Dierks	48	Chief Commercial Officer
Andrew Serafin	45	Chief Business Officer
Non-Employee Directors		
Jeffrey S. Aronin	51	Director, Chairman
Martin Edwards, MBChB	63	Director
Antonio Gracias	48	Director
Jack Bech Nielsen	55	Director
Aaron Royston, M.D.	35	Director
Juan A. Sabater	55	Director
Andreas Wicki, Ph.D.	60	Director

Executive Officers

John C. Jacobs. Mr. Jacobs has served as our Chief Executive Officer and on our board of directors since June 2018. Previously, Mr. Jacobs served as our Executive Vice President and Chief Commercial Officer from October 2017 to June 2018. Prior to joining us, Mr. Jacobs served as the Senior Vice President and General Manager of the Respiratory Business Unit of Teva Pharmaceuticals Industries Ltd., or Teva, a public pharmaceutical company, from September 2017 to October 2017. He also served as Senior Vice President of Commercial Operations and Innovation of Teva, from September 2016 to September 2017, and as Vice President and General Manager of Teva's Branded Business in Canada from July 2014 to September 2016. Mr. Jacobs has held positions of increasing scope and responsibility at major pharmaceutical companies including Cephalon Inc., a former public biopharmaceutical and biotechnology company, Wyeth, LLC, a public pharmaceutical company, and Pfizer Inc., a public pharmaceutical and biotechnology company. He has over 25 years of commercial, operations, business and leadership experience across multiple therapeutic areas including central nervous system, sleep disorders, pain care and respiratory, as well as rare disease and other specialty markets. Mr. Jacobs received a B.S. in business from State University of New York College at Plattsburgh and an M.B.A. from The State University of New York at Binghamton. We believe that Mr. Jacobs is qualified to serve on our board of directors due to his skills and experience in brand marketing in the biopharmaceutical industry.

Susan L. Drexler. Susan L. Drexler has served as our Chief Financial Officer since October 2019. From April 2018 to June 2019, Ms. Drexler served in various roles as the Interim Chief Financial Officer and Vice President of Business Development at Ocugen, Inc. From August 2015 to November 2017, Ms. Drexler served in senior roles in Business Development and Market Intelligence roles at AmerisourceBergen Corporation. From July 2007 to June 2015, Ms. Drexler held a senior development finance role at Shire Pharmaceuticals. Earlier in her career, Ms. Drexler held roles of increasing responsibility in finance consulting at Duff & Phelps, LLC and senior audit roles at PricewaterhouseCoopers LLP. Ms. Drexler earned a B.S. in Accounting from Albright College and an M.B.A. from the Joseph M. Katz Graduate School of Business at the University of Pittsburgh. Ms. Drexler is a Certified Public Accountant in the State of Pennsylvania.

Jeffrey Dayno, M.D. Dr. Dayno has served as our Chief Medical Officer since November 2017. Dr. Dayno also served as Chief Medical Officer of Eaglet Co., now known as Zyla Life Sciences, from July 2014 to October 2017. Prior to joining Eaglet Co., Dr. Dayno served as Vice President of Global Medical Affairs at ViroPharma, Inc., from August 2011 to January 2014, at which time it was acquired by Shire Pharmaceuticals. Since March 2016, Dr. Dayno has served on the board of directors of Atrin Pharmaceuticals, LLC, a private biopharmaceutical company. Dr. Dayno completed his residency in neurology at Temple University Hospital then completed a fellowship in stroke and cerebrovascular diseases at Henry Ford Hospital in Detroit, Michigan, as part of a National Institutes of Health program grant in stroke. He has over 10 years of experience in clinical and academic medicine and was on the faculty at Jefferson Medical College. Dr. Dayno also has over 20 years of experience in the pharmaceutical industry in leadership roles in companies including Merck & Co., Inc., a public pharmaceutical company, and Cephalon Inc., a formerly public biopharmaceutical and biotechnology company, which was acquired by Teva. He was one of the founding members and served as the Chairman of the Board of the Philadelphia Stroke Council, a non-profit organization dedicated to patient awareness and professional education to advance the efforts toward acute stroke treatment. Since March 2013, Dr. Dayno has been a member of the board of visitors of Temple University School of Medicine. Dr. Dayno received a B.A. in international studies from Trinity College and an M.D. from Temple University School of Medicine.

Jeffrey Dierks. Mr. Dierks has served as our Chief Commercial Officer since July 2018. Prior to his role as Chief Commercial Officer, Mr. Dierks served as our Vice President of Marketing from October 2017 to July 2018. Prior to joining Harmony, Mr. Dierks served in senior marketing roles leading the U.S. Pain Care & Wakefulness portfolio from June 2014 to December 2016 and U.S. Migraine Marketing from December 2016 to October 2017 at Teva Pharmaceuticals. Before joining Teva, Mr. Dierks held commercial roles of increasing responsibility at several major pharmaceutical companies, including Janssen Pharmaceuticals Inc., Endo Pharmaceuticals and Wyeth Pharmaceuticals. In 2017, PM360 magazine honored Mr. Dierks as a transformational leader in the pharmaceutical industry and in 2010 with the Trailblazer Award. Mr. Dierks has over 20 years of commercial experience and has led brand teams across numerous therapeutic areas including central nervous system, sleep disorders, pain care and migraines, as well as rare diseases. Mr. Dierks received a B.A. in political science from Western Maryland College and an M.B.A. in marketing from Temple University's Fox School of Business.

Andrew Serafin. Mr. Serafin has served as our Chief Business Officer since December 2018. Mr. Serafin previously served as our Senior Vice President of Business Development and Corporate Strategy from September 2017 to December 2018. Previously, Mr. Serafin served as the Vice President of Business Development at Marathon Pharmaceuticals, LLC, a private development-stage biopharmaceutical company, from August 2015 to May 2017. He also served as the Vice President of Business Development and General Counsel of AltaThera Pharmaceuticals, LLC, a private pharmaceutical company, from April 2015 to August 2015, and the Vice President of Deal Integration and Associate General Counsel of Lundbeck Inc., or Lundbeck, from July 2006 to March 2015. He also served as acting General Counsel of Lundbeck for six months during his time with the company. Mr. Serafin has over 20 years of experience in mergers and acquisitions and corporate legal counseling in the pharmaceutical, healthcare and technology sectors. He received a B.S. in finance from University of Illinois at Urbana-Champaign, a J.D. from Loyola University Chicago School of Law and an M.B.A. from Northwestern University Kellogg School of Management.

Directors

John C. Jacobs**.** Mr. Jacobs' business background information is set forth under "Executive Officers" above.

Jeffrey S. Aronin. Mr. Aronin founded Harmony Biosciences and has served on our board of directors and as non-executive Chairman since October 2017. In June 2017, Mr. Aronin founded

Paragon Biosciences which he leads as Chairman and Chief Executive Officer. Paragon Biosciences is a life science innovator that invests in, builds, and advises a portfolio of bioscience companies. In addition to serving on our board, Mr. Aronin serves on the boards of other Paragon privately-held portfolio companies, including Qlarity Imaging, LLC, which develops artificial intelligence-enabled diagnostic tools, Castle Creek Pharma, LLC, which is dedicated to rare genetic dermatology, Emalex Biosciences Inc., which is dedicated to treating neurological conditions, and Skyline Biosciences, LLC, which is dedicated to treating oncology conditions. From January 2011 to May 2017, Mr. Aronin was the Chairman and Chief Executive Officer of Marathon Pharmaceuticals, LLC, a private research-based biopharmaceutical company that developed drugs for rare diseases, which was subsequently acquired by PTC Therapeutics. Prior to that, Mr. Aronin founded Ovation Pharmaceuticals, Inc., or Ovation, where he served as President and Chief Executive Officer from 2000 to 2009. After Lundbeck A/S acquired Ovation in 2009, Mr. Aronin served as Chief Executive Officer of Lundbeck Inc. until 2011. Since June 2008, Mr. Aronin has served on the public board of directors of Discover Financial Services, Inc. Mr. Aronin also currently serves on the boards of several non-profit organizations including The Aspen Institute and MATTER, which Aronin founded to support life science innovation. Mr. Aronin received a B.S. in marketing from Northern Illinois University and an M.B.A. from DePaul University. We believe that Mr. Aronin is qualified to serve on our board of directors due to his vast skills and experience in biopharmaceutical strategy, innovation, business development, commercialization, lifecycle management, capital structure and finance.

Martin Edwards, MBChB. Dr. Edwards has served on our board of directors since August 2017. He has served in various roles and most recently as a Senior Partner at Novo Holdings A/S, a Danish private limited liability company, since October 2003. In this capacity, Dr. Edwards also serves on the boards of Nuvelution Pharma, Inc., Inozyme Pharma, Inc., Karus Ltd., F2G Ltd., and Vantia Therapeutics Ltd. He is also independent chairman of the board of directors of KalVista Pharmaceuticals, Inc. and an independent board member of Verona Pharma PLC, both public biopharmaceutical companies. Previously, Dr. Edwards served on the board of directors of CoLucid Pharmaceuticals, Inc., also a public biopharmaceutical company, from September 2015 to January 2017 and on the board of directors of private biotechnology companies. Dr. Edwards holds an MBChB from the University of Manchester and an M.B.A. from the University of Warwick. He is a member of the Royal College of Physicians, a member with distinction of the Royal College of General Practitioners and a Fellow of the Faculty of Pharmaceutical Medicine.

Antonio J. Gracias. Mr. Gracias has served on our board of directors since September 2017. Since September 2001, Mr. Gracias has been Chief Executive Officer and Chief Investment Officer of Valor Management LLC, or Valor, a private equity firm. Mr. Gracias has served as a director of Castle Creek Pharmaceuticals since September 2018. He also served as a director of Marathon Pharmaceuticals, LLC from November 2013 to December 2018 and SolarCity Corporation from 2012 to 2016. Mr. Gracias has served on the board of directors of Tesla, Inc., since May 2007, including as Lead Independent Director from September 2010 to April 2019. Mr. Gracias also serves as director of SpaceX. He has over 20 years of experience investing in a variety of sectors including private equity, public equity and real estate transactions. Mr. Gracias received a joint B.S. / M.S.F.S. degree in international finance and economics from Georgetown University School of Foreign Service and a J.D. from the University of Chicago Law School. We believe that Mr. Gracias is qualified to serve on our board of directors due to his skills and experience in investment strategy, portfolio company management and improvement, and finance in several industries, including pharmaceuticals and healthcare.

Jack B. Nielsen. Mr. Nielsen has served on our board of directors since September 2017. Mr. Nielsen has served as a Managing Director at Vivo Capital, LLC, a healthcare-focused investment firm, since August 2017, and as a consultant at Vivo Capital from March 2017 to July 2017. From April 2001 to February 2017, Mr. Nielsen worked within the Novo Holdings A/S venture activities in several

roles, most recently being employed as a Senior Partner. Mr. Nielsen has served on the board of directors of Reata Pharmaceuticals, Inc., a public pharmaceutical company, since June 2006. He has also served on the board of directors of Aligos Therapeutics, Inc. since August 2018 and MacuLogix, Inc. since March 2019. Mr. Nielsen previously served on the board of directors of public biotechnology companies including Crinetics Pharmaceuticals, Inc, Merus, N.V., Apollo Endosurgery, Inc. and Akebia Therapeutics, Inc. He also served on the board of directors of several private biotechnology and pharmaceutical companies including PROCEPT BioRobotics Co., Kanyos Bio, Inc., Unchained Labs, Inc., Anokion Therapeutics, Alios Biopharma, Inc. and ProteinSimple, Inc. Mr. Nielsen received a M.Sc. in chemical engineering from the Technical University of Denmark and a Masters in management of technology and economics from the Center for Technology, Economics and Management at the Technical University of Denmark. We believe that Mr. Nielsen is qualified to serve on our board of directors due to his experience as a venture capitalist and serving on various biotechnology and biopharmaceutical company boards.

Aaron Royston, M.D. Dr. Royston has served as a member of our board of directors since September 2017. Dr. Royston is a Partner at venBio Partners, a life sciences investment firm, and has been with venBio Partners since November 2015. Prior to joining venBio Partners, Dr. Royston worked for Vivo Capital, a global life sciences investment firm from July 2014 to November 2015. Previously, he worked at Bain & Company from July 2013 to July 2014, where he advised biotechnology companies on a broad range of strategic and operational issues. Earlier in his career, Dr. Royston coordinated clinical research at Mount Sinai Medical Center, where his research has been published and presented in multiple medical journals and conferences. In 2011, Dr. Royston was recognized by the Obama Administration as a Champion of Change for his work in technology and innovation. Dr. Royston serves on the board of directors of Akero Therapeutics, a public biotechnology company, and previously served on the board of Menlo Therapeutics, Inc., a public biotechnology company, and currently serves on the board of directors of several private companies. Dr. Royston received a B.S. in biological sciences from Duke University, and an M.D. and M.B.A. from the University of Pennsylvania. We believe that Dr. Royston is qualified to serve on our board of directors due to his clinical and biotechnology industry experience.

Juan A. Sabater. Mr. Sabater has served on our board of directors since 2017. Mr. Sabater has served in various roles at Valor since 2010, most recently as President. Prior to joining Valor, Mr. Sabater was a Managing Director of Goldman Sachs & Co. in their Investment Banking Division, from 1998 to 2006. He also currently serves on the board of several private companies and organizations including The Frick Collection and Girls Who Code Inc. Mr. Sabater currently serves as the Co-Chairman of Augeo Affinity Marketing, Inc., and also sits on the board of trustees of The Hewitt School. He received an A.B. in history from Princeton University and a J.D. from Stanford Law School. Mr. Sabater was also a former officer in the United States Army Reserve. We believe that Mr. Sabater is qualified to serve on our board of directors due to his expansive skillset including his management experience with a nationally recognized private equity firm and an investment banking company, along with his demonstrated business acumen.

Andreas Wicki, Ph.D. Dr. Wicki has served on our board of directors since September 2017. Dr. Wicki has served as Chief Executive Officer of HBM Healthcare Investments AG (formerly HBM BioVentures AG) since July 2001. From 1998 to 2001, Dr. Wicki was the Senior Vice President of the European Analytical Operations at MDS Inc. From 1990 to 1998, he was co-owner and Chief Executive Officer of ANAWA Laboratorien AG and Clinserve AG, two life sciences contract research companies. Dr. Wicki currently serves on the board of directors of Pacira BioSciences, Inc., a public pharmaceutical company, Buchler GmbH, HBM Healthcare Investments (Cayman) Ltd., HBM BioCapital Ltd., Viela Bio, Inc., a public clinical-stage biotechnology company, and Vitaeris, Inc., a private clinical-stage biopharmaceutical company. Dr. Wicki is a life sciences entrepreneur and

investor with over 20 years of experience in the pharmaceutical and biotechnology industries. Dr. Wicki holds an M.Sc. and Ph.D. in chemistry from the University of Bern, Switzerland. We believe Dr. Wicki is qualified to serve on our board of directors due to his extensive experience with pharmaceutical companies, his financial expertise and his years of experience providing strategic and advisory services to pharmaceutical and biotechnology organizations.

Family Relationships

There are no family relationships among any of our executive officers or directors.

Composition of our Board of Directors

Our board of directors currently consists of eight directors. Our amended and restated certificate of incorporation and amended and restated bylaws will provide that our board of directors may consist of up to directors and that our board of directors will be divided into three classes, as nearly equal in number as possible, with the directors in each class serving for a three-year term, and one class being elected each year by our stockholders. Dr. Edwards will resign as one of our directors immediately prior to the effectiveness of the registration statement on Form S-1, of which this prospectus forms a part.

When considering whether directors have the experience, qualifications, attributes or skills, taken as a whole, to enable our board of directors to satisfy its oversight responsibilities effectively in light of our business and structure, the board of directors focuses primarily on each person's background and experience as reflected in the information discussed in each of the directors' individual biographies set forth above. We believe that our directors provide an appropriate mix of experience and skills relevant to the size and nature of our business.

Director Independence

Prior to the consummation of this offering, our board of directors undertook a review of the independence of our directors and considered whether any director has a material relationship with us that could compromise that director's ability to exercise independent judgment in carrying out that director's responsibilities. Our board of directors has affirmatively determined that Messrs. , and are each an "independent director," as defined under the Exchange Act and the rules of .

Committees of Our Board of Directors

Our board of directors directs the management of our business and affairs, as provided by Delaware law, and conducts its business through meetings of the board of directors and standing committees. We will have a standing audit committee, nominating and corporate governance committee and compensation committee. In addition, from time to time, special committees may be established under the direction of the board of directors when necessary to address specific issues.

Audit Committee

Our audit committee will be responsible for, among other things:

- appointing, compensating, retaining, evaluating, terminating and overseeing our independent registered public accounting firm;
- discussing with our independent registered public accounting firm their independence from management;

- reviewing with our independent registered public accounting firm the scope and results of their audit;
- approving all audit and permissible non-audit services to be performed by our independent registered public accounting firm;
- overseeing the financial reporting process and discussing with management and our independent registered public accounting firm the interim and annual financial statements that we file with the SEC;
- reviewing and monitoring our accounting principles, accounting policies, financial and accounting controls and compliance with legal and regulatory requirements;
- reviewing our policies on risk assessment and risk management;
- reviewing related party transactions; and
- establishing procedures for the confidential anonymous submission of concerns regarding questionable accounting, internal controls or auditing matters.

Upon the consummation of this offering, our audit committee will consist of Messrs. , and , with Mr. serving as chair. Rule 10A-3 of the Exchange Act and the rules require that our audit committee have at least one independent member upon the listing of our common stock, have a majority of independent members within 90 days of the date of this prospectus and be composed entirely of independent members within one year of the date of this prospectus. Our board of directors has affirmatively determined that Messrs. , and each meet the definition of "independent director" for purposes of serving on the audit committee under Rule 10A-3 and the rules. Each member of our audit committee meets the financial literacy requirements of listing standards. In addition, our board of directors has determined that Mr. will qualify as an "audit committee financial expert," as such term is defined in Item 407(d)(5) of Regulation S-K. Our board of directors will adopt a new written charter for the audit committee, which will be available on our principal corporate website at *www.harmonybiosciences.com* substantially concurrently with the consummation of this offering. The information on or accessed through our website is deemed not to be incorporated in this prospectus or to be part of this prospectus.

Nominating and Corporate Governance Committee

Our nominating and corporate governance committee will be responsible for, among other things:

- identifying individuals qualified to become members of our board of directors, consistent with criteria approved by our board of directors;
- evaluating the overall effectiveness of our board of directors and its committees; and
- reviewing developments in corporate governance compliance and developing and recommending to our board of directors a set of corporate governance guidelines and principles.

Upon the consummation of this offering, our nominating and corporate governance committee will consist of Messrs. , and , with Mr. serving as chair. Our board of directors will adopt a new written charter for the nominating and corporate governance committee, which will be available on our principal corporate website at *www.harmonybiosciences.com* substantially concurrently with the consummation of this offering. The information on or accessed through our website is deemed not to be incorporated in this prospectus or to be part of this prospectus.

Compensation Committee

Our compensation committee will be responsible for, among other things:

- reviewing and approving the compensation of our directors, Chief Executive Officer and other executive officers; and
- appointing and overseeing any compensation consultants.

Upon the consummation of this offering, our compensation committee will consist of Messrs. , and , with Mr. serving as chair. Our board has determined that Messrs. , and are "non-employee directors" as defined in Section 16b-3 of the Exchange Act. Our board of directors will adopt a new written charter for the compensation committee, which will be available on our principal corporate website at *www.harmonybiosciences.com* substantially concurrently with the consummation of this offering. The information on or accessed through our website is deemed not to be incorporated in this prospectus or to be part of this prospectus.

Risk Oversight

Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including risks relating to our financial condition, development and commercialization activities, operations, strategic direction and intellectual property as more fully discussed under "Risk Factors" in this prospectus. Management is responsible for the day-to-day management of risks we face, while our board of directors, as a whole and through its committees, has responsibility for the oversight of risk management. In its risk oversight role, our board of directors has the responsibility to satisfy itself that the risk management processes designed and implemented by management are adequate and functioning as designed.

The role of the board of directors in overseeing the management of our risks is conducted primarily through committees of the board of directors, as disclosed in the descriptions of each of the committees above and in the charters of each of the committees. The full board of directors (or the appropriate board committee in the case of risks that are under the purview of a particular committee) discusses with management our major risk exposures, their potential impact on us, and the steps we take to manage them. When a board committee is responsible for evaluating and overseeing the management of a particular risk or risks, the chairman of the relevant committee reports on the discussion to the full board of directors during the committee reports portion of the next board meeting. This enables the board of directors and its committees to coordinate the risk oversight role, particularly with respect to risk interrelationships.

Risk Considerations in our Compensation Program

We conducted an assessment of our compensation policies and practices for our employees and concluded that these policies and practices are not reasonably likely to have a material adverse effect on our Company.

Compensation Committee Interlocks and Insider Participation

None of our executive officers serves as a member of the board of directors or compensation committee (or other committee performing equivalent functions) of any entity that has one or more executive officers serving on our board of directors or compensation committee.

Code of Ethics and Code of Conduct

Prior to the completion of this offering, we will adopt a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer,

principal financial officer, principal accounting officer or controller, or persons performing similar functions. A copy of the code will be posted on our website, *www.harmonybiosciences.com*. In addition, we intend to post on our website all disclosures that are required by law or the listing standards concerning any amendments to, or waivers from, any provision of the code. The information on or accessed through our website is deemed not to be incorporated in this prospectus or to be part of this prospectus.

EXECUTIVE COMPENSATION

This section discusses the material components of the executive compensation program for our executive officers who are named in the "2019 Summary Compensation Table" below. In 2019, our "named executive officers" and their positions were as follows:

- John C. Jacobs, Chief Executive Officer;
- Jeffrey Dayno, Chief Medical Officer;
- Andrew Serafin, Chief Business Officer; and
- John Vittoria, former Chief Financial Officer.

Mr. Vittoria served as our Chief Financial Officer from November 2018 until October 2019, and transitioned out of the Company in November 2019.

This discussion may contain forward-looking statements that are based on our current plans, considerations, expectations and determinations regarding future compensation programs. Actual compensation programs that we adopt following the completion of this offering may differ materially from the currently planned programs summarized in this discussion.

2019 Summary Compensation Table

The following table sets forth information concerning the compensation of our named executive officers for the year ended December 31, 2019.

Name and Principal Position	Salary ($)	Bonus ($)(1)	Option Awards ($)(2)	All Other Compensation ($)(3)	Total ($)
John C. Jacobs *Chief Executive Officer*	454,000	391,575	—	88	845,663
Jeffrey Dayno *Chief Medical Officer*	414,000	238,050	20,000	148	672,175
Andrew Serafin *Chief Business Officer*	340,500	195,788	—	754	537,042
John Vittoria *former Chief Financial Officer*	281,875	—	—	451,420	733,295

(1) Amounts reported include actual annual bonuses earned in 2019 under our annual bonus program to reward each of the named individuals' contributions to the Company in 2019. We provide additional information regarding the annual bonuses in "—Narrative to Summary Compensation Table—2019 Bonuses" below.

(2) Amounts reflect the full grant-date fair value of stock options granted during 2019 computed in accordance with ASC Topic 718, rather than the amounts paid to or realized by the named individual. We provide information regarding the assumptions used to calculate the value of all option awards made to executive officers in Note 3 to our financial statements included elsewhere in this prospectus.

(3) Amounts reported include Company-paid perquisites, gross-up payments to cover personal income taxes pertaining to Company-paid long-term disability coverage ($45, $106, $37 and $71 for Messrs. Jacob, Dayno, Serafin and Vittoria, respectively) and, with respect to Mr. Vittoria, severance benefits ($451,313).

Narrative to Summary Compensation Table

2019 Salaries

The named executive officers receive a base salary to compensate them for services rendered to our Company. The base salary payable to each named executive officer is intended to provide a fixed component of compensation reflecting the executive's skill set, experience, role and responsibilities.

The annual base salaries for Messrs. Jacobs, Dayno, Serafin and Vittoria for 2019 were $454,000, $414,000, $340,500 and $307,500, respectively. Effective January 1, 2020, the base salaries payable to Messrs. Jacobs, Dayno and Serafin increased by 4.2% to $473,068, $431,388 and $354,801, respectively.

2019 Bonuses

Under our annual bonus program, our board of directors may approve, in its discretion, annual cash bonuses based on its assessment of the applicable executive's performance for the year. In 2019, each of Messrs. Jacobs, Dayno and Serafin was eligible to earn a discretionary cash bonus targeted at $340,500, $207,000 and $170,250, respectively, to reward their contributions to the Company. In connection with Mr. Serafin's promotion to Chief Business Officer, the Company increased Mr. Serafin's target bonus opportunity from 40% to 50% of his base salary, effective January 1, 2019. For calendar year 2019, the actual annual cash bonuses earned by each of Messrs. Jacobs, Dayno and Serafin were $391,575, $238,050 and $195,788, respectively.

Each of these cash bonuses awarded to or earned by the named executive officers in 2019 are set forth above in the Summary Compensation Table in the column entitled "*Bonus*."

Equity Compensation

Certain of our named executive officers currently hold stock option awards under the Harmony Biosciences II, Inc. Equity Incentive Plan, or the Equity Incentive Plan. Specifically, in 2019, Mr. Dayno was granted stock options covering a number of shares of our common stock as set forth below. The options generally vest in equal installments on the first five anniversaries of the applicable vesting commencement date, subject to continued employment through the applicable vesting date, and accelerate in full upon a "change in control" (as defined in the Equity Incentive Plan). For additional information about the Equity Incentive Plan, please see the section titled "—Executive Compensation Plans—Equity Incentive Plan" below.

The following table sets forth the stock option awards granted to our named executive officers in the 2019 fiscal year.

Named Executive Officer	Number of Shares Subject to Options Granted in 2019
Jeffery Dayno	50,000

We intend to adopt a 2020 Incentive Award Plan, referred to below as the 2020 Plan, in order to facilitate the grant of cash and equity incentives to directors, employees (including our named executive officers) and consultants of our Company and certain of its affiliates and to enable our Company and certain of its affiliates to obtain and retain services of these individuals, which is essential to our long-term success. We expect that the 2020 Plan will be effective on the date on which it is adopted by our board of directors, subject to approval of such plan by our stockholders. For additional information about the 2020 Plan, please see the section titled "—Executive Compensation Plans—2020 Incentive Award Plan" below.

Other Elements of Compensation

Retirement Plans

We currently maintain a 401(k) retirement savings plan for our employees, including our named executive officers, who satisfy certain eligibility requirements. Our named executive officers are eligible to participate in the 401(k) plan on the same terms as other full-time employees. The Internal Revenue Code, or the Code, allows eligible employees to defer a portion of their compensation, within prescribed limits, on a pre-tax basis through contributions to the 401(k) plan. We believe that providing a vehicle for tax-deferred retirement savings though our 401(k) plan adds to the overall desirability of our executive compensation package and further incentivizes our employees, including our named executive officers, in accordance with our compensation policies. We did not make any matching contributions in 2019 under our 401(k) plan.

Employee Benefits and Perquisites

Health/Welfare Plans. All of our full-time employees, including our named executive officers, are eligible to participate in our health and welfare plans, including:

- medical, dental and vision benefits;
- medical and dependent care flexible spending accounts; and
- short-term and long-term disability insurance.

We also provide life insurance and accidental death and dismemberment insurance to our vice presidents and above, including our named executive officers, that is over and above the insurance provided to our full-time employees generally.

We believe the perquisites described above are necessary and appropriate to provide a competitive compensation package to our named executive officers.

Tax Gross-Ups

We make gross-up payments to cover the personal income taxes of our full-time employees, including our named executive officers that pertain to the Company-paid long-term disability coverage provided by us.

Outstanding Equity Awards at Fiscal Year-End

The following table summarizes the number of shares of common stock underlying outstanding equity incentive plan awards for each named executive officer as of December 31, 2019.

			Option Awards			
Name	Grant Date	Vesting Commencement Date (1)	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price ($)	Option Expiration Date
John Jacobs	10/2/2017	10/1/2017	1,034,273	1,551,410	$ 1.00	10/2/2027
	10/1/2018	10/1/2018	200,000	800,000	$ 1.00	10/1/2028
Jeffery Dayno	11/13/2017	11/1/2017	340,000	510,000	$ 1.00	11/13/2028
	1/7/2019	1/1/2019	—	50,000	$ 1.00	1/7/2029
Andrew Serafin	10/1/2017	10/1/2017	400,000	600,000	$ 1.00	10/1/2027
	10/1/2018	10/1/2018	30,000	120,000	$ 1.00	10/1/2028
John Vittoria	11/14/2018	11/1/2018	80,000	—	$ 1.00	2/28/2020

(1) 20% of the shares of our common stock underlying the stock options vest and become exercisable annually on the first five anniversaries of the vesting commencement date, subject to continued employment through the applicable vesting date, and accelerate in full upon the occurrence of a “change in control” (as defined in the Equity Incentive Plan).

Executive Compensation Arrangements

The following summarizes the material terms of the employment offer letters and employment agreements with each of our named executive officers.

John C. Jacobs Employment Agreement

On September 6, 2017, we entered into an employment agreement with John C. Jacobs. Under the agreement, Mr. Jacobs' employment will continue until terminated upon written notice by either party in accordance with the employment agreement.

Pursuant to his employment agreement, Mr. Jacobs is entitled to receive an annual base salary of $400,000 per year; as noted above, Mr. Jacobs' 2019 annual base salary was $454,000. In addition, Mr. Jacobs (and his spouse and/or eligible dependents) are eligible to participate in the health and welfare benefit plans and programs maintained by us for the benefit of our employees with comparable responsibilities.

Mr. Jacobs is eligible to earn annual discretionary cash bonuses, determined by our board of directors (or a subcommittee thereof) in its sole discretion based on its assessment of individual and our performance. Mr. Jacobs' target bonus and maximum bonus opportunities are 50% and 75%, respectively, of his annual base salary. The payment of any annual bonus, to the extent any annual bonus becomes payable, will be contingent upon Mr. Jacob's continued employment through the applicable payment date.

In connection with entering into his employment agreement, Mr. Jacobs was awarded a stock option to purchase 2,585,683 shares of our common stock. The option vests as to 20% of the shares underlying the option on each of the first five anniversaries of the grant date, subject to Mr. Jacobs continued employment with the Company through each applicable vesting date, provided, that upon a "change in control" (as defined in Mr. Jacobs' employment agreement), Mr. Jacobs' stock option will accelerate and vest in full subject to his continued employment through such date.

Under his employment agreement, if Mr. Jacobs' employment is terminated without "cause" or due to his resignation for "good reason" (each, as defined in his employment agreement), then, subject to his timely execution and non-revocation of a general release of claims, he will be eligible to receive (i) 12 months of continued payment of base salary; (ii) 12 months of continued coverage under our group health plans at the same level and cost to Mr. Jacobs as was in place prior to the termination date; and (iii) up to three months of outplacement services. If either such termination occurs within 12 months following a "change in control," then, in addition to the payments and benefits described above, Mr. Jacobs will receive a lump-sum cash payment equal to his target annual bonus for the year in which the termination occurs, pro-rated through the date of such termination.

Mr. Jacobs' employment agreement contains customary confidentiality provisions, as well as standard non-compete and employee non-solicitation restrictions effective during employment and for one year thereafter. Mr. Jacobs' employment agreement includes a "best pay" provision under Section 280G of the Code, pursuant to which any "parachute payments" that become payable to him will be reduced so that such payments are not subject to the excise tax under Section 4999 of the Code.

Jeffrey Dayno Offer Letter

On October 10, 2017, we entered into an offer letter with Jeffrey Dayno. Mr. Dayno's employment under the offer letter is at-will, and will continue until terminated at any time by either party.

Pursuant to his offer letter, Mr. Dayno is entitled to receive an annual base salary of $400,000 per year; as noted above, Mr. Dayno's 2019 annual base salary was $414,000. In addition, Mr. Dayno is eligible to participate in the health and welfare benefit plans and programs maintained by us for the benefit of our employees.

Mr. Dayno is eligible to earn annual cash bonuses under our bonus program, based on the achievement of individual performance goals relating to our growth and overall performance. Mr. Dayno's target bonus opportunity is 50% of his annual base salary. The payment of any annual bonus, to the extent any such bonus becomes payable, will be contingent upon Mr. Dayno's continued employment through the applicable payment date.

In connection with entering into his offer letter, Mr. Dayno was awarded a stock option to purchase 850,000 shares of our common stock. The option vests as to 20% of the shares underlying the option on each of the first five anniversaries of Mr. Dayno's employment start date, subject to his continued employment with the Company through each applicable vesting date, provided, that upon a "change in control" (as defined in the Equity Incentive Plan), Mr. Dayno's stock option will accelerate and vest in full subject to his continued employment through such date.

Andrew Serafin Offer Letter

On September 8, 2017, we entered into an offer letter with Andrew Serafin. Mr. Serafin's employment under the offer letter is at-will, and will continue until terminated at any time by either party.

Pursuant to this offer letter, Mr. Serafin is entitled to receive an annual base salary of $300,000 per year; as noted above, Mr. Serafin's 2019 annual base salary was $340,500. In addition, Mr. Serafin is eligible to participate in the health and welfare benefit plans and programs maintained by us for the benefit of our employees.

Mr. Serafin is eligible to earn annual cash bonuses under our bonus program, based on the achievement of individual performance goals relating to our growth and overall performance. Pursuant to his offer letter, Mr. Serafin's target bonus opportunity is up to 40% of his annual base salary, and, as noted above, Mr. Serafin's 2019 target bonus opportunity was increased to 50% of his annual base salary. The payment of any annual bonus, to the extent any such bonus becomes payable, will be contingent upon Mr. Serafin's continued employment through the applicable payment date.

In connection with entering into his offer letter, Mr. Serafin was awarded a stock option to purchase 1,000,000 shares of our common stock. The option vests as to 20% of the shares underlying the option on each of the first five anniversaries of the grant date, subject to Mr. Serafin's continued employment with the Company through each applicable vesting date, provided, that upon a "change in control" (as defined in the Equity Incentive Plan), Mr. Serafin's stock option will accelerate and vest in full subject to his continued employment through such date.

John Vittoria Offer Letter; Separation Arrangement

On September 29, 2018, we entered into an offer letter with John Vittoria. Mr. Vittoria left the Company on November 30, 2019.

Pursuant to this offer letter, Mr. Vittoria was entitled to receive an annual base salary of $300,000 per year; as noted above, Mr. Vittoria's 2019 annual base salary was $307,500. In addition, Mr. Vittoria was eligible to participate in the health and welfare benefit plans and programs maintained by us for the benefit of our employees.

Mr. Vittoria was eligible to earn annual cash bonuses under our bonus program, based on the achievement of individual performance goals relating to our growth and overall performance. Mr. Vittoria's target bonus opportunity was 40% of his annual base salary.

In addition, under his offer letter, Mr. Vittoria was eligible to receive a relocation allowance of $45,000 in connection with his relocation from New York to Philadelphia to begin his employment with us. This relocation allowance was paid in a lump sum to Mr. Vittoria in calendar year 2018; provided that Mr. Vittoria did not voluntarily terminate his employment with us on or prior to the first anniversary of his start date, or November 12, 2019. Mr. Vittoria was not required to pay back the relocation allowance to the Company in connection with his separation.

In connection with his offer letter, Mr. Vittoria was awarded a stock option to purchase 400,000 shares of our common stock. As of Mr. Vittoria's termination date, 80,000 shares subject to his option were vested and unexercised and these vested shares will remain outstanding and exercisable until February 28, 2020. The remaining shares underlying Mr. Vittoria's stock option were cancelled and forfeited.

In addition, in connection with Mr. Vittoria's departure from the Company, as noted above, Mr. Vittoria received the following severance benefits: (i) an aggregate amount equal to his annual base salary, payable in a lump sum; (ii) 12 months of continued coverage under our group health plans at the same level and cost to Mr. Vittoria as was in place prior to the termination date; and (iii) a pro-rated 2019 bonus.

Director Compensation

In 2019, we did not provide compensation to our non-employee directors.

However, in connection with this offering, we intend to approve and implement a compensation program for our non-employee directors that consists of annual retainer fees and long-term equity awards. The program is expected to provide directors with a , subject to continued service on our board of directors. Each is expected to be denominated as a . In addition, each non-employee director is expected to receive an annual cash retainer for his or her services in an amount equal to $ and an annual equity award in a denominated dollar value equal to $.

Executive Compensation Plans

The following summarizes the material terms of the long-term incentive compensation plan in which our NEOs will be eligible to participate following the consummation of this offering and the Equity Incentive Plan under which we have previously made periodic grants of equity and equity-based awards to our NEOs and other key employees.

Equity Incentive Plan

Our board of directors and our stockholders approved the Equity Incentive Plan on August 7, 2017.

Under the Equity Incentive Plan, 35,496,000 shares of our common stock are reserved for issuance under the plan. The maximum amount of shares that may be granted with respect to stock option awards and/or stock appreciation rights, or SARs, under the Equity Incentive Plan is 13,333,000 and no more than 3,549,600 shares may be granted to any one participant with respect to incentive stock options, or ISOs. The Equity Incentive Plan will expire in December 2027 unless earlier terminated by our board of directors. Following the effectiveness of the 2020 Plan, the Equity Incentive Plan will terminate and we will not make any further awards under the Equity Incentive Plan. However, any outstanding awards granted under the Equity Incentive Plan will remain outstanding, subject to the terms of the Equity Incentive Plan and applicable award agreement. Shares of our common stock subject to awards granted under the Equity Incentive Plan that expire, lapse or are terminated, exchanged for or settled in cash, surrendered, repurchased, canceled without having been fully exercised or forfeited following the effective date of the 2020 Plan will become available for issuance under the 2020 Plan in accordance with its terms.

Administration. The board of directors (or the compensation committee) administers the Equity Incentive Plan. Subject to the provisions of the Equity Incentive Plan, the administrator has the authority to designate the persons to whom awards are to be made; determine the types of awards to grant; determine the number of shares to be subject to such awards; determine the terms and conditions of any award; grant fully-vested awards; determine whether, and under what circumstances, awards may be settled or exercised in cash, shares, awards or other property; interpret, administer or reconcile any consistency or defect in the Equity Incentive Plan or in any award agreement; establish, amend, suspend or waive any rules and regulations and appoint such agents as the administrator shall deem appropriate to administer the Equity Incentive Plan; accelerate the vesting or exercisability of awards; and make any other determination and take any other action that the administrator deems necessary or desirable for the administration of the Equity Incentive Plan.

Eligibility. Awards under the Equity Incentive Plan may be granted to individuals who are our current or prospective employees, consultants and members of our board of directors.

Awards. The Equity Incentive Plan permits the award of stock options, including ISOs and nonqualified stock options, or NSOs, SARs, restricted stock, restricted stock units, or RSUs, and stock bonuses. To date, only stock options and restricted stock have been granted under the Equity Incentive Plan and only awards of stock options remain outstanding.

- *Stock Options and SARs*. Stock options provide for the purchase of shares of our common stock in the future at an exercise price set on the grant date. ISOs, in contrast to NSOs, may provide tax deferral beyond exercise and favorable capital gains tax treatment to their holders if certain holding period and other requirements of the Code are satisfied. SARs entitle their holder, upon exercise, to receive from us an amount equal to the appreciation of the shares subject to the award between the grant date and the exercise date. The exercise price of a stock option or SAR may not be less than 100% of the fair market value of the underlying share on the grant date (or 110% in the case of ISOs granted to certain significant stockholders), except with respect to certain substitute awards granted in connection with a corporate transaction. The term of a stock option or SAR may not be longer than ten years (or five years in the case of ISOs granted to certain significant stockholders).
- *Restricted Stock*. Restricted stock is an award of nontransferable shares of our common stock that are subject to certain vesting conditions and other restrictions. Participants granted restricted stock under the Equity Incentive Plan may, to the extent applicable, have the right to vote such stock and to receive dividends with respect to such stock.
- *RSUs*. RSUs are contractual promises to deliver shares of our common stock in the future, which may also remain forfeitable unless and until specified conditions are met and may

be accompanied by the right to receive the equivalent value of dividends paid on shares of common stock prior to the delivery of the underlying shares (i.e., dividend equivalent rights). The plan administrator may provide that the delivery of the shares underlying RSUs will be deferred on a mandatory basis or at the election of the participant. The terms and conditions applicable to RSUs will be determined by the plan administrator, subject to the conditions and limitations contained in the Equity Incentive Plan.

- *Stock Bonus Awards*. Stock bonus awards are awards of fully vested shares of our common stock and awards denominated in the shares of our common stock, each of which may be granted to participants and may also be available as a payment form in the settlement of other awards, as standalone payments and as payment in lieu of compensation to which a participant is otherwise entitled.
- *Dividends and Dividend Equivalents*. Dividends and dividend equivalents represent the right to receive the equivalent value of dividends paid on shares of our common stock and may be granted alone or in tandem with awards of RSUs or restricted stock. Dividends and dividend equivalents are credited as of the dividend record dates during the period between the date an award is granted and the date such award vests, is exercised, is distributed or expires, as determined by the plan administrator.

Corporate Transactions. In the event of a “change in control” (as defined in the Equity Incentive Plan), all outstanding awards will be subject to the terms of the applicable award agreement or, if such treatment is not specified in the applicable award agreement, the applicable merger, purchase or reorganization agreement. All of the award agreements underlying the outstanding option awards provide for full acceleration on a change in control. In addition, in the event of a corporate transaction or change in capital structure, the board of directors may provide for the equitable adjustment of the terms of outstanding awards, the substitution, assumption or termination of all outstanding awards, or cancellation of all outstanding awards in exchange for a cash payment in an amount equal to the fair market value of the shares of our common stock subject to the awards immediately prior to the consummation of such transaction (less any exercise price, as applicable). Prior to any such adjustment, the Company will give notice of such adjustment to the participants holding outstanding awards.

Amendment or Termination of the Equity Incentive Plan and Awards Thereunder. Our board of directors may terminate, amend or modify the Equity Incentive Plan at any time, subject to the written consent of any participant whose rights under the plan would be materially and adversely affected as a result of such termination, amendment or modification. However, to the extent necessary to comply with any applicable law or stock exchange rule, stockholder approval of any amendment or modification to an award must be obtained to reduce the option price per share after the option has been granted or to substitute any outstanding option or SAR award. As described above, the Equity Incentive Plan will terminate as of the effective date of the 2020 Plan.

2020 Incentive Award Plan

We intend to adopt the 2020 Incentive Award Plan, or the 2020 Plan, subject to approval by our stockholders, under which we may grant cash and equity incentive awards to eligible service providers in order to attract, motivate and retain the talent for which we compete. The material terms of the 2020 Plan, as it is currently contemplated, are summarized below. Our board of directors is still in the process of developing, approving and implementing the 2020 Plan and, accordingly, this summary is subject to change.

Eligibility and Administration. Our employees, consultants and directors, and employees, consultants and directors of our subsidiaries, will be eligible to receive awards under the 2020 Plan.

Following our initial public offering, the 2020 Plan will be administered by our board of directors with respect to awards to non-employee directors and by our compensation committee with respect to other participants, each of which may delegate its duties and responsibilities to committees of our board of directors and/or officers (referred to, collectively, as the plan administrator below), subject to certain limitations that may be imposed under the 2020 Plan, Section 16 of the Exchange Act, and/or stock exchange rules, as applicable. The plan administrator will have the authority to make all determinations and interpretations under, prescribe all forms for use with, and adopt rules for the administration of, the 2020 Plan, subject to its express terms and conditions. The plan administrator will also set the terms and conditions of all awards under the 2020 Plan, including any vesting and vesting acceleration conditions.

Shares Available. An aggregate of shares of our common stock will be available for issuance under awards granted pursuant to the 2020 Plan, which shares may be authorized but unissued shares, or shares purchased in the open market. Notwithstanding anything to the contrary in the 2020 Plan, no more than shares of our common stock may be issued pursuant to the exercise of ISOs under the 2020 Plan.

The number of shares available for issuance will be increased by (i) the number of shares of common stock that remain available for issuance under the Equity Incentive Plan as of the effective date of the 2020 Plan, (ii) the number of shares represented by awards outstanding under our Equity Incentive Plan that expire, lapse or are terminated, exchanged for or settled in cash, surrendered, repurchased, canceled without having been fully exercised or forfeited following the effective date of the 2020 Plan, with the maximum number of shares to be added to the 2020 Plan pursuant to clauses (i) and (ii) above equal to shares, and (iii) an annual increase on the first day of each calendar year beginning January 1, 2021 and ending on and including January 1, 2030, equal to the lessor of (A) of the aggregate number of shares of common stock outstanding on the final day of the immediately preceding calendar year and (B) such smaller number of shares as is determined by our board of directors.

If an award under the 2020 Plan expires, lapses or is terminated, exchanged for or settled in cash, surrendered, repurchased, canceled without having been fully exercised or forfeited, any shares subject to such award may, to the extent of such forfeiture, expiration or cash settlement, be used again for new grants under the 2020 Plan. Further, shares delivered to us to satisfy the applicable exercise or purchase price of an award under the 2020 Plan or the Equity Incentive Plan and/or to satisfy any applicable tax withholding obligations (including shares retained by us from the award under the 2020 Plan or the Equity Incentive Plan being exercised or purchased and/or creating the tax obligation) will become or again be available for award grants under the 2020 Plan. The payment of dividend equivalents in cash in conjunction with any awards under the 2020 Plan will not reduce the shares available for grant under the 2020 Plan. However, the following shares may not be used again for grant under the 2020 Plan: (i) shares subject to SARs that are not issued in connection with the stock settlement of the SAR on exercise, and (ii) shares purchased on the open market with the cash proceeds from the exercise of options.

Awards granted under the 2020 Plan upon the assumption of, or in substitution for, awards authorized or outstanding under a qualifying equity plan maintained by an entity with which we enter into a merger or similar corporate transaction will not reduce the shares available for grant under the 2020 Plan but will count against the maximum number of shares that may be issued upon the exercise of ISOs.

The 2020 Plan provides that the sum of any cash compensation and the aggregate grant date fair value (determined as of the date of the grant under ASC Topic 718, or any successor thereto) of all awards granted to a non-employee director as compensation for services as a non-employee director during any calendar year may not exceed the amount equal to $750,000, increased to $1,000,000, in the fiscal year of a non-employee director’s initial service as a non-employee director.

Awards. The 2020 Plan provides for the grant of stock options, including ISOs and NSOs, SARs, restricted stock, dividend equivalents, RSUs and other stock or cash based awards. Certain awards under the 2020 Plan may constitute or provide for a deferral of compensation, subject to Section 409A of the Code, which may impose additional requirements on the terms and conditions of such awards. All awards under the 2020 Plan will be evidenced by award agreements, which will detail all terms and conditions of the awards, including any applicable vesting and payment terms and post-termination exercise limitations. Awards other than cash awards generally will be settled in shares of common stock, but the plan administrator may provide for cash settlement of any award. A brief description of each award type follows.

- *Stock Options and SARs*. Stock options provide for the purchase of shares of our common stock in the future at an exercise price set on the grant date. ISOs, in contrast to NSOs, may provide tax deferral beyond exercise and favorable capital gains tax treatment to their holders if certain holding period and other requirements of the Code are satisfied. SARs entitle their holder, upon exercise, to receive from us an amount equal to the appreciation of the shares subject to the award between the grant date and the exercise date. The exercise price of a stock option or SAR may not be less than 100% of the fair market value of the underlying share on the grant date (or 110% in the case of ISOs granted to certain significant stockholders), except with respect to certain substitute awards granted in connection with a corporate transaction. The term of a stock option or SAR may not be longer than ten years (or five years in the case of ISOs granted to certain significant stockholders).
- *Restricted Stock*. Restricted stock is an award of nontransferable shares of our common stock that are subject to certain vesting conditions and other restrictions.
- *RSUs*. RSUs are contractual promises to deliver shares of our common stock in the future, which may also remain forfeitable unless and until specified conditions are met and may be accompanied by the right to receive the equivalent value of dividends paid on shares of common stock prior to the delivery of the underlying shares (i.e., dividend equivalent rights). The plan administrator may provide that the delivery of the shares underlying RSUs will be deferred on a mandatory basis or at the election of the participant. The terms and conditions applicable to RSUs will be determined by the plan administrator, subject to the conditions and limitations contained in the 2020 Plan.
- *Other Stock or Cash Based Awards*. Other stock or cash based awards are awards of cash, fully vested shares of our common stock and other awards valued wholly or partially by referring to, or otherwise based on, shares of our common stock. Other stock or cash based awards may be granted to participants and may also be available as a payment form in the settlement of other awards, as standalone payments and as payment in lieu of compensation to which a participant is otherwise entitled.
- *Dividend Equivalents*. Dividend equivalents represent the right to receive the equivalent value of dividends paid on shares of our common stock and may be granted alone or in tandem with awards other than stock options or SARs. Dividend equivalents are credited as of the dividend record dates during the period between the date an award is granted and the date such award vests, is exercised, is distributed or expires, as determined by the plan administrator.

Certain Transactions. The plan administrator has broad discretion to take action under the 2020 Plan, as well as make adjustments to the terms and conditions of existing and future awards, to prevent the dilution or enlargement of intended benefits and facilitate necessary or desirable changes in the event of certain transactions and events affecting our common stock, such as stock dividends, stock splits, mergers, acquisitions, consolidations and other corporate transactions. In addition, in the event of certain non-reciprocal transactions with our stockholders known as “equity restructurings,” the plan administrator will make equitable adjustments to the 2020 Plan and outstanding awards. In the

event of a change in control of our Company (as defined in the 2020 Plan), to the extent that the surviving entity declines to continue, convert, assume or replace outstanding awards, then all such awards will become fully vested and exercisable in connection with the transaction. Awards under the 2020 Plan are generally non-transferrable, except by will or the laws of descent and distribution, or, subject to the plan administrator's consent, pursuant to a domestic relations order, and are generally exercisable only by the participant.

Foreign Participants, Claw-Back Provisions, Transferability and Participant Payments. The plan administrator may modify award terms, establish subplans and/or adjust other terms and conditions of awards, subject to the share limits described above, in order to facilitate grants of awards subject to the laws and/or stock exchange rules of countries outside of the United States. All awards will be subject to the provisions of any claw-back policy implemented by our Company to the extent set forth in such claw-back policy and/or in the applicable award agreement. With regard to tax withholding, exercise price and purchase price obligations arising in connection with awards under the 2020 Plan, the plan administrator may, in its discretion, accept cash or check, shares of our common stock that meet specified conditions, a "market sell order" or such other consideration as it deems suitable.

Plan Amendment and Termination. Our board of directors may amend or terminate the 2020 Plan at any time; however, no amendment, other than an amendment that increases the number of shares available under the 2020 Plan, may materially and adversely affect an award outstanding under the 2020 Plan without the consent of the affected participant, and stockholder approval will be obtained for any amendment to the extent necessary to comply with applicable laws or to increase the director limit. The plan administrator will have the authority, without the approval of our stockholders, to "reprice" any stock option or SAR, or cancel any stock option or SAR in exchange for cash or another award when the option or SAR price per share exceeds the fair market value of the underlying shares. The 2020 Plan will remain in effect until the tenth anniversary of the date the board of directors adopted the 2020 Plan, unless earlier terminated by our board of directors.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following are summaries of certain provisions of transactions within the past three years to which we have been a party, in which the amount involved exceeds or will exceed $120,000 and in which any of our directors, executive officers or holders of more than 5% of our capital stock, or immediate family member thereof, had or will have a direct or indirect material interest, and are qualified in their entirety by reference to all of the provisions of such agreements.

We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that we would pay or receive, as applicable, in arm's-length transactions.

Related Party Agreements in Effect Prior to this Offering

Series A Convertible Preferred Stock

From September 22, 2017 through January 8, 2018, we issued and sold an aggregate of 285,000,000 shares of our Series A convertible preferred stock, or Series A stock, at a purchase price of $1.00 per share for aggregate consideration of approximately $285.0 million.

The participants in this convertible preferred stock financing included certain holders of more than 5% of our capital stock and their affiliates. The following table sets forth the aggregate number of shares of Series A stock issued to these related parties in this convertible preferred stock financing:

Stockholder	Shares of Series A Stock	Total Purchase Price
Valor IV Pharma Holdings, LLC	75,000,000	$ 75,000,000
Entities affiliated with FMR LLC (Fidelity)(1)	40,000,000	$ 40,000,000
HBM Healthcare Investments (Cayman) Ltd.	30,000,000	$ 30,000,000
Entities affiliated with Vivo Capital LLC(2)	30,000,000	$ 30,000,000
Marshman Fund Trust II	25,000,000	$ 25,000,000
Novo Holdings A/S	25,000,000	$ 25,000,000
VenBio Global Strategic Fund, II, L.P.	25,000,000	$ 25,000,000
Quantum Strategic Partners Ltd.	11,400,000	$ 11,400,000

(1) Consists of 10,934,380 shares of Series A convertible preferred stock purchased by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund, 6,514,984 shares of Series A convertible preferred stock purchased by Fidelity Growth Company Commingled Pool: Fidelity Management & Trust Co., 2,550,636 shares of Series A convertible preferred stock purchased by Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund, 3,606,378 shares of Series A convertible preferred stock purchased by Fidelity Central Investment Portfolios LLC: Fidelity Health Care Central Fund, 1,195,827 shares of Series A convertible preferred stock purchased by Variable Insurance Products Fund IV: Health Care Portfolio, 10,935,215 shares of Series A convertible preferred stock purchased by Fidelity Select Portfolios: Health Care Portfolio, and 4,262,580 shares of Series A convertible preferred stock purchased by Fidelity Advisor Series VII: Fidelity Advisor Health Care Fund.

(2) Consists of 26,360,000 shares of Series A convertible preferred stock purchased by Vivo Capital Fund VIII, L.P. and 3,640,000 shares of Series A convertible preferred stock purchased by Vivo Capital Surplus Fund VIII, L.P.

Series B Convertible Preferred Stock

On January 8, 2018, we issued and sold an aggregate of 8,000,000 shares of our Series B convertible preferred stock, or Series B stock, at a purchase price of $1.25 per share for aggregate consideration of approximately $10.0 million.

The participants in this convertible preferred stock financing included certain holders of more than 5% of our capital stock and their affiliates. The following table sets forth the aggregate number of shares of Series B stock issued to these related parties in this convertible preferred stock financing:

Stockholder	Shares of Series B Stock	Total Purchase Price
Quantum Strategic Partners Ltd.	6,080,000	$ 7,600,000

Series C Convertible Preferred Stock

On August 9, 2019, we issued and sold an aggregate of 25,510,205 shares of our Series C convertible preferred stock, or Series C stock, at a purchase price of $1.96 per share for aggregate consideration of approximately $50.0 million.

The participants in this convertible preferred stock financing included certain holders of more than 5% of our capital stock and their affiliates. The following table sets forth the aggregate number of shares of Series C stock issued to these related parties in this convertible preferred stock financing:

Stockholder	Shares of Series C Stock	Total Purchase Price
Entities affiliated with FMR LLC (Fidelity)(1)	11,948,907	$ 23,419,858
HBM Healthcare Investments (Cayman) Ltd.	3,241,219	$ 6,352,789
Novo Holdings A/S	1,860,107	$ 3,645,810
Valor IV Pharma Holdings, LLC	1,786,985	$ 3,502,491
Entities affiliated with Vivo Capital LLC(2)	1,714,286	$ 3,360,001
Entities affiliated with Quantum Strategic Partners Ltd.(3)	1,712,544	$ 3,356,586

(1) Consists of 664,710 shares of Series C convertible preferred stock purchased by Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund, 2,404,058 shares of Series C convertible preferred stock purchased by Fidelity Growth Company Commingled Pool, 2,033,272 shares of Series C convertible preferred stock purchased by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund, 2,067,257 shares of Series C convertible preferred stock purchased by Fidelity Advisor Series VII: Fidelity Advisor Health Care Fund, 1,845,926 shares of Series C convertible preferred stock purchased by Fidelity Select Portfolios: Health Care Portfolio, 427,082 shares of Series C convertible preferred stock purchased by Variable Insurance Products Fund IV: Health Care Portfolio, 1,486,194 shares of Series C convertible preferred stock purchased by Fidelity Central Investment Portfolios LLC: Fidelity Health Care Central Fund, and 1,020,408 shares of Series C convertible preferred stock purchased by Fidelity Select Portfolios: Pharmaceutical Portfolio.

(2) Consists of 1,506,286 shares of Series C convertible preferred stock purchased by Vivo Capital Fund VIII, L.P. and 208,000 shares of Series C convertible preferred stock purchased by Vivo Capital Surplus Fund VIII, L.P.

(3) Consists of 1,709,116 shares of Series C convertible preferred stock purchased by QSIP LP and 3,428 shares of Series C convertible preferred stock purchased by SCI Partners LP.

Management and Other Agreements

We are party to a management services agreement, or the Management Services Agreement, with Paragon Biosciences, LLC, or Paragon, entered into on September 22, 2017, or the Effective Date, pursuant to which Paragon provides to us certain professional services. In addition, the Chairman of our Board of Directors, Jeffrey S. Aronin, is the Chairman and Chief Executive Officer of Paragon. Marshman Fund Trust I holds 99% of the LLC interests of Paragon. Mr. Aronin serves as the sole trustee of Marshman Fund Trust I and has sole voting and dispositive power with respect to such LLC interests. In exchange for services provided to us under the Management Services Agreement, we pay to Paragon a management fee of $0.3 million per each calendar month. This fee is reduced to

$0.2 million per each calendar month following the third anniversary of the Effective Date. For each of the years ended December 31, 2018 and 2019, we incurred approximately $4.0 million in management fee expense and other expenses to Paragon, which are included in general and administrative expense in the consolidated financial statements of operations. We have the right to terminate the Management Services Agreement upon the consummation of this offering. However, in the event such termination occurs prior to the fourth anniversary of the Effective Date, the terms of the Management Services Agreement require us to pay to Paragon 100% of the remaining amounts to be paid to Paragon under the Management Services Agreement between the date of such termination and the fourth anniversary of the Effective Date. We currently plan to terminate the Management Services Agreement upon the consummation of this offering.

We are also party to a right of use agreement with Paragon whereby we have access to and the right to use certain office space leased by Paragon in Chicago, Illinois. For the year ended December 31, 2019, we paid a fee of $ million pursuant to this agreement.

On March 30, 2018, we entered into an agreement regarding an office lease at 1033 Skokie Boulevard whereby we paid to an affiliate of Paragon $0.4 million to offset the costs of an early termination of the lease by such affiliate and we entered into a new office space lease with the landlord. The lease expired January 31, 2020.

Second Amended and Restated Investors' Rights Agreement

In connection with the issuance of our Series C preferred stock on August 9, 2019, we entered into a Second Amended and Restated Investors' Rights Agreement, or the IRA, pursuant to which certain holders of our preferred stock, or the Preferred Investors, many of which are beneficial holders of more than 5% of our capital stock or are entities with which certain of our directors are affiliated, are (and following the closing of this offering will be) entitled to rights with respect to the registration of their shares under the Securities Act, as described in additional detail below. Consistent with the Preferred Investors' obligations under the IRA, in connection with this offering, each Preferred Investor that has registration rights agreed not to sell or otherwise dispose of any securities without the prior written consent of the underwriters for a period of 180 days after the date of this prospectus, subject to certain terms and conditions. For more information regarding such restrictions, see the section captioned "Underwriting."

Demand Registration Rights

Pursuant to the IRA, the Preferred Investors are entitled to certain demand registration rights, including to demand registration of their registrable securities 180 days following the completion of this offering. The Preferred Investors holding more than 50% of the registrable securities have the right to require us, on not more than five occasions, to file a registration statement under the Securities Act in order to register the resale of their shares of common stock. We may, in certain circumstances, defer such registrations, and the underwriters have the right, subject to certain limitations, to limit the number of shares included in such registrations.

Piggyback Registration Rights

If we propose to register the offer and sale of any of our securities under the Securities Act, in connection with the public offering of such securities, the Preferred Investors will be entitled to certain "piggyback" registration rights, allowing them to request to include their registrable securities in such registration, subject to certain limitations. If our proposed registration involves an underwriting, the managing underwriter of such offering will have the right to limit the number of shares to be underwritten for reasons related to the marketing of the shares.

S-3 Registration Rights

After we are qualified for registration on Form S-3, the Preferred Investors, as holders of registrable securities, may make a written request that we register the offer and sale of their shares on Form S-3, *provided* that no such registration is required to be made (i) during the period that is 30 days before the Company's good faith estimate of the date of filing of, and ending on a date that is 90 days after the effective date of, a Company-initiated registration or (ii) at such time as we have effected two such registrations in the last 12 months. We may, in certain circumstances, defer such registrations, and the underwriters have the right, subject to certain limitations, to limit the number of shares included in such registrations.

Expenses

Subject to specified conditions and limitations, we are required to pay all expenses, other than underwriting discounts and commissions, stock transfer taxes, and fees and disbursements of counsel for any holder (except selling holder counsel) incurred in connection with any exercise of these registration rights.

Indemnification

The IRA contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling holders of registrable securities in the event of any damages from an untrue (or allegedly untrue) statement of a material fact or an omission (or alleged omission) of a material fact in the applicable registration statement attributable to us or our violation of the Securities Act, the Exchange Act, any state securities law, or any rule or regulation promulgated under the Securities Act, the Exchange Act, or any state securities law, and the selling stockholders are obligated to indemnify us for any damages from an untrue (or allegedly untrue) statement of a material fact or an omission (or alleged omission) of a material fact in the applicable registration statement attributable to us or our violation of the Securities Act, the Exchange Act, any state securities law, or any rule or regulation promulgated under the Securities Act, the Exchange Act, or any state securities law, only to the extent that such damages arise out of or are based upon actions or omissions made in reliance upon the written information furnished by or on behalf of such selling stockholder(s), subject to certain limitations.

Termination

The registration rights terminate upon the earliest of: (i) such date after the completion of this offering on which all shares of registrable securities may be sold during any three (3) month period pursuant to Rule 144 of the Securities Act, (ii) the fifth anniversary of the completion of this offering, (iii) the occurrence of a deemed liquidation event or (iv) the date that no registrable securities remain outstanding that have not previously been sold to the public pursuant to a registration or in reliance on Rule 144 of the Securities Act.

Second Amended and Restated Voting Agreement

In connection with the issuance of our Series C preferred stock on August 9, 2019, we entered into a Second Amended and Restated Voting Agreement, or the Voting Agreement, which, among other things, provides the terms for the voting of shares with respect to the constituency of our board of directors. Pursuant to the terms of the Voting Agreement, the following directors were elected to serve as members of our board of directors, and, as of the date of this prospectus, continue to so serve: Jeffrey S. Aronin, John C. Jacobs, Antonio Gracias, Juan A. Sabater, Jack Bech Nielsen, Martin Edwards, Aaron Royston and Dr. Andreas Wicki. Mr. Aronin was selected to serve on our board of

directors as designated by Marshman Fund Trust II, Mr. Jacobs was selected to serve on our board of directors as our CEO, Messrs. Gracias and Sabater were selected to serve on our board of directors as designated by Valor IV Pharma Holdings, LLC, or the Valor Directors, Mr. Nielsen was selected to serve on our board of directors as designated by Vivo Capital Fund VIII, L.P. and Vivo Capital Surplus Fund VIII, L.P., or the Vivo Director, Mr. Edwards was selected to serve on our board of directors as designated by Novo Holdings A/S, or the Novo Director, Mr. Royston was selected to serve on our board of directors as designated by venBio Global Strategic Fund II, L.P., or the venBio Director, Dr. Wicki was selected to serve on our board of directors as designated by HBM Healthcare Investments (Cayman) Ltd., together with the Valor Directors, the Vivo Director, the Novo Director and the venBio Director and the Series A Directors, possess relevant industry experience and are acceptable to a majority of the Preferred Investors as parties to the Voting Agreement.

The Voting Agreement, including its provisions concerning the rights of certain of the Preferred Investors to designate directors, will terminate automatically upon the consummation of this offering.

Second Amended and Restated Right of First Refusal and Co-Sale Agreement

In connection with the issuance of our Series C preferred stock on August 9, 2019, we entered into a Second Amended and Restated Right of First Refusal and Co-Sale Agreement, or the ROFR and Co-Sale Agreement, with certain of our Preferred Stockholders, many of which are beneficial holders of more than 5% of our capital stock or are entities with which certain of our directors are affiliated. The ROFR and Co-Sale Agreement, among other things: (a) grants our investors certain rights of first refusal and co-sale with respect to proposed transfers of our securities by certain Preferred Stockholders; and (b) grants us certain rights of first refusal with respect to proposed transfers of our securities by certain Preferred Stockholders.

The ROFR and Co-Sale Agreement will automatically terminate immediately prior to the completion of this offering.

Employment Agreements

We intend to enter into an employment agreement with each of our named executive officers in connection with this offering. See “Executive Compensation—Executive Compensation Arrangements.”

Director and Officer Indemnification and Insurance

Prior to the consummation of this offering, we intend to enter into separate indemnification agreements with each of our directors and executive officers. We have also purchased directors’ and officers’ liability insurance. See “Description of Capital Stock—Limitations on Liability and Indemnification of Officers and Directors.”

Our Policy Regarding Related Party Transactions

Our board of directors recognizes the fact that transactions with related persons present a heightened risk of conflicts of interests, improper valuation or the perception thereof. Prior to the consummation of this offering, our board of directors will adopt a written policy on transactions with related persons that is in conformity with the requirements for issuers having publicly held common stock that is listed on the . Under the new policy:

- any related person transaction, and any material amendment or modification to a related person transaction, must be reviewed and approved or ratified by a committee of the board of directors composed solely of independent directors who are disinterested or by the disinterested members of the board of directors; and

- any employment relationship or transaction involving an executive officer and any related compensation must be approved by the compensation committee of the board of directors or recommended by the compensation committee to the board of directors for its approval.

In connection with the review and approval or ratification of a related person transaction:

- management must disclose to the committee or disinterested directors, as applicable, the name of the related person and the basis on which the person is a related person, the material terms of the related person transaction, including the approximate dollar value of the amount involved in the transaction, and all the material facts as to the related person's direct or indirect interest in, or relationship to, the related person transaction;
- management must advise the committee or disinterested directors, as applicable, as to whether the related person transaction complies with the terms of our agreements governing our material outstanding indebtedness that limit or restrict our ability to enter into a related person transaction;
- management must advise the committee or disinterested directors, as applicable, as to whether the related person transaction will be required to be disclosed in our applicable filings under the Securities Act or the Exchange Act, and related rules, and, to the extent required to be disclosed, management must ensure that the related person transaction is disclosed in accordance with the Securities Act and the Exchange Act and related rules; and
- management must advise the committee or disinterested directors, as applicable, as to whether the related person transaction constitutes a "personal loan" for purposes of Section 402 of the Sarbanes-Oxley Act.

In addition, the related person transaction policy provides that the committee or disinterested directors, as applicable, in connection with any approval or ratification of a related person transaction involving a non-employee director should consider whether such transaction would compromise the director's status as an "independent" or "non-employee" director, as applicable, under the rules and regulations of the SEC, the and the Code.

PRINCIPAL STOCKHOLDERS

The following table sets forth information with respect to the beneficial ownership of our common stock as of , 2020 (i) reflecting the automatic conversion of all outstanding shares of our convertible preferred stock into shares of our common stock, (ii) the payment of an accrued dividend to holders of our convertible preferred stock in the aggregate amount of shares of our common stock, in each case immediately prior to the closing of this offering, and (iii) as adjusted to give effect to this offering, for:

- each person known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- each of our named executive officers; and
- all of our executive officers and directors as a group.

The number of shares beneficially owned by each stockholder as described in this prospectus is determined under rules issued by the SEC. Under these rules, beneficial ownership includes any shares as to which the individual or entity has sole or shared voting power or investment power. In computing the number of shares beneficially owned by an individual or entity and the percentage ownership of that person, shares of common stock subject to options, or other rights, held by such person that are currently exercisable or will become exercisable within 60 days of the date of this prospectus, are considered outstanding, although these shares are not considered outstanding for purposes of computing the percentage ownership of any other person. The percentage ownership of each individual or entity after giving effect to the automatic conversion of all outstanding shares of our convertible preferred stock into shares of our common stock and the payment of an accrued dividend to holders of our convertible preferred stock in the aggregate amount of shares of our common stock, in each case immediately prior to the closing of this offering, and before this offering is computed on the basis of total shares of our common stock outstanding, in each case, immediately following the conversion of all outstanding shares of our convertible preferred stock into shares of our common stock, in each case immediately prior to the closing of this offering (other than this offering). Unless otherwise indicated, the address of all listed stockholders is 630 W. Germantown Pike, Suite 215, Plymouth Meeting, Pennsylvania 19462.

Each of the stockholders listed has sole voting and investment power with respect to the shares beneficially owned by the stockholder unless noted otherwise, subject to community property laws where applicable.

	Shares beneficially owned prior to the offering				Shares beneficially owned after the offering			
Name of beneficial owner	Common stock	Options exercisable within 60 days	Aggregate number of shares beneficially owned	%	Assuming no exercise of option to purchase additional shares	%	Assuming exercise of option to purchase additional shares	%
5% or more stockholders:								
Valor IV Pharma Holdings, LLC(1)								
Entities affiliated with FMR LLC (Fidelity)(2)								
HBM Healthcare Investments (Cayman) Ltd.(3)								
Entities affiliated with Vivo Capital LLC(4)								
Marshman Fund Trust II(5)								
Novo Holdings A/S(6)								
Entities affiliated with Quantum Strategic Partners Ltd.(7)								
venBio Global Strategic Fund II LP(8)								
Named executive officers and directors:								
John C. Jacobs								
Jeffrey Dayno								
Andrew Serafin								
John Vittoria								
Jeffrey S. Aronin								
Martin Edwards(6)								
Antonio Gracias(1)								
Jack Bech Nielsen(4)								
Aaron Royston(8)								
Juan A. Sabater(1)								
Andreas Wicki(3)								
All current directors and executive officers as a group (11 persons)								

* Represents beneficial ownership of less than 1% of outstanding shares of our common stock.

(1)
(2)
(3)
(4)
(5)
(6)
(7)
(8)

DESCRIPTION OF CAPITAL STOCK

General

At or prior to the consummation of this offering, we will file an amended and restated certificate of incorporation and we will adopt our amended and restated bylaws. Our amended and restated certificate of incorporation will authorize capital stock consisting of:

- shares of common stock, par value $0.00001 per share; and
- shares of preferred stock, par value $0.00001 per share.

We are selling shares of common stock in this offering (shares if the underwriters exercise their option to purchase additional shares of our common stock in full). All shares of our common stock outstanding upon consummation of this offering will be fully paid and non-assessable.

The following summary describes the material provisions of our capital stock. We urge you to read our amended and restated certificate of incorporation and our amended and restated bylaws, which are included as exhibits to the registration statement of which this prospectus forms a part.

Certain provisions of our amended and restated certificate of incorporation and our amended and restated bylaws summarized below may be deemed to have an anti-takeover effect and may delay or prevent a tender offer or takeover attempt that a stockholder might consider in its best interest, including those attempts that might result in a premium over the market price for the shares of common stock.

Common Stock

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Upon our dissolution or liquidation, after payment in full of all amounts required to be paid to creditors and to the holders of preferred stock having liquidation preferences, if any, the holders of shares of our common stock will be entitled to receive pro rata our remaining assets available for distribution for distribution to stockholders after the payment of all of our debts and other liabilities, subject to the prior rights of any preferred stock then outstanding.

Preferred Stock

Upon the closing of this offering, (i) all outstanding shares of our convertible preferred stock will be automatically converted into shares of our common stock, (ii) all holders of our convertible preferred stock will be paid an accrued dividend in common stock in the aggregate amount of shares of our common stock and (iii) all outstanding shares of our redeemable preferred stock will automatically be cancelled.

Upon the consummation of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately after consummation of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

Registration Rights

Our Investors' Rights Agreement provides that certain holders of our preferred stock have certain registration rights as set forth below. The registration of shares of our common stock by the exercise of registration rights described below would enable the holders to sell these shares without restriction under the Securities Act when the applicable registration statement is declared effective. We will pay the registration expenses, other than underwriting discounts and commissions, stock transfer taxes, and fees and disbursements of counsel for any holder, except for the fees and disbursements of the selling holder counsel, of the shares registered by the demand, piggyback and Form S-3 registrations described below.

Generally, in an underwritten offering, the managing underwriter, if any, has the right, subject to specified conditions, to limit the number of shares such holders may include. The demand, piggyback and Form S-3 registration rights described below will expire on the five-year anniversary of the closing of this offering, or with respect to any particular stockholder, such time after the closing of this offering that such stockholder can sell all of its shares entitled to registration rights under Rule 144 of the Securities Act during any 90-day period.

Demand Registration Rights

Any holder or holders of more than 50% of our common stock then outstanding converted from our convertible preferred stock will be entitled to certain demand registration rights. At any time beginning 180 days after the closing of this offering, the holders of more than 50% of these shares may request that we register all or a portion of their shares on a Form S-1 registration statement; provided, that we are obligated to effect only five such registrations. Upon receipt of a request to file a Form S-1 registration statement, we must notify all other holders of our common stock converted from our convertible preferred stock and, within 60 days, file a Form S-1 registration statement under the Securities Act. We are not obligated to take any action to effect any registration during the period that is 60 days before our good faith estimate of the date of filing of, and ending on a date that is 180 days after the effective date of, a registration statement initiated by us. Additionally, if our board of directors determines that it would be materially detrimental to us and our stockholders to effect such a registration, we have the right to defer such registration, not more than twice in any 12-month period, for a period of not more than 120 days.

Piggyback Registration Rights

In connection with this offering, pursuant to the Investors' Rights Agreement, each holder of each series of our convertible preferred stock was entitled to, and the necessary percentage of holders waived, their rights to notice of this offering and to include their shares of registrable securities in this offering. After the completion of this offering, in the event that we propose to register any of our securities under the Securities Act, either for our own account or for the account of other security

holders, the holders of common stock converted from our convertible preferred stock will be entitled to certain piggyback registration rights allowing the holder to include their shares in such registration, subject to certain marketing and other limitations. If a holder decides not to include all of its shares in any registration statement filed by us, it shall nevertheless continue to have the right to include its shares in any subsequent registration statement or registration statements as we may file with respect to offerings of our securities. We have the right to terminate or withdraw any registration initiated whether or not any holder has elected to include securities in such registration upon prompt notice to such holder or holders.

Form S-3 Registration

After the completion of this offering, any holder or holders of the common stock then outstanding converted from our convertible preferred stock will be entitled to certain Form S-3 registration rights. One or more holders of these shares may make a written request that we register the offer and sale of their shares on a registration statement on Form S-3 if we are eligible to file a registration statement on Form S-3. Upon receipt of a request to file a Form S-3 registration statement, we must notify all other holders of our common stock converted from our convertible preferred stock and, within 45 days, file a Form S-3 registration statement under the Securities Act. We are not obligated to take any action to effect any registration during the period that is 30 days before our good faith estimate of the date of filing of, and ending on a date that is 90 days after the effective date of, a registration statement initiated by us. Additionally, if our board of directors determines that it would be seriously detrimental to us and our stockholders to effect such a registration, we have the right to defer such registration, not more than twice in any 12-month period, for a period of up to 120 days.

Forum Selection

Our amended and restated certificate of incorporation will provide that unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will, to the fullest extent permitted by applicable law, be the sole and exclusive forum for: (i) any derivative action or proceeding brought on our behalf; (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, other employees or stockholders to us or our stockholders; (iii) any action asserting a claim arising pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws, or as to which the DGCL confers exclusive jurisdiction on the Court of Chancery; or (iv) any action asserting a claim governed by the internal affairs doctrine. This exclusive forum provision will not apply to claims arising under the Securities Act, the Exchange Act or other federal securities laws for which there is exclusive federal or concurrent federal and state jurisdiction. Any person or entity purchasing or otherwise acquiring or holding any interest in shares of our capital stock will be deemed to have notice of and consented to this provision.

Dividends

Declaration and payment of any dividend will be subject to the discretion of our board of directors. The time and amount of dividends will be dependent upon our business prospects, results of operations, financial condition, cash requirements and availability, debt repayment obligations, capital expenditure needs, contractual restrictions, covenants in the agreements governing our current and future indebtedness, industry trends, the provisions of Delaware law affecting the payment of distributions to stockholders and any other factors our board of directors may consider relevant. We currently intend to retain all available funds and any future earnings to fund the development and growth of our business and to repay indebtedness, and therefore do not anticipate declaring or paying any cash dividends on our common stock in the foreseeable future. See “Dividend Policy” and “Risk Factors—Risks Related to this Offering and Ownership of our Common Stock—We have never paid dividends on our capital stock and we do not intend to pay dividends for the foreseeable future. Consequently, any gains from an investment in our common stock will likely depend on whether the price of our common stock increases.”

Anti-Takeover Provisions

Our amended and restated certificate of incorporation and amended and restated bylaws, as they will be in effect immediately prior to the consummation of this offering, will contain provisions that may delay, defer or discourage another party from acquiring control of us. We expect that these provisions, which are summarized below, will discourage coercive takeover practices or inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of us to first negotiate with our board of directors, which we believe may result in an improvement of the terms of any such acquisition in favor of our stockholders. However, they also give our board of directors the power to discourage acquisitions that some stockholders may favor. See "Risk Factors—Risks Related to This Offering and Ownership of Our Common Stock—Our charter documents and Delaware law could prevent a takeover that stockholders consider favorable and could also reduce the market price of our stock."

Authorized but Unissued Shares

The authorized but unissued shares of our common stock and our preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the listing standards of the . These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

Classified Board of Directors

Our amended and restated certificate of incorporation will provide that our board of directors will be divided into three classes, with the classes as nearly equal in number as possible and each class serving three-year staggered terms. In all other cases and at any other time, directors may only be removed from our board of directors for cause by the affirmative vote of a majority of the shares entitled to vote. See "Management—Composition of our Board of Directors." These provisions may have the effect of deferring, delaying or discouraging hostile takeovers, or changes in control of us or our management.

Stockholder Action; Special Meeting of Stockholders

Our amended and restated certificate of incorporation will provide that our stockholders will not be able to take action by written consent for any matter and may only take action at annual or special meetings. As a result, a holder controlling a majority of our capital stock would not be able to amend our amended and restated bylaws or remove directors without holding a meeting of our stockholders called in accordance with our amended and restated bylaws, unless previously approved by our board of directors. Our amended and restated certificate of incorporation will further provide that special meetings of our stockholders may be called only by the chairman of our board of directors, our chief executive officer, our president or another officer selected by a majority of our board of directors, thus limiting the ability of a stockholder to call a special meeting. These provisions might delay the ability of our stockholders to force consideration of a proposal or for stockholders controlling a majority of our capital stock to take any action, including the removal of directors.

Advance Notice Requirements for Stockholder Proposals and Director Nominations

In addition, our amended and restated bylaws will establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. In order for any matter to be "properly brought" before a meeting, a stockholder will have to comply with advance notice and duration of ownership requirements and provide us with certain information. Stockholders at an annual meeting

may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors or by a qualified stockholder of record on the record date for the meeting, who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying stockholder actions that are favored by the holders of a majority of our outstanding voting securities until the next stockholder meeting.

Amendment of Certificate of Incorporation or Bylaws

The DGCL provides generally that the affirmative vote of the holders of a majority in voting power of the shares entitled to vote is required to amend a corporation's certificate of incorporation, unless a corporation's certificate of incorporation requires a greater percentage. Upon consummation of this offering, our bylaws may be amended or repealed by a majority vote of our board of directors or by the affirmative vote of the holders a majority of the votes which all our stockholders would be eligible to cast in an election of directors.

Section 203 of the DGCL

We are subject to Section 203 of the DGCL, which prohibits persons deemed "interested stockholders" from engaging in a "business combination" with a publicly held Delaware corporation for three years following the date these persons become interested stockholders unless the business combination is, or the transaction in which the person became an interested stockholder was, approved in a prescribed manner or another prescribed exception applies. Generally, an "interested stockholder" is a person who, together with affiliates and associates, owns, or within three years prior to the determination of interested stockholder status did own, 15% or more of a corporation's voting stock. Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to the interested stockholder. The existence of this provision may have an anti-takeover effect with respect to transactions not approved in advance by the board of directors, such as discouraging takeover attempts that might result in a premium over the market price of our common stock.

Limitations on Liability and Indemnification of Officers and Directors

Our amended and restated bylaws provide indemnification for our directors and officers to the fullest extent permitted by the DGCL, along with the right to have expenses incurred in defending proceedings paid in advance of their final disposition. Prior to the consummation of this offering, we intend to enter into indemnification agreements with each of our directors and executive officers that may, in some cases, be broader than the specific indemnification and advancement provisions contained under our amended and restated bylaws and provided under Delaware law. In addition, as permitted by Delaware law, our amended and restated certificate of incorporation includes provisions that eliminate the personal liability of our directors for monetary damages resulting from breaches of certain fiduciary duties as a director. The effect of this provision is to restrict our rights and the rights of our stockholders to recover monetary damages against a director for breach of fiduciary duties as a director.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers or persons controlling our company pursuant to the foregoing provisions, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act, and is, therefore, unenforceable.

Corporate Opportunity Doctrine

Delaware law permits corporations to adopt provisions renouncing any interest or expectancy in certain opportunities that are presented to the corporation or its officers, directors or stockholders. Our

amended and restated certificate of incorporation will, to the maximum extent permitted from time to time by Delaware law, renounce any interest or expectancy that we have in, or right to be offered an opportunity to participate in, specified business opportunities that are from time to time presented to our officers, directors or certain of our stockholders or their respective affiliates, other than those opportunities our officers, directors, stockholders or affiliates are presented with while acting in their capacity as an employee, officer or director of us or our affiliates. Our amended and restated certificate of incorporation will provide that, to the fullest extent permitted by law, any director or stockholder who is not employed by us or our affiliates will not have any duty to refrain from (i) engaging in a corporate opportunity in the same or similar lines of business in which we or our affiliates now engage or propose to engage; or (ii) otherwise competing with us or our affiliates. In addition, to the fullest extent permitted by law, if any director or stockholder, other than a director or stockholder who is employed by us or our affiliates acting in their capacity as an employee or director of us or our affiliates, acquires knowledge of a potential transaction or other business opportunity which may be a corporate opportunity for itself or himself or its or his affiliates or for us or our affiliates, such person will have no duty to communicate or offer such transaction or business opportunity to us or any of our affiliates and they may take any such opportunity for themselves or offer it to another person or entity. To the fullest extent permitted by Delaware law, no potential transaction or business opportunity may be deemed to be a corporate opportunity of ours or our subsidiary. Our amended and restated certificate of incorporation will not renounce our interest in any business opportunity that is expressly offered to an employee director, employee officer or employee in his or her capacity as a director, officer or employee of Harmony Biosciences Holdings, Inc.

Dissenters' Rights of Appraisal and Payment

Under the DGCL, with certain exceptions, our stockholders will have appraisal rights in connection with a merger or consolidation of Harmony Biosciences Holdings, Inc. Pursuant to the DGCL, stockholders who properly demand and perfect appraisal rights in connection with such mergers or consolidations will have the right to receive payment of the fair value of their shares as determined by the Delaware Court of Chancery, subject to certain limitations.

Stockholders' Derivative Actions

Under the DGCL, any of our stockholders may bring an action in our name to procure a judgment in our favor, also known as a derivative action, in certain circumstances. Among other things, either the stockholder bringing any such action must be a holder of our shares at the time of the transaction to which the action relates or such stockholder's stock must have thereafter devolved by operation of law, and such stockholder must continuously hold shares through the resolution of such action.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is .

Trading Symbol and Market

We intend to apply to list our common stock on the under the symbol " ."

SHARES ELIGIBLE FOR FUTURE SALE

Immediately prior to this offering, there was no public market for our common stock. Future sales of substantial amounts of common stock in the public market, or the perception that such sales may occur, could adversely affect the market price of our common stock. Although we have applied to have our common stock listed on the , we cannot assure you that there will be an active public market for our common stock.

Upon the closing of this offering, we will have outstanding an aggregate of shares of common stock, assuming the issuance of shares of common stock offered by us in this offering. Of these shares, all shares of common stock sold in this offering will be freely tradable without restriction or further registration under the Securities Act, except for any shares purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act, whose sales would be subject to the Rule 144 resale restrictions described below, other than the holding period requirement.

Lock-Up Agreements

We, our officers and directors and holders of substantially all of our common stock and securities convertible into or exchangeable for our common stock will agree that, without the prior written consent of Goldman Sachs & Co. LLC, Jefferies LLC and Piper Sandler & Co., as representatives of the underwriters, we and they will not, subject to certain exceptions, during the period ending 180 days after the date of this prospectus:

- offer, sell, contract to sell, pledge, grant any option to purchase, make any short sale or otherwise transfer or dispose of, directly or indirectly or publicly disclose the intention to make any offer, sale, pledge or disposition of any shares of our common stock, or any options or warrants to purchase any shares of our common stock, or any securities convertible into, or exchangeable for, or that represent the right to receive, shares of our common stock; or
- enter into any swap or other arrangement that transfers to another, all or a portion of the economic consequences of ownership of our common stock or any securities convertible into or exercisable or exchangeable for shares of our common stock,

whether any transaction described above is to be settled by delivery of our common stock or such other securities, in cash or otherwise.

The representatives of the underwriters have advised us that they have no present intent or arrangement to release any shares subject to a lock-up, and will consider the release of any lock-up on a case-by-case basis. Upon a request to release any shares subject to a lock-up, the representatives of the underwriters would consider the particular circumstances surrounding the request, including, but not limited to, the length of time before the lock-up expires, the number of shares requested to be released, reasons for the request, the possible impact on the market or our common stock and whether the holder of our shares requesting the release is an officer, director or other affiliate of ours.

Upon the expiration of the applicable lock-up periods, substantially all of the shares subject to such lock-up restrictions will become eligible for sale, subject to the limitations discussed above.

Rule 144

Affiliate Resales of Restricted Securities

In general, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, a person who is an affiliate of ours, or who was an affiliate at any time during the 90 days before a sale, who has beneficially owned shares of our common stock for at least 180 days

would be entitled to sell in “broker’s transactions” or certain “riskless principal transactions” or to market makers, a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding; and
- the average weekly trading volume in our common stock on the during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Affiliate resales under Rule 144 are also subject to the availability of current public information about us. In addition, if the number of shares being sold under Rule 144 by an affiliate during any three-month period exceeds 5,000 shares or has an aggregate sale price in excess of $50,000, the seller must file a notice on Form 144 with the SEC and the concurrently with either the placing of a sale order with the broker or the execution directly with a market maker.

Non-Affiliate Resales of Restricted Securities

Under Rule 144, a person who is not an affiliate of ours at the time of sale, and has not been an affiliate at any time during the 90 days preceding a sale, and who has beneficially owned shares of our common stock for at least six months but less than a year, is entitled to sell such shares subject only to the availability of current public information about us. If such person has held our shares for at least one year, such person can resell without regard to any Rule 144 restrictions, including the 90-day public company requirement and the current public information requirement.

Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

Rule 701

In general, under Rule 701, any of our employees, directors, officers, consultants or advisors who purchases shares from us in connection with a compensatory stock or option plan or other written agreement before the effective date of the registration statement of which this prospectus forms a part is entitled to sell such shares 90 days after such effective date in reliance on Rule 144. Our affiliates can resell shares in reliance on Rule 144 without having to comply with the holding period requirement, and non-affiliates of the issuer can resell shares in reliance on Rule 144 without having to comply with the current public information and holding period requirements.

The SEC has indicated that Rule 701 will apply to typical stock options granted by an issuer before it becomes subject to the reporting requirements of the Exchange Act, along with the shares acquired upon exercise of such options, including exercises after an issuer becomes subject to the reporting requirements of the Exchange Act.

Registration Rights

Pursuant to our Investor Rights Agreement, beginning six months after the completion of this offering, the holders of up to shares of our common stock, or certain transferees, will be entitled to certain rights with respect to the registration of the offer and sale of those shares under the Securities Act. See the section titled “Description of Capital Stock—Registration Rights” for a description of these registration rights. If the offer and sale of these shares of our common stock are registered, the shares will be freely tradable without restriction under the Securities Act, subject to the Rule 144 limitations applicable to affiliates, and a large number of shares may be sold into the public market.

Registration Statements on Form S-8

We intend to file one or more registration statements on Form S-8 under the Securities Act to register all shares of common stock subject to outstanding stock options, and common stock issuable,

under our equity incentive plans. We expect to file the registration statement covering shares offered pursuant to these stock plans shortly after the date of this prospectus, permitting the resale of such shares by non-affiliates in the public market without restriction under the Securities Act and the sale by affiliates in the public market subject to compliance with the resale provisions of Rule 144.

MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following discussion is a summary of the material U.S. federal income tax consequences to Non-U.S. Holders (as defined below) of the purchase, ownership and disposition of our common stock issued pursuant to this offering, but does not purport to be a complete analysis of all potential tax effects. The effects of other U.S. federal tax laws, such as estate and gift tax laws, and any applicable state, local or non-U.S. tax laws are not discussed. This discussion is based on the U.S. Internal Revenue Code of 1986, as amended, or the Code, Treasury Regulations promulgated thereunder, judicial decisions, and published rulings and administrative pronouncements of the U.S. Internal Revenue Service, or the IRS, in each case in effect as of the date of this prospectus. These authorities may change or be subject to differing interpretations. Any such change or differing interpretation may be applied retroactively in a manner that could adversely affect a Non-U.S. Holder of our common stock. We have not sought and will not seek any rulings from the IRS regarding the matters discussed below. There can be no assurance the IRS or a court will not take a contrary position to that discussed below regarding the tax consequences of the purchase, ownership and disposition of our common stock.

This discussion is limited to Non-U.S. Holders that hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion does not address all U.S. federal income tax consequences relevant to a Non-U.S. Holder's particular circumstances, including the impact of the alternative minimum tax or the Medicare contribution tax on net investment income. In addition, it does not address consequences relevant to Non-U.S. Holders subject to special rules, including, without limitation:

- U.S. expatriates and former citizens or long-term residents of the United States;
- persons holding our common stock as part of a hedge, straddle or other risk reduction strategy or as part of a conversion transaction or other integrated investment;
- banks, insurance companies, and other financial institutions;
- brokers, dealers or traders in securities;
- "controlled foreign corporations," "passive foreign investment companies," and corporations that accumulate earnings to avoid U.S. federal income tax;
- partnerships or other entities or arrangements treated as partnerships for U.S. federal income tax purposes (and investors therein);
- tax-exempt organizations or governmental organizations;
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation;
- tax-qualified retirement plans;
- "qualified foreign pension funds" as defined in Section 897(l)(2) of the Code and entities all of the interests of which are held by qualified foreign pension funds; and
- persons subject to special tax accounting rules as a result of any item of gross income with respect to the stock being taken into account in an applicable financial statement.

If an entity treated as a partnership for U.S. federal income tax purposes holds our common stock, the tax treatment of a partner in the partnership will depend on the status of the partner, the activities of the partnership and certain determinations made at the partner level. Accordingly, partnerships holding our common stock and the partners in such partnerships should consult their tax advisors regarding the U.S. federal income tax consequences to them.

THIS DISCUSSION IS FOR INFORMATIONAL PURPOSES ONLY AND IS NOT TAX ADVICE. INVESTORS SHOULD CONSULT THEIR TAX ADVISORS WITH RESPECT TO THE APPLICATION OF THE U.S. FEDERAL INCOME TAX LAWS TO THEIR PARTICULAR SITUATIONS AS WELL AS ANY TAX CONSEQUENCES OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK ARISING UNDER THE U.S. FEDERAL ESTATE OR GIFT TAX LAWS OR UNDER THE LAWS OF ANY STATE, LOCAL OR NON-U.S. TAXING JURISDICTION OR UNDER ANY APPLICABLE INCOME TAX TREATY.

Definition of a Non-U.S. Holder

For purposes of this discussion, a "Non-U.S. Holder" is any beneficial owner of our common stock that is neither a "U.S. person" nor an entity treated as a partnership for U.S. federal income tax purposes. A U.S. person is any person that, for U.S. federal income tax purposes, is or is treated as any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation created or organized under the laws of the United States, any state thereof, or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust that (1) is subject to the primary supervision of a U.S. court and the control of one or more "United States persons" (within the meaning of Section 7701(a)(30) of the Code), or (2) has a valid election in effect to be treated as a United States person for U.S. federal income tax purposes.

Distributions

As described in the section entitled "Dividend Policy," we do not anticipate declaring or paying dividends to holders of our common stock in the foreseeable future. However, if we make distributions of cash or property on our common stock, such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and first be applied against and reduce a Non-U.S. Holder's adjusted tax basis in its common stock, but not below zero. Any excess will be treated as capital gain and will be treated as described below under "—Sale or Other Taxable Disposition."

Subject to the discussion below on effectively connected income, dividends paid to a Non-U.S. Holder of our common stock will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends (or such lower rate specified by an applicable income tax treaty, provided the Non-U.S. Holder furnishes a valid IRS Form W-8BEN or W-8BEN-E (or other applicable documentation) certifying qualification for the lower treaty rate). A Non-U.S. Holder that does not timely furnish the required documentation, but that qualifies for a reduced treaty rate, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS. Non-U.S. Holders should consult their tax advisors regarding their entitlement to benefits under any applicable income tax treaty.

If dividends paid to a Non-U.S. Holder are effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the Non-U.S. Holder maintains a permanent establishment in the United States to which such dividends are attributable), the Non-U.S. Holder will be exempt from the U.S. federal withholding tax described above. To claim the exemption, the Non-U.S. Holder must furnish to the applicable

withholding agent a valid IRS Form W-8ECI, certifying that the dividends are effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States.

Any such effectively connected dividends will be subject to U.S. federal income tax on a net income basis at the regular graduated rates. A Non-U.S. Holder that is a corporation also may be subject to a branch profits tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on such effectively connected dividends, as adjusted for certain items. Non-U.S. Holders should consult their tax advisors regarding any applicable tax treaties that may provide for different rules.

Sale or Other Taxable Disposition

Subject to the discussion below on information reporting, backup withholding and payments made to foreign accounts, a Non-U.S. Holder will not be subject to U.S. federal income tax on any gain realized upon the sale or other taxable disposition of our common stock unless:

- the gain is effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the Non-U.S. Holder maintains a permanent establishment in the United States to which such gain is attributable);
- the Non-U.S. Holder is a non-resident alien individual present in the United States for 183 days or more during the taxable year of the disposition and certain other requirements are met; or
- our common stock constitutes a U.S. real property interest, or USRPI, by reason of our status as a U.S. real property holding corporation, or USRPHC, for U.S. federal income tax purposes.

Gain described in the first bullet point above generally will be subject to U.S. federal income tax on a net income basis at the regular graduated rates. A Non-U.S. Holder that is a corporation also may be subject to a branch profits tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on such effectively connected gain, as adjusted for certain items.

Gain described in the second bullet point above will be subject to U.S. federal income tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty), which may be offset by U.S. source capital losses of the Non-U.S. Holder (even though the individual is not considered a resident of the United States), provided the Non-U.S. Holder has timely filed U.S. federal income tax returns with respect to such losses.

With respect to the third bullet point above, we believe we currently are not, and do not anticipate becoming, a USRPHC. Because the determination of whether we are a USRPHC depends, however, on the fair market value of our USRPIs relative to the fair market value of our non-U.S. real property interests and our other business assets, there can be no assurance we currently are not a USRPHC or will not become one in the future. Even if we are or were to become a USRPHC, gain arising from the sale or other taxable disposition by a Non-U.S. Holder of our common stock will not be subject to U.S. federal income tax if our common stock is "regularly traded," as defined by applicable Treasury Regulations, on an established securities market, and such Non-U.S. Holder owned, actually and constructively, 5% or less of our common stock throughout the shorter of the five-year period ending on the date of the sale or other taxable disposition or the Non-U.S. Holder's holding period.

Non-U.S. Holders should consult their tax advisors regarding potentially applicable income tax treaties that may provide for different rules.

Information Reporting and Backup Withholding

Payments of dividends on our common stock will not be subject to backup withholding, provided the applicable withholding agent does not have actual knowledge or reason to know the holder is a

United States person and the holder either certifies its non-U.S. status, such as by furnishing a valid IRS Form W-8BEN, W-8BEN-E or W-8ECI, or otherwise establishes an exemption. However, information returns are required to be filed with the IRS in connection with any dividends on our common stock paid to the Non-U.S. Holder, regardless of whether any tax was actually withheld. In addition, proceeds of the sale or other taxable disposition of our common stock within the United States or conducted through certain U.S.-related brokers generally will not be subject to backup withholding or information reporting, if the applicable withholding agent receives the certification described above and does not have actual knowledge or reason to know that such holder is a United States person, or the holder otherwise establishes an exemption. Proceeds of a disposition of our common stock conducted through a non-U.S. office of a non-U.S. broker generally will not be subject to backup withholding or information reporting.

Copies of information returns that are filed with the IRS may also be made available under the provisions of an applicable treaty or agreement to the tax authorities of the country in which the Non-U.S. Holder resides or is established.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules may be allowed as a refund or a credit against a Non-U.S. Holder's U.S. federal income tax liability, provided the required information is timely furnished to the IRS.

Additional Withholding Tax on Payments Made to Foreign Accounts

Withholding taxes may be imposed under Sections 1471 to 1474 of the Code (such Sections commonly referred to as the Foreign Account Tax Compliance Act, or FATCA) on certain types of payments made to non-U.S. financial institutions and certain other non-U.S. entities. Specifically, a 30% withholding tax may be imposed on dividends on, or (subject to the proposed Treasury Regulations discussed below) gross proceeds from the sale or other disposition of, our common stock paid to a "foreign financial institution" or a "non-financial foreign entity" (each as defined in the Code), unless (1) the foreign financial institution undertakes certain diligence and reporting obligations, (2) the non-financial foreign entity either certifies it does not have any "substantial United States owners" (as defined in the Code) or furnishes identifying information regarding each substantial United States owner, or (3) the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from these rules. If the payee is a foreign financial institution and is subject to the diligence and reporting requirements in (1) above, it must enter into an agreement with the U.S. Department of the Treasury requiring, among other things, that it undertake to identify accounts held by certain "specified United States persons" or "United States owned foreign entities" (each as defined in the Code), annually report certain information about such accounts, and withhold 30% on certain payments to non-compliant foreign financial institutions and certain other account holders. Foreign financial institutions located in jurisdictions that have an intergovernmental agreement with the United States governing FATCA may be subject to different rules.

Under the applicable Treasury Regulations and administrative guidance, withholding under FATCA generally applies to payments of dividends on our common stock. While withholding under FATCA would have applied also to payments of gross proceeds from the sale or other disposition of stock on or after January 1, 2019, recently proposed Treasury Regulations eliminate FATCA withholding on payments of gross proceeds entirely. Taxpayers generally may rely on these proposed Treasury Regulations until final Treasury Regulations are issued.

Prospective investors should consult their tax advisors regarding the potential application of withholding under FATCA to their investment in our common stock.

UNDERWRITING

We and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. Goldman Sachs & Co. LLC, Jefferies LLC and Piper Sandler & Co. are the representatives of the underwriters.

Underwriters	Number of Shares
Goldman Sachs & Co. LLC	
Jefferies LLC	
Piper Sandler & Co.	
Total	

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters have an option to buy up to an additional shares from us to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. They may exercise that option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above.

The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters by us. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase up to additional shares from us.

	No Exercise	Full Exercise
Per Share	$	$
Total	$	$

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to $ per share from the initial public offering price. After the initial offering of the shares, the representatives may change the offering price and the other selling terms. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make internet distributions on the same basis as other allocations.

We and our executive officers, directors, and holders of substantially all of our common stock and securities convertible into or exchangeable for our common stock have agreed or will agree with the underwriters, subject to certain exceptions, not to dispose of or hedge any of our or their common

stock or securities convertible into or exchangeable for shares of common stock during the period from the date of this prospectus continuing through the date 180 days after the date of this prospectus, except with the prior written consent of Goldman Sachs & Co. LLC, Jefferies LLC and Piper Sandler & Co. See the section of this prospectus titled “Shares Eligible for Future Sale” for a discussion of certain transfer restrictions.

Prior to the offering, there has been no public market for the shares. The initial public offering price will be negotiated among us and the representatives. Among the factors to be considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, will be our historical performance, estimates of our business potential and earnings prospects, an assessment of our management and the consideration of the above factors in relation to market valuation of companies in related businesses.

We intend to apply to list our common stock on the under the symbol “ .”

In connection with the offering, the underwriters may purchase and sell shares of our common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A “covered short position” is a short position that is not greater than the amount of additional shares for which the underwriters’ option described above may be exercised. The underwriters may cover any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option described above. “Naked” short sales are any short sales that create a short position greater than the amount of additional shares for which the option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of our stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on the , in the over-the-counter market or otherwise.

We estimate that our share of the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately $ million. We have agreed to reimburse the underwriters for certain of their expenses in an amount up to $.

We have agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates have provided, and may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively trade securities, derivatives, loans, commodities, currencies, credit default swaps and other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities or instruments of the issuer (directly, as collateral securing other obligations or otherwise) or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas or publish or express independent research views in respect of such assets, securities or instruments and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

European Economic Area

In relation to each Member State of the European Economic Area (each, a “Member State”), no offer of shares of our Class A common stock may be made to the public in that Member State other than:

- to any legal entity which is a qualified investor as defined in the Prospectus Regulation;
- to fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Regulation), subject to obtaining the prior consent of the representatives; or
- in any other circumstances falling within Article 1(4) of the Prospectus Regulation, provided that no such offer of shares shall require us or any of our representatives to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation and each person who initially acquires any shares or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with each of the representatives and us that it is a “qualified investor” as defined in the Prospectus Regulation.

In the case of any shares being offered to a financial intermediary as that term is used in Article 5 of the Prospectus Regulation, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares acquired by it in the offer have not been acquired on a

nondiscretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares to the public other than their offer or resale in a Member State to qualified investors as so defined or in circumstances in which the prior consent of the representatives has been obtained to each such proposed offer or resale.

For the purposes of this provision, the expression an “offer of shares to the public” in relation to any shares in any Member State means the communication in any form and by means of sufficient information on the terms of the offer and the shares to be offered so as to enable an investor to decide to purchase shares, the expression “Prospectus Regulation” means Regulation (EU) 2017/1129 (as amended).

This European Economic Area selling restriction is in addition to any other selling restrictions set out below.

United Kingdom

In the United Kingdom, this prospectus is only addressed to and directed at qualified investors who are (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (the Order); or (ii) high net worth entities and other persons to whom it may lawfully be communicated, falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as “relevant persons”). Any investment or investment activity to which this prospectus relates is available only to relevant persons and will only be engaged in with relevant persons. Any person who is not a relevant person should not act or rely on this prospectus or any of its contents.

Canada

The securities may be sold in Canada only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions, and Ongoing Registrant Obligations. Any resale of the securities must be made in accordance with an exemption form, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser’s province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser’s province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Hong Kong

The securities may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies

(Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32 of the Laws of Hong Kong) ("Companies (Winding Up and Miscellaneous Provisions) Ordinance") or which do not constitute an invitation to the public within the meaning of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong), or Securities and Futures Ordinance, or (ii) to "professional investors" as defined in the Securities and Futures Ordinance and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance, and no advertisement, invitation or document relating to the securities may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" in Hong Kong as defined in the Securities and Futures Ordinance and any rules made thereunder.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor (as defined under Section 4A of the Securities and Futures Act, Chapter 289 of Singapore (the "SFA")) under Section 274 of the SFA, (ii) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to conditions set forth in the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor, the securities (as defined in Section 239(1) of the SFA) of that corporation shall not be transferable for 6 months after that corporation has acquired the shares under Section 275 of the SFA except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (2) where such transfer arises from an offer in that corporation's securities pursuant to Section 275(1A) of the SFA, (3) where no consideration is or will be given for the transfer, (4) where the transfer is by operation of law, (5) as specified in Section 276(7) of the SFA, or (6) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore, or Regulation 32.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a trust (where the trustee is not an accredited investor (as defined in Section 4A of the SFA)) whose sole purpose is to hold investments and each beneficiary of the trust is an accredited investor, the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferable for 6 months after that trust has acquired the shares under Section 275 of the SFA except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (2) where such transfer arises from an offer that is made on terms that such rights or interest are acquired at a consideration of not less than S$200,000 (or its equivalent in a foreign currency) for each transaction (whether such amount is to be paid for in cash or by exchange of securities or other assets), (3) where no consideration is or will be given for the transfer, (4) where the transfer is by operation of law, (5) as specified in Section 276(7) of the SFA, or (6) as specified in Regulation 32.

Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Act of Japan (Act No. 25 of 1948, as amended), or the FIEA. The securities may not be offered or sold, directly or indirectly, in Japan or to or for the benefit of any resident of Japan (including any person resident in Japan or any corporation or other entity organized under the laws of Japan) or to others for reoffering or resale, directly or indirectly, in Japan or to or for the benefit of any resident of Japan, except pursuant to an exemption from the registration requirements of the FIEA and otherwise in compliance with any relevant laws and regulations of Japan.

Australia

No placement document, prospectus, product disclosure statement or other disclosure document has been lodged with the Australian Securities and Investments Commission, or ASIC, in relation to the offering. This offering document does not constitute a prospectus, product disclosure statement or other disclosure document under the Corporations Act 2001 (the "Corporations Act"), and does not purport to include the information required for a prospectus, product disclosure statement or other disclosure document under the Corporations Act.

Any offer in Australia of the shares may only be made to persons (the "Exempt Investors") who are "sophisticated investors" (within the meaning of section 708(8) of the Corporations Act), "professional investors" (within the meaning of section 708(11) of the Corporations Act) or otherwise pursuant to one or more exemptions contained in section 708 of the Corporations Act so that it is lawful to offer the shares without disclosure to investors under Chapter 6D of the Corporations Act.

The shares applied for by Exempt Investors in Australia must not be offered for sale in Australia in the period of 12 months after the date of allotment under the offering, except in circumstances where disclosure to investors under Chapter 6D of the Corporations Act would not be required pursuant to an exemption under section 708 of the Corporations Act or otherwise or where the offer is pursuant to a disclosure document which complies with Chapter 6D of the Corporations Act. Any person acquiring shares must observe such Australian on-sale restrictions.

This offering document contains general information only and does not take account of the investment objectives, financial situation or particular needs of any particular person. It does not contain any securities recommendations or financial product advice. Before making an investment decision, investors need to consider whether the information in this offering document is appropriate to their needs, objectives and circumstances, and, if necessary, seek expert advice on those matters.

Dubai International Financial Centre

This offering document relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority, or DFSA. This offering document is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth in this prospectus and has no responsibility for the offering document. The securities to which this offering document relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the securities offered should conduct their own due diligence on the securities. If you do not understand the contents of this offering document you should consult an authorized financial advisor.

Switzerland

We have not and will not register with the Swiss Financial Market Supervisory Authority, or FINMA, as a foreign collective investment scheme pursuant to Article 119 of the Federal Act on Collective Investment Scheme of 23 June 2006, as amended, or CISA, and accordingly the securities being offered pursuant to this prospectus have not and will not be approved, and may not be licensable, with FINMA. Therefore, the securities have not been authorized for distribution by FINMA as a foreign collective investment scheme pursuant to Article 119 CISA and the securities offered hereby may not be offered to the public (as this term is defined in Article 3 CISA) in or from Switzerland. The securities may solely be offered to "qualified investors," as this term is defined in Article 10 CISA, and in the circumstances set out in Article 3 of the Ordinance on Collective Investment Scheme of 22 November 2006, as amended, or CISO, such that there is no public offer. Investors, however, do not benefit from protection under CISA or CISO or supervision by FINMA. This prospectus and any other materials relating to the securities are strictly personal and confidential to each offeree and do not constitute an offer to any other person. This prospectus may only be used by those qualified investors to whom it has been handed out in connection with the offer described in this prospectus and may neither directly or indirectly be distributed or made available to any person or entity other than its recipients. It may not be used in connection with any other offer and shall in particular not be copied and/or distributed to the public in Switzerland or from Switzerland. This prospectus does not constitute an issue prospectus as that term is understood pursuant to Article 652a and/or 1156 of the Swiss Federal Code of Obligations. We have not applied for a listing of the securities on the SIX Swiss Exchange or any other regulated securities market in Switzerland, and consequently, the information presented in this prospectus does not necessarily comply with the information standards set out in the listing rules of the SIX Swiss Exchange and corresponding prospectus schemes annexed to the listing rules of the SIX Swiss Exchange.

LEGAL MATTERS

The validity of the shares of common stock offered hereby will be passed upon for us by Latham & Watkins LLP, Chicago, Illinois. Goodwin Procter LLP, Boston, Massachusetts has acted as counsel for the underwriters in connection with certain legal matters related to this offering.

EXPERTS

The consolidated financial statements of Harmony Biosciences Holdings, Inc. and its subsidiary as of and for the year ended December 31, 2018 included in this prospectus and registration statement have been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report appearing herein and elsewhere in the registration statement, which report expresses an unqualified opinion on the financial statements and includes an explanatory paragraph referring to substantial doubt that exists regarding the ability of the Company to continue as a going concern. Such financial statements have been so included in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock offered hereby. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement or the exhibits and schedules filed with the registration statement. For further information about us and the common stock offered hereby, we refer you to the registration statement and the exhibits filed with the registration statement. Statements contained in this prospectus regarding the contents of any contract or any other document that is filed as an exhibit to the registration statement are not necessarily complete, and each such statement is qualified in all respects by reference to the full text of such contract or other document filed as an exhibit to the registration statement. The SEC also maintains an internet website that contains reports, proxy statements and other information about registrants, like us, that file electronically with the SEC. The address of that website is *www.sec.gov*.

Upon the closing of this offering, we will be required to file periodic reports, proxy statements, and other information with the SEC pursuant to the Exchange Act. These reports, proxy statements, and other information will be available on the website of the SEC referred to above.

We also maintain a website at *www.harmonybiosciences.com*, through which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Information contained on or accessed through our website is not a part of this prospectus and the inclusion of our website address in this prospectus is an inactive textual reference only.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors of
Harmony Biosciences Holdings, Inc. and Subsidiary:

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheet of Harmony Biosciences Holdings, Inc. (formerly Harmony Biosciences II, Inc.) and subsidiary (the "Company") as of December 31, 2018, the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' deficit, and cash flows for the year ended December 31, 2018, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018, and the results of its operations and its cash flows for the year ended December 31, 2018, in conformity with accounting principles generally accepted in the United States of America.

Going Concern

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 2 to the financial statements, the Company is experiencing difficulty in generating sufficient cash flow to meet its obligations and sustain its operations, which raises substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 2. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

Chicago, Illinois
February 14, 2020

We have served as the Company's auditor since 2017

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
CONSOLIDATED BALANCE SHEET
(U.S. dollars in thousands except share and per share data)

	December 31, 2018
ASSETS	
CURRENT ASSETS:	
Cash and cash equivalents	$ 83,523
Prepaid expenses	703
Other current assets	2,458
Total current assets	86,684
NONCURRENT ASSETS:	
Property and equipment, net	1,576
Restricted cash	500
Other noncurrent assets	522
Total noncurrent assets	2,598
TOTAL ASSETS	$ 89,282
LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' DEFICIT	
CURRENT LIABILITIES:	
Trade payables	$ 1,462
Accrued compensation	3,953
Accrued expenses	1,816
Total current liabilities	7,231
NONCURRENT LIABILITIES:	
Deferred rent	262
Other noncurrent liabilities	261
Total noncurrent liabilities	523
TOTAL LIABILITIES	7,754
COMMITMENTS AND CONTINGENCIES (Note 7)	
CONVERTIBLE PREFERRED STOCK	
Convertible preferred stock, net of placement costs	
Series A convertible preferred stock—$1.00 stated value; 286,000,000 shares authorized; 285,000,000 shares issued and outstanding at December 31, 2018	313,299
Series B convertible preferred stock—$1.25 stated value; 8,030,000 shares authorized; 8,000,000 shares issued and outstanding at December 31, 2018	10,902
STOCKHOLDERS' DEFICIT:	
Common stock—$0.00001 par value; 398,030,000 shares authorized; 63,888,876 issued and outstanding at December 31, 2018	1
Accumulated deficit	(242,674)
TOTAL STOCKHOLDERS' DEFICIT	(242,673)
TOTAL LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' DEFICIT	$ 89,282

The accompanying notes are an integral part of the consolidated financial statements

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
CONSOLIDATED STATEMENT OF OPERATIONS AND COMPREHENSIVE LOSS
(U.S. dollars in thousands except share and per share data)

	Year Ended December 31, 2018
OPERATING EXPENSES:	
Research and development	$ 12,372
Sales and marketing	16,861
General and administrative	12,206
Total operating expenses	41,439
Operating loss	(41,439)
Interest income (expense)	1,541
Loss before taxes	(39,898)
Income taxes	—
Net loss and comprehensive loss	$ (39,898)
Accumulation of yield on preferred stock	(30,185)
Net loss available to common stockholders	$ (70,083)
LOSS PER SHARE:	
Loss per share, basic and diluted	$ (0.96)
Weighted average number of shares of common stock, basic and diluted	72,765,366

The accompanying notes are an integral part of the consolidated financial statements

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
CONSOLIDATED STATEMENT OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(U.S. dollars in thousands except share and per share data)

	Convertible Preferred Stock Series A & B		Common Stock		Additional paid-in capital	Accumulated deficit	Total stockholders' deficit
	Shares	Amount	Shares (1)	Amount			
Balance as of December 31, 2017	270,000,000	266,750	77,221,876	$ 1	$ —	$ (168,020)	$ (168,019)
Net loss	—	—	—	—	—	(39,898)	(39,898)
Issuance of Series A convertible preferred stock, net of issuance cost	15,000,000	14,913	—	—		—	—
Issuance of Series B convertible preferred stock, net of issuance cost	8,000,000	9,905	—	—		—	—
Repurchase and cancellation of common shares	—	—	(13,333,000)	—	—	(3,200)	(3,200)
Preferred stock dividend, Series A	—	29,207	—	—	(1,079)	(28,128)	(29,207)
Preferred stock accretion, Series A	—	2,431	—	—	—	(2,431)	(2,431)
Preferred stock dividend, Series B	—	978	—	—	—	(978)	(978)
Preferred stock accretion, Series B	—	19	—	—	—	(19)	(19)
Stock-based compensation	—	—	—	—	1,079	—	1,079
Balance as of December 31, 2018	293,000,000	324,201	63,888,876	$ 1	$ —	$ (242,674)	$ (242,673)

(1) Common shares of Harmony Biosciences Holdings, Inc

The accompanying notes are an integral part of the consolidated financial statements

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
CONSOLIDATED STATEMENT OF CASH FLOWS
(U.S. dollars in thousands except share and per share data)

	Year Ended December 31, 2018
CASH FLOWS FROM OPERATING ACTIVITIES:	
Net loss	$ (39,898)
Adjustments to reconcile net loss to net cash used in operating activities:	
Depreciation	184
Stock-based compensation expense	1,079
Change in operating assets and liabilities:	
Prepaid expenses and other assets	(3,111)
Trade payables	(610)
Other liabilities and accrued expenses	3,557
Net cash used in operating activities	(38,799)
CASH FLOWS FROM INVESTING ACTIVITIES:	
Purchase of property and equipment	(1,342)
Net cash used in investing activities	(1,342)
CASH FLOWS FROM FINANCING ACTIVITIES:	
Proceeds from issuance of preferred stock	25,000
Preferred stock issuance costs	(185)
Repurchase of common stock	(3,200)
Net cash provided by financing activities	21,615
NET INCREASE (DECREASE) IN CASH	(18,526)
CASH, CASH EQUIVALENTS, AND RESTRICTED CASH—Beginning of period	102,549
CASH, CASH EQUIVALENTS, AND RESTRICTED CASH—End of period	$ 84,023
Supplemental Disclosure of Cash Flow Information:	
Supplemental Disclosures of Noncash Investing and Financing Activities:	
Series A Preferred Stock accrued return	$ 29,207
Series A accretion of issuance costs	2,431
Series B Preferred Stock accrued return	978
Series B accretion of issuance costs	20

The accompanying notes are an integral part of the consolidated financial statements

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

As of December 31, 2018 and for the Year Ended December 31, 2018
(U.S. dollars in thousands except share and per share data)

1. ORGANIZATION AND DESCRIPTION OF BUSINESS

Our operating subsidiary, Harmony Biosciences, LLC, was formed on May 17, 2017. Harmony Biosciences Holdings, Inc. (the "Company") was founded on July 25, 2017 as Harmony Biosciences II, LLC, a Delaware limited liability company, and the Company converted to a Delaware corporation named Harmony Biosciences II, Inc. on September 19, 2017. On February 3, 2020, the Company changed its name to Harmony Biosciences Holdings, Inc. The Company is a holding company and has no operations. The Company's operations are conducted in its wholly owned subsidiary, Harmony Biosciences, LLC ("Harmony"). Harmony is a commercial-stage pharmaceutical company focused on developing and commercializing innovative therapies for patients suffering from rare central nervous system disorders living with unmet medical needs. The Company is headquartered in Plymouth Meeting, Pennsylvania.

2. GOING CONCERN

The consolidated financial statements have been prepared as though the Company will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The Company has incurred operating losses and negative cash flows from operations since inception. As of December 31, 2018, the Company has an accumulated deficit of $242,674. Management expects to continue to incur operating losses and negative cash flows from operations in 2019. In addition, as more fully described in Note 4, the Company is subject to milestone payments associated with a license agreement, of which $127,000 were triggered in 2019 with other potential milestones of $142,000 yet to be triggered. The Company has financed its operations to date with proceeds from the sale of preferred securities.

The Company will need to raise additional capital in order to continue to fund operations, including milestone obligations under its licensing agreement. The Company believes that it will be able to obtain additional capital through equity financings or other arrangements to fund operations; however, there can be no assurance that such additional financing, if available, can be obtained on terms acceptable to the Company. If the Company is unable to obtain such additional financing, future operations would need to be scaled back or discontinued.

Accordingly, these factors raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the consolidated financial statements are issued. The consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might be necessary should the Company be unable to continue as a going concern.

3. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP) and include all adjustments necessary for the fair presentation of the Company's financial position for the periods presented. All intercompany accounts and transactions have been eliminated in consolidation.

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

As of December 31, 2018 and for the Year Ended December 31, 2018
(U.S. dollars in thousands except share and per share data)

Significant Risks and Uncertainties

The Company's operations are subject to a number of factors that can affect its operating results and financial condition. Such factors include, but are not limited to, the results of clinical testing and trial activities of the Company's product candidates; the Company's ability to obtain regulatory approval to market its products; competition from products manufactured and sold or being developed by other companies; the price of, and demand for, the Company's products, if approved; the Company's ability to negotiate favorable licensing or other manufacturing and marketing agreements for its product candidates; and the Company's ability to raise capital.

The Company currently has one commercially approved product, WAKIX, and there can be no assurance that the Company's research and development and clinical trials will result in any successfully commercialized products in addition to WAKIX. Developing and commercializing a product requires significant time and capital and is subject to regulatory review and approval as well as competition from other biotechnology and pharmaceutical companies. The Company operates in an environment of rapid change and is dependent upon the continued services of its employees and consultants and obtaining and protecting intellectual property.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosures of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Actual results could differ from those estimates.

The Company utilizes significant estimates and assumptions in determining the fair value of its common stock. The board of directors has determined the estimated fair value of the Company's common stock based on a number of objective and subjective factors, including external market conditions affecting the pharmaceutical industry sector; discounted cash flows; and the likelihood of achieving a liquidity event, such as an initial public offering of common stock or a sale of the Company.

Additionally, accounting for stock-based compensation requires fair value estimates of the equity instruments granted. The two factors that most affect charges or credits to operations related to stock-based compensation are the estimated fair value of the common stock for which stock-based compensation is recorded and the estimated volatility of such fair value.

Operating Segments

Harmony holds all its tangible assets and operations in the U.S. operating segments are defined as components of an entity for which separate financial information is available and that is regularly reviewed by the Chief Operating Decision Makers (CODM) in deciding how to allocate resources to an individual segment and in assessing performance. The Company has determined it operates in a single operating segment and has one reportable segment.

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

As of December 31, 2018 and for the Year Ended December 31, 2018
(U.S. dollars in thousands except share and per share data)

Fair Value of Financial Instruments

The Company's consolidated financial statements include cash, cash equivalents, accounts payable, and accrued liabilities, all of which are short term in nature and, accordingly, approximate fair value.

It is the Company's policy, in general, to measure nonfinancial assets and liabilities at fair value on a nonrecurring basis. The instruments are not measured at fair value on an ongoing basis, but are subject to fair value adjustments in certain circumstances (such as evidence of impairment), which, if material, are disclosed in the accompanying footnotes.

The Company measures certain assets and liabilities at fair value in accordance with Accounting Standards Codification ("ASC") 820, *Fair Value Measurements and Disclosures*. ASC 820 defines fair value as the price that would be received to sell an asset or paid to transfer a liability (the exit price) in an orderly transaction between market participants at the measurement date. The guidance in ASC 820 outlines a valuation framework and creates a fair value hierarchy that serves to increase the consistency and comparability of fair value measurements and the related disclosures. In determining fair value, the Company maximizes the use of quoted prices and observable inputs. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from independent sources. The fair value hierarchy is broken down into three levels based on the source of inputs as follows:

Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities.

Level 2—Valuations based on observable inputs and quoted prices in active markets for similar assets and liabilities.

Level 3—Valuations based on unobservable inputs and models that are supported by little or no market activity.

The Company's financial assets which are measured at fair value on a recurring basis were comprised of cash, cash equivalents, and restricted cash of $84,023 at December 31, 2018 based on Level 1 inputs.

Cash, Cash Equivalents and Restricted Cash

Cash and cash equivalents consist of cash and, if applicable, highly liquid investments with an original maturity of three months or less when purchased, including investments in Money Market Funds. The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the balance sheet that sum to the total of the same such amounts shown in the statement of cash flows.

	As of December 31, 2018
Cash and cash equivalents	$ 83,523
Restricted cash	500
Total cash, cash equivalents, and restricted cash shown in the statement of cash flows	$ 84,023

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

As of December 31, 2018 and for the Year Ended December 31, 2018
(U.S. dollars in thousands except share and per share data)

Amounts included in restricted cash represent those amounts required to be held as a security deposit in the form of letters of credit for the Company's credit card program.

Concentrations of Credit Risk

Substantially all of the Company's cash and money market funds are held with a single financial institution. Due to its size, the Company believes this financial institution represents minimal credit risk. Deposits in this institution may exceed the amount of insurance provided on such deposits by the Federal Deposit Insurance Corporation for U.S. institutions. The Company has not experienced any losses on its deposits of cash and cash equivalents. Management believes that the Company is not exposed to significant credit risk due to the financial position of the depository institutions in which those deposits are held.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation is computed using the straight-line method over the estimated useful lives of the assets, generally between three and ten years. Leasehold improvements are amortized using the straight-line method over the shorter of the estimated useful life of the asset or the term of the lease. The Company's leasehold improvements primarily relate to its new corporate headquarters in Plymouth Meeting, PA, and are generally being amortized through the end of the lease term in July 2024. When assets are retired or otherwise disposed of, the cost and accumulated depreciation are removed from the balance sheet and any resulting gain or loss is reflected in the statement of operations and comprehensive loss in the period realized.

Accrued Compensation

The Company accrues for liabilities under discretionary employee and executive bonus plans. These estimated compensation liabilities are based on progress against corporate objectives approved by the Company's board of directors, compensation levels of eligible individuals, and target bonus percentage levels. The board of directors reviews and evaluates the performance against these objectives and ultimately determines what discretionary payments are made. As of December 31, 2018, the Company accrued approximately $3,953 for liabilities associated with these employee and executive bonus plans.

Accrued Research and Development Costs

The Company records accrued liabilities for estimated costs of research and development activities conducted by collaboration partners and third-party service providers, which include the conduct of preclinical studies and clinical trials, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced, and includes these costs in accrued and other current liabilities on the balance sheets and within research and development expense on the statement of operations and comprehensive loss.

The Company accrues for these costs based on factors such as estimates of the work completed and in accordance with agreements established with its collaboration partners and third-party service

providers. The Company makes significant judgments and estimates in determining the accrued liabilities balance in each reporting period. As actual costs become known, the Company adjusts its accrued liabilities. The Company has not experienced any material differences between accrued costs and actual costs incurred since its inception.

Leases

The Company leases office space and recognizes related rent expense on a straight-line basis over the term of the lease. The Company has negotiated certain landlord/tenant incentives, rent holidays and escalations in the base price of rent payments under operating leases. The Company recognizes these incentives, rent holidays and rent escalations on a straight-line basis over the lease term. Deferred rent balances are classified as current or noncurrent in balance sheet based upon the period when reversal of the liability is expected to occur.

Convertible Preferred Stock

Preferred securities that are redeemable for cash or other assets are to be classified outside of permanent equity if they are redeemable (1) at a fixed or determinable price on a fixed or determinable date, (2) at the option of the holder, or (3) upon the occurrence of an event that is not solely within the issuer's control. The holders of preferred units have the right to redeem the units based on the passage of time and as a result are probable of becoming redeemable. As such, the Company concluded its preferred securities should be classified as convertible preferred stock. The redemption amount at each balance sheet date also includes amounts representing dividends not currently declared or paid, but which will be payable under the redemption and should be recognized as part of the instrument's carrying value (see Note 8 for further detail).

Research and Development Expenses

Research and development costs are expensed as incurred. Liabilities due to third parties in connection with research and development collaborations prior to regulatory approval are expensed as incurred.

Upfront payments and milestone payments made for licensing of technology are expensed as research and development in the period in which they are incurred. Advance payments for goods and services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Stock-Based Compensation

The Company recognizes compensation expense relating to stock-based payment transactions in operating results using a fair value measurement method, in accordance with Financial Accounting Standards Board (FASB) ASC 718, *Compensation-Stock Compensation*. ASC 718 requires all stock-based payments to employees to be recognized in operating results as compensation expense based on fair value over the requisite service period of the awards. The vesting period has a time-based provision consisting of a five-year period, with 20% vesting on each anniversary of the vesting

start date. Upon a change of control, any unvested awards will immediately vest. The Company determines the fair value of stock-based awards using the Black-Scholes option-pricing model, which uses both historical and current market data to estimate fair value. The method incorporates various assumptions, such as the risk-free interest rate, expected volatility, expected dividend yield, and expected life of the options.

The Company accounts for stock-based payments granted to nonemployees in accordance with ASC 505-50, *Equity Based Payments to Non-Employees*. The Company determines the fair value of the stock-based payment as the fair value of the equity instruments issued. It is measured on the grant date. The Company also has nonemployee stock awards subject to a performance condition and is recognized based on probable outcome. As of December 31, 2018, the Company determined that the performance condition was not probable. The fair value of the equity instruments is remeasured each reporting period over the requisite service period (see Note 10 for further detail).

Basic and Diluted Loss per Share

Basic net loss per share is determined using the weighted average number of shares of common stock outstanding during each period. Diluted net income per share includes the effect, if any, from the potential exercise or conversion of securities, such as convertible preferred stock and stock options, which would result in the issuance of incremental shares of common stock. The computation of diluted net loss per shares does not include the conversion of securities that would have an anti-dilutive effect. The basic and diluted computations of net loss per share for the Company are the same because the effects of the Company's convertible securities would be anti-dilutive (see Note 12 for further detail).

Income Taxes

Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and net operating loss and credit carryforwards. Deferred tax assets and liabilities are measured at rates expected to apply to taxable income in the years in which those temporary differences and carryforwards are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date. A valuation allowance is recorded when it is more likely than not that all or a portion of the net deferred tax assets will be realized.

The Company recognized the provisional income tax effects of the Tax Cuts and Jobs Act (the "Tax Reform Act") in its 2017 consolidated financial statements in accordance with Staff Accounting Bulletin No. 118, issued on December 22, 2017, which provides guidance on accounting for tax effects of the Tax Reform Act. As of December 22, 2018, the Company has completed its assessment of the income tax impacts of the Tax Reform Act. The Company has determined that there were no material impacts on its reported income tax provision amounts as of December 31, 2018. The impacts of the Tax Reform Act have been fully taken into consideration as of December 31, 2018 (see Note 11 for further detail).

Comprehensive Loss

The Company's comprehensive loss was the same as its reported net loss for all periods presented.

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARY
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

As of December 31, 2018 and for the Year Ended December 31, 2018
(U.S. dollars in thousands except share and per share data)

Emerging Growth Company Status

The Company is an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the JOBS Act). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. The Company has elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that it is (i) no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, these financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

Subsequent Events

The Company has evaluated and, as necessary, made changes to these consolidated financial statements for subsequent events through February 14, 2020, the date these consolidated financial statements were available to be issued. All subsequent events that provided additional evidence about conditions existing at the date of the consolidated statements of financial position were incorporated into the consolidated financial statements (see Note 14 for further detail).

Recently Issued Accounting Pronouncements

In March 2016, the FASB issued Accounting Standards Update (ASU) No. 2016-09, *Compensation—Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting*, with amended guidance that simplifies several aspects of the accounting for employee share-based payment transactions, including the accounting for forfeitures, as well as classification in the statement of cash flows. Since inception the Company has elected early adoption of ASU No. 2016-09 to recognize forfeitures as they occur.

In February 2016, the FASB issued amended guidance to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities in the balance sheet and disclosing key information about leasing arrangements. The new guidance clarifies the criteria for distinguishing between a finance lease and operating lease, as well as classification between the two types of leases, which is substantially unchanged from the previous lease guidance. Further, the new guidance requires a lessee to recognize in the statement of financial position a liability to make lease payments (the lease liability) and a right-of-use asset, initially measured at the present value of the lease payments. For finance leases, a lessee should recognize interest on the lease liability separately from amortization of the right-of-use asset. For operating leases, a lessee should recognize a single lease cost, calculated so that the cost of the lease is allocated over the lease term on a generally straight-line basis. For leases with a term of 12 months or less, a lessee is permitted to make an accounting policy election not to recognize lease assets and lease liabilities. The new standard will become effective for the Company's fiscal year ending December 31, 2020. The Company is currently assessing the impact of this amended guidance and the timing of adoption.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments*. ASU No. 2016-15 provides specific

guidance on eight cash flow issues where current guidance is unclear or does not include any specifics on classification, including contingent consideration payments made after a business combination and distributions received from equity method investees, among other items. The new standard will become effective for the Company's fiscal year ending December 31, 2019. The Company expects such provisions would result in an immaterial impact and will be reflected in the consolidated statement of cash flows for 2019.

In November 2016, the FASB issued ASU No. 2016-18, *Statement of Cash Flows (Topic 230): Restricted Cash*. This standard requires entities to show the changes in the total of cash, cash equivalents, restricted cash, and restricted cash equivalents in the statement of cash flows and no longer present transfers between cash and cash equivalents and restricted cash and restricted cash equivalents in the statement of cash flows. Since inception, the Company has early adopted the provisions of this ASU, with such provisions reflected in the consolidated statements of cash flows.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation – Stock Compensation (Topic 718): Improvements to Nonemployee Share – Based Payment Accounting.* The amended guidance is meant to simplify the accounting for share-based payments to nonemployees by aligning it with the accounting for share-based payments to employees, including, but not limited to, forfeitures, measurement date, and term used for measurement date. The Company has elected early adoption of ASU No. 2018-07, effective as of January 1, 2019. The Company expects such provisions would result in an immaterial impact and will be reflected in the consolidated balance sheets and statement of operations and comprehensive loss for 2019.

In August 2018, the FASB issued ASU No. 2018-15, *Intangibles – Goodwill and Other – Internal-Use Software (Topic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract.* The amended guidance requires a customer in a cloud computing arrangement that is a service contract to follow the internal-use software guidance in ASC-350-40, *Intangibles - Goodwill and Other - Internal-Use Software,* to determine which implementation costs to capitalize as an asset. The Company has elected early adoption of ASU No. 2018-15, effective as of September 30, 2018 with such provisions resulting in an immaterial impact reflected in the consolidated balance sheets and statement of operations and comprehensive loss.

4. LICENSE AGREEMENTS

On July 28, 2017, Harmony entered into a License and Commercialization Agreement (the "Agreement") with Bioprojet Societe Civile de Recherche ("Bioprojet") whereby Harmony acquired the exclusive right to commercialize the pharmaceutical compound Pitolisant for the treatment, and/or prevention, of narcolepsy, obstructive sleep apnea, idiopathic hypersomnia, and Parkinson's disease as well as any other indications unanimously agreed by the parties in the United States and its territories. On August 15, 2019 the Company received FDA approval of Wakix® (pitolisant) for treatment of excessive daytime sleepiness (EDS) in adult patients with narcolepsy. The Agreement called for an initial license payment of $150,000, which was recorded to research and development expense in the consolidated statement of operations and comprehensive loss for the period from May 17, 2017 (inception) to December 31, 2017. A milestone of $50,000 was due upon acceptance by the FDA of Pitolisant's New Drug Application (NDA), which was achieved on February 12, 2019. In

(U.S. dollars in thousands except share and per share data)

addition, a milestone of $77,000, including a $2,000 fee, was due upon FDA approval of WAKIX, which was achieved on August 15, 2019. The Agreement also requires sales-based milestones, a fixed trademark royalty and a tiered royalty, both based on net sales, become due and payable to Bioprojet on a quarterly basis with an additional milestone of $102,000 due upon FDA approval of other specific indications and $40,000 due upon reaching specific sales milestone.

5. ACCRUED EXPENSES

Accrued expenses consist of the following:

	As of December 31, 2018
Professional fees, consulting, and other services	$ 1,434
Debt issuance costs	313
Employee travel and other expenses	69
	$ 1,816

6. DEBT

On July 27, 2017, in connection with the Bioprojet Agreement described in Note 4, the Company entered into an agreement to issue an 8% convertible note in an aggregate of $150,000 in principal amount, whereby $100,000 of the principal could be settled through exchange for the issuance of preferred securities of Harmony Biosciences II, LLC upon consummation of an equity financing transaction. In addition, upon consummation of an equity financing transaction, holders of the notes would be issued warrants to purchase common units representing a total of 4% of the issued and outstanding common units determined on an “as converted” basis.

As part of the September 22, 2017 $270,000 Series A convertible preferred stock raise, as described in Note 8, the Company exchanged $100,000 of the original $150,000 principal amount into Series A convertible preferred stock and repaid, in cash, $50,000 of the remaining principal balance and any accrued interest on the notes through this date.

7. COMMITMENTS AND CONTINGENCIES

Litigation

From time to time, the Company is subject to claims and suits arising in the ordinary course of business. The Company accrues for such liabilities when they are known if they are deemed probable and can be reasonably estimated.

Lease Agreements

In April 2018, the Company entered into an operating lease for approximately nine thousand square feet of office space in Northbrook, IL, which expires in January 2020. In June 2018, the Company entered into an operating lease for approximately seven thousand square feet of office space in Plymouth Meeting, PA. The offices of the Company are rented under an operating lease agreement.

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The terms of the lease payments provide for rental payments on a monthly basis and on a graduated scale. The Company recognizes rent expense on a straight-line basis over the lease period and has accrued for rent expense incurred but not paid. In addition, tenant improvement allowances recorded are amortized as a reduction to rent expense on a straight-line basis over the lease term. Rent expense was $381 for the year ended December 31, 2018.The following table sets forth the lease payment obligations as of December 31, 2018, for the periods indicated below:

Years ending December 31,	
2019	$ 265
2020	459
2021	468
2022	478
2023	487
2024	205
Thereafter	—
Total	**$2,362**

8. CONVERTIBLE PREFERRED STOCK

Series A Preferred Stock

On September 22, 2017, the Company issued 270,000,000 shares of Series A convertible preferred stock for a purchase price of $1.00 per share, or $270,000 in the aggregate. On January 8, 2018, the Company issued an additional 15,000,000 shares of Series A convertible preferred stock for a purchase price of $1.00 per share, or $15,000 in the aggregate. As of December 31, 2018, there were 286,000,000 Series A convertible preferred stock authorized of which 285,000,000 were issued and outstanding. Each outstanding share of Series A convertible preferred stock accrues dividends at 10% per annum of the Series A original issue price, subject to adjustment for stock splits, combinations, recapitalizations, stock dividends and similar transactions. Preferred dividends on the Series A convertible preferred stock are cumulative and are compounded annually. The cumulative unpaid preferred return was $36,604 at December 31, 2018. For the period ended December 31, 2018 accretion of issuance costs of $2,431 was recorded as a direct charge to retained earnings, while issuance costs not yet accreted of $8,305 are recorded as a direct reduction of Series A convertible preferred stock in the Company's consolidated balance sheet.

Series B Preferred Stock

On January 8, 2018, the Company issued 8,000,000 shares of Series B convertible preferred stock for a purchase price of $1.25 per share, or $10,000 in the aggregate. As of December 31, 2018, there were 8,030,000 shares of Series B convertible preferred stock authorized, of which 8,000,000 were issued and outstanding. Each outstanding share of Series B convertible preferred stock accrues dividends at 10% per annum of the Series B original issue price, subject to adjustment for stock splits, combinations, recapitalizations, stock dividends and similar transactions. Preferred dividends on the Series B convertible preferred stock are cumulative and are compounded annually. The cumulative unpaid preferred return was $978 at December 31, 2018. As of December 31, 2018, accretion of issuance costs of $20 was recorded as a direct charge to retained earnings, while issuance costs not

yet accreted of $75 are recorded as a direct reduction of Series B convertible preferred stock in the Company's consolidated balance sheet.

Redemption

The holders of a majority of the issued and outstanding Series A and Series B convertible preferred stock may require that the Company redeem all of the issued and outstanding shares of Series A convertible preferred stock and Series B convertible preferred stock at any time on or after September 22, 2021. The per share redemption price will be equal to the Series A original issue price for the Series A convertible preferred stock and the Series B original issue price for the Series B convertible preferred stock, plus, in each case, the amount of accrued and unpaid preferred dividends with respect to such shares.

Optional Conversion Rights

Each share of Series A convertible preferred stock and Series B convertible preferred stock is convertible, at any time at the option of the holder, into such number of fully paid shares of common stock as is determined by dividing (x) the applicable original issuance price by (y) the conversion price in effect at the time of conversion. Accordingly, each share of Series A and Series B convertible preferred stock is convertible into common stock on a one-for-one basis. Each applicable conversion price is subject to adjustment for any stock dividends, stock splits or stock combinations, reclassifications or exchanges of similar stock, upon a reorganization, merger or consolidation of the Company, or upon the issuance or sale by the Company of common stock for consideration less than the applicable conversion price.

Mandatory Conversion Rights

Each share of Series A convertible preferred stock and Series B convertible preferred stock will automatically convert into the number of shares of common stock determined in accordance with the conversion rate applicable to optional conversions, as described above, upon the closing of the sale of shares of the Company's common stock to the public at a price of at least $2.00 per share (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the common stock), in a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, resulting in at least $100,000 of gross proceeds, net of underwriting discounts and commissions, to the Company.

Dividends

The holders of Series A convertible preferred stock and Series B convertible preferred stock are entitled to receive, when and if declared by the board of directors of the Company, cumulative dividends equal to a 10% per annum of Series A convertible preferred stock and 10% per annum of Series B convertible preferred stock. In addition, the holders of the outstanding shares of Series A and Series B convertible preferred stock are entitled to receive, when and if declared by the board of directors of the Company, a dividend at least equal to any dividend payable on the Company's common stock as if all convertible preferred stock had been converted to common stock. No dividends have been declared as of December 31, 2018.

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Liquidation

In the event of any liquidation, dissolution, or winding up of the Company, either voluntary or involuntary, the holders of Series A convertible preferred stock and Series B convertible preferred stock shall be entitled to receive pro rata, prior and in preference to any distribution to the holders of the common stock, an amount equal to the greater of (i) the original issuance prices of each series (in each case, as adjusted for stock splits, stock dividends or distributions, recapitalizations, and similar events) and all accrued but unpaid dividends, if any or (ii) such amount per share as would have been payable had all shares of Series A convertible preferred stock and Series B convertible preferred stock been converted to common stock. If the assets and funds to be distributed among the holders of convertible preferred stock are insufficient to permit the payment to such holders, then the entire assets and funds of the Company legally available for distribution will be distributed ratably among the holders of convertible preferred stock in proportion to the preferential amount each such holder is otherwise entitled to receive.

Voting Rights

Each share of convertible preferred stock has a number of votes equal to the number of shares of common stock into which it is convertible. The holders of convertible preferred stock, voting together as a single class, shall be entitled to elect six members of the Company's board of directors. The holders of common stock have the right to elect two members of the Company's board of directors. With respect to any other matter presented to the stockholders for their consideration or action at any meeting of the board of directors, the holders of the Series A convertible preferred stock and Series B convertible preferred stock are entitled to cast the number of votes equal to the number of whole shares of common stock into which such preferred shares are convertible as of the record date for determining stockholders entitled to vote on such matter. Except as provided by law or by the other provisions of the Certificate of Incorporation, holders of the Series A convertible preferred stock and Series B convertible preferred stock are entitled to vote together with the holders of common stock as a single class. In addition, certain matters, prior to being able to be undertaken by the Company, require the approval of a majority of the holders of the Company's convertible preferred stock, voting as a separate class.

9. STOCKHOLDERS' DEFICIT

Common Stock

On September 19, 2017, Harmony Biosciences II, LLC was converted to a C corporation named Harmony Biosciences II, Inc., at which point 63,333,000 of outstanding common units were converted to 63,333,000 of common shares.

On September 22, 2017, the Company issued 13,888,870 warrants, with an exercise price of $0.01 per share, to the holders of the 8% convertible notes upon the consummation of an equity financing transactions, as described in Note 6, and these warrants were immediately exercised resulting in the issuance of 13,888,870 common shares and proceeds of $139.

On August 31, 2018, the Company repurchased and canceled 13,333,000 of common shares from the former chief executive officer for $3,200.

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As of December 31, 2018, there were 398,030,000 common shares authorized of which 63,888,876 were issued and outstanding. After the preferences of the preferred stock are paid, distributions are made to the holders of the common shares.

Holders of common shares are entitled to one vote for each share of common shares held. Holders of common shares have voting privileges with respect to the election of two of the eight directors of the board of directors of the Company, and any other matter presented to the shareholders for their consideration or action at any meeting of the board of directors. Holders of common shares may not vote on amendments to the Company's Certificate of Incorporation that relate solely to the terms of one or more outstanding Series of preferred stock if the holders of such affected Series are entitled, either separately or together with the holders of one or more other such Series, to vote thereon pursuant to the Certificate of Incorporation or pursuant to the Delaware General Corporation Law.

10,000,000 common shares held by an investor are subject to certain forfeiture provisions that are dependent upon the outcome of certain future events. In accordance with ASC 505-50-30, *Equity Based Payments to Non-Employees*, no expense has been reflected in the Company's consolidated results of operations for all periods. On November 15, 2019 the Company removed the provision associated with this forfeiture.

10. STOCK INCENTIVE PLAN AND STOCK-BASED COMPENSATION

Stock Incentive Plan

On August 7, 2017, the Company adopted an equity incentive plan (the "Plan"). Under the Plan, directors, officers, employees, consultants, and advisors of the Company can be paid incentive compensation measured by the value of the Company's common shares through grants of stock options, stock appreciation rights, or restricted stock.

Awards under the Plan have a 10-year contractual term and vest over the vesting period specified in the applicable award agreement (generally five years from the date of grant), at achievement of a performance requirement, or upon change of control (as defined in the applicable plan).

Changes in stock options granted under the Plan as of December 31, 2018, are as follows:

	Number of Shares	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term
Awards outstanding—December 31, 2017	8,615,683	$ 1.00	9.78
Stock options issued	8,374,600	$ 1.00	
Stock options forfeited	(625,000)	$ 1.00	
Awards outstanding—December 31, 2018	16,365,283	$ 1.00	9.07

As of December 31, 2018, 1,535,137 options issued under the Plan were vested. The Company has elected early adoption of ASU No. 2016-09 to recognize forfeitures as they occur. As a result of the adoption, the Company reversed $10 of stock-based compensation previously recorded.

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Value of Stock Options

The Company has valued awards for each of the plans included herein using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, the Company estimates its expected stock volatility based on historical volatility of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for the time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The assumptions used to value the awards are summarized in the following table.

	2018
Dividend yield	0.00%
Expected volatility	122.00%
Risk-free interest rate	2.39%
Lack of marketability discount	43.00%
Expected term (years)	6.50

The weighted-average per share fair market value of awards issued under the Plan was $0.40 in 2018.

Stock-based compensation expense was $1,079 for the year ended December 31, 2018 and was recorded in the consolidated statement of operations and comprehensive loss in the following line items:

	Year Ended December 31, 2018
Research and development expense	$ 209
Sales and marketing expense	415
General and administrative expense	455
	$ 1,079

Awards issued under the Plan are reflected as a component of equity in these consolidated financial statements. The Company will recognize compensation expense for these awards as summarized in the following table.

Years Ending December 31,	Stock Compensation Expense
2019	$1,309
2020	1,309
2021	1,309
2022	1,175
2023	220

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11. INCOME TAXES

Details of the provision for income taxes consist of the following:

	Year Ended December 31, 2018
Federal	$ (9,006)
State	(3,145)
Valuation allowance	12,151
Total	$ —
Current	$ —
Deferred	(12,151)
Valuation allowance	12,151
Total	$ —

On December 22, 2017, the Tax Reform Act was signed into law by the President, resulting in significant changes to the US income tax code. The Tax Reform Act, among other things, reduces the US federal income tax rate to 21% from a top rate of 35% starting in 2018. The reasons for the difference between the statutory federal income tax rate and the Company's effective income tax rate as of December 31, 2018, are as follows:

	As of December 31, 2018
Federal income tax rate	21.0%
Provisional effect of the tax reform act	—
State taxes	7.9
Other	1.5
Valuation allowance	(30.4)
Total	—%

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Significant components of the Company's deferred tax assets and liabilities as of December 31, 2018, are as follows:

	As of December 31, 2018	
	Assets	Liabilities
Acquired in-process research and development	$ 39,581	$ —
Net operating loss carryforward	16,619	—
Accrued compensation	1,432	—
Credits	837	—
Deferred rent	95	—
Fixed assets	—	85
Other	57	29
Total	$ 58,621	$ 114
Net deferred tax asset	$ 58,507	$ —
Valuation allowance	(58,507)	—
Total	$ —	$ —

The Company has considered available positive and negative evidence to estimate if sufficient future taxable income will be generated to allow utilization of the existing deferred tax assets. The Company has incurred operating losses and negative cash flows from operations since inception and expects a loss in 2019. At December 31, 2018 the Company had federal net operating loss carryforwards of $12,066 with no expiration. At December 31, 2018 the Company had state net operating loss carryforwards of $4,553 with a 20-year expiration. Utilization of the net operating loss carryforwards may be subject to a substantial limitation due to ownership change limitations that may occur in the future, as required by Section 382 of the Internal Revenue Code of 1986, as amended (the "Code"), as well as similar state provisions. These ownership changes may limit the amount of net operating loss and tax credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. In general, an "ownership change" as defined by Section 382 of the Code results from a transaction or series of transactions over a three-year period resulting in an ownership change of more than 50 percentage points of the outstanding stock of a company by certain stockholders or public groups. In light of these considerations as well as uncertainty as to when the Company might generate taxable income, the Company has recorded a full valuation allowance of $58,507. The amount of the net deferred tax asset considered realizable could be adjusted in the future if estimates of taxable income change or if objective negative evidence is no longer present and additional weight may be given to subjective evidence.

12. NET LOSS PER SHARE

The Company used the two-class method to compute net income (loss) per common share because the Company has issued securities (convertible preferred stock) that entitle the holder to participate in dividends and earnings of the Company. Under this method, net income is reduced by the amount of any dividends earned and the accretion of convertible preferred stock to its redemption value during the period. The remaining earnings (undistributed earnings) are allocated to common stock and each series of convertible preferred stock to the extent that each preferred security may

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share in the earnings as if all of the earnings for the period had been distributed. The total earnings allocated to common stock is then divided by the number of outstanding shares to which the earnings are allocated to determine the earnings per share. The two-class method is not applicable during periods with a net loss, as the holders of the convertible preferred stock have no obligation to fund losses.

Diluted net income (loss) per common share is computed under the two-class method by using the weighted average number of shares of common stock outstanding, plus, for periods with net income attributable to common stockholders, the potential dilutive effects of stock options, warrants, and convertible debt. In addition, the Company analyzes the potential dilutive effects of the outstanding convertible preferred stock under the 'if-converted' method when calculating diluted earnings per share, in which it is assumed that the outstanding convertible preferred stock converts into common stock at the beginning of the period or when issued if later. The Company reports the more dilutive of the approaches (two-class or 'if converted') as their diluted net income per share during the period.

The Company has reported a net loss for the year ended December 31, 2018 and the basic and diluted net loss per share attributable to common stockholders are the same for the year because all convertible preferred stock and stock options have been excluded from the computation of diluted weighted-average shares outstanding because such securities would have an antidilutive impact.

The following table sets forth the computation of basic and diluted net loss per share:

	Year Ended December 31, 2018
Numerator	
Net Loss	$ (39,898)
Accumulation of yield on preferred stock	(30,185)
Net loss available to common shareholders	$ (70,083)
Denominator	
Weighted-average common shares outstanding, basic and diluted	72,765,366
Net loss per share attributed to common stockholders, basic and diluted	$ (0.96)

Potential common shares issuable upon conversion of preferred stock and exercise of stock options that are excluded from the computation of diluted weighted-average shares outstanding are as follows:

	Year Ended December 31, 2018
Stock options to purchase common stock	13,825,301
Convertible preferred stock	292,495,890
Total	306,321,191

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13. RELATED-PARTY TRANSACTIONS

The Company is party to a management agreement for professional services provided by a related party. The related party is an entity that shares common ownership with the Company. In addition, the Company's Chairman of the board of directors is the president and owner of the entity. For the year ended December 31, 2018, the Company incurred $4,276 in management fee expense and other expenses to this related party, which are included in general and administrative expense in the consolidated statement of operations and comprehensive loss. In addition, the Company participates in certain transactions with separate related parties that also share common ownership with the Company, primarily related to combined employee health plans. As of December 31, 2018, the amount due to related parties included in accounts payable is $182 and the amount due from related parties included in other assets is $42.

14. SUBSEQUENT EVENTS

On February 12, 2019, the Company received FDA acceptance for the NDA. This event triggered a milestone payment of $50,000 associated with its license agreement with Bioprojet. This milestone was settled in cash on February 26, 2019.

On February 28, 2019, the Company entered into a multi-draw loan agreement with CRG Servicing LLC for an aggregate of $200,000 (the "Loan"), which matures in March 2025. The Loan bears a fixed rate of 12%. The Loan agreement requires compliance with certain financial covenants. The Company can draw three tranches of the Loan based on achieving specific milestones and dates. The Company may elect to pay the interest on the outstanding principal amount as follows: (i) only 7.5% of the 12% per annum in cash, paid quarterly, starting in March 2019, and (ii) 4.5% of the 12% per annum interest as compounded interest, added to the aggregate outstanding principal balance quarterly; the amount of any such compounded interest being a paid-in-kind loan. As of December 31, 2018, there was $313 of debt issuance costs incurred and recorded as a noncurrent asset, of which $313 were paid out of the March 2019 proceeds. These costs will be amortized as additional interest expense over the six-year loan term. As of December 31, 2018, the Company had no outstanding debt.

On March 5, 2019, the Company borrowed the first tranche of the Loan for $20,000, resulting in cash proceeds received of $19,400, net of issuance costs.

On May 13, 2019, the Company borrowed the second tranche of the Loan for $55,000, resulting in cash proceeds received of $54,200, net of issuance costs.

On August 9, 2019, the Company raised $50,000 by issuing 25,510,205 Series C preferred stock.

On August 15, 2019, the Company received FDA approval of WAKIX® (pitolisant) for the treatment of EDS in adult patients with narcolepsy. This event triggered a milestone payment of $77,000 associated with its license agreement with Bioprojet. The milestone was settled in two cash payments, $2,000 on August 27, 2019 and $75,000 on November 12, 2019. During the fourth quarter of 2019 the Company launched and commercialized Wakix.

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On November 8, 2019, the Company borrowed the third tranche of the Loan for $25,000, resulting in cash proceeds received of $24,625, net of issuance costs.

On January 9, 2020 the Company entered into a credit agreement with OrbiMed Royalty & Credit Opportunities III, LP for an aggregate amount of $200,000 (the “New Loan”), which matures in January 2026. The New Loan bears an interest rate equal to the sum of (i) the greater of (a) 1-month LIBOR or (b) 2.00% per annum, plus (ii) 11.00% per annum, paid in cash monthly in arrears on the last day of each month starting in January 2020. In addition to entering into the New Loan, the Company extinguished the previous Loan with CRG Servicing LLC which required a payoff amount of $120,893 consisting of principal repayment, interest, and exit fees. Net cash received as a result of the transaction, less debt issuance costs of $5,778, was $73,313.

Shares

Common Stock

Goldman Sachs & Co. LLC **Jefferies** **Piper Sandler**

Through and including , 2020 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

PART II

INFORMATION NOT REQUIRED IN THE PROSPECTUS

Item 13. Other expenses of issuance and distribution.

The following table sets forth all fees and expenses, other than the underwriting discounts and commissions payable solely by Harmony Biosciences Holdings, Inc. in connection with the offer and sale of the securities being registered. All amounts shown are estimated except for the SEC registration fee, the Financial Industry Regulatory Authority, Inc., or FINRA, filing fee and the exchange listing fee.

	Amount to be paid
SEC registration fee	$ *
FINRA filing fee	*
Exchange listing fee	*
Accounting fees and expenses	*
Legal fees and expenses	*
Printing expenses	*
Transfer agent and registrar fees	*
Miscellaneous expenses	*
Total	**$ ***

* To be completed by amendment.

Item 14. Indemnification of directors and officers.

Section 102 of the General Corporation Law of the State of Delaware permits a corporation to eliminate the personal liability of directors of a corporation to the corporation or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit. Our amended and restated certificate of incorporation provides that no director of Harmony Biosciences Holdings, Inc. shall be personally liable to it or its stockholders for monetary damages for any breach of fiduciary duty as a director, notwithstanding any provision of law imposing such liability, except to the extent that the General Corporation Law of the State of Delaware prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145 of the General Corporation Law of the State of Delaware provides that a corporation has the power to indemnify a director, officer, employee, or agent of the corporation, or a person serving at the request of the corporation for another corporation, partnership, joint venture, trust or other enterprise in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he was or is a party or is threatened to be made a party to any threatened, ending or completed action, suit or proceeding by reason of such position, if such person acted in good faith and in a manner he reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that

the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

Upon consummation of this offering, our amended and restated certificate of incorporation and amended and restated bylaws will provide indemnification for our directors and officers to the fullest extent permitted by the General Corporation Law of the State of Delaware. We will indemnify each person who was or is a party or threatened to be made a party to any threatened, pending or completed action, suit or proceeding (other than an action by or in the right of us) by reason of the fact that he or she is or was, or has agreed to become, a director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (all such persons being referred to as an "Indemnitee"), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding and any appeal therefrom, if such Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, and, with respect to any criminal action or proceeding, he or she had no reasonable cause to believe his or her conduct was unlawful. Our amended and restated certificate of incorporation and amended and restated bylaws will provide that we will indemnify any Indemnitee who was or is a party to an action or suit by or in the right of us to procure a judgment in our favor by reason of the fact that the Indemnitee is or was, or has agreed to become, a director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise, or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees) and, to the extent permitted by law, amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding, and any appeal therefrom, if the Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, except that no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to us, unless a court determines that, despite such adjudication but in view of all of the circumstances, he or she is entitled to indemnification of such expenses. Notwithstanding the foregoing, to the extent that any Indemnitee has been successful, on the merits or otherwise, he or she will be indemnified by us against all expenses (including attorneys' fees) actually and reasonably incurred in connection therewith. Expenses must be advanced to an Indemnitee under certain circumstances.

Prior to the consummation of this offering, we intend to enter into separate indemnification agreements with each of our directors and executive officers. Each indemnification agreement will provide, among other things, for indemnification to the fullest extent permitted by law and our amended and restated certificate of incorporation and amended and restated bylaws against any and all expenses, judgments, fines, penalties and amounts paid in settlement of any claim. The indemnification agreements will provide for the advancement or payment of all expenses to the indemnitee and for the reimbursement to us if it is found that such indemnitee is not entitled to such indemnification under applicable law and our amended and restated certificate of incorporation and amended and restated bylaws.

We maintain a general liability insurance policy that covers certain liabilities of directors and officers of our corporation arising out of claims based on acts or omissions in their capacities as directors or officers.

In any underwriting agreement we enter into in connection with the sale of common stock being registered hereby, the underwriters will agree to indemnify, under certain conditions, us, our directors, our officers and persons who control us within the meaning of the Securities Act of 1933, as amended (the "Securities Act") against certain liabilities.

Item 15. Recent sales of unregistered securities.

During the past three years, we issued securities that were not registered under the Securities Act as set forth below. The following is a summary of transactions during the preceding three fiscal years involving sales of our securities that were not registered under the Securities Act:

(a) Issuance of Capital Stock

From September 22, 2017 through January 8, 2018, we issued and sold an aggregate of 285,000,000 shares of our Series A convertible preferred stock, or Series A stock, at a purchase price of $1.00 per share for aggregate consideration of approximately $285,000,000.

On January 8, 2018, we issued and sold an aggregate of 8,000,000 shares of our Series B convertible preferred stock, or Series B stock, at a purchase price of $1.25 per share for aggregate consideration of approximately $10,000,000.

On August 9, 2019, we issued and sold an aggregate of 25,510,203 shares of our Series C convertible preferred stock at a purchase price of $1.96 per share, for aggregate consideration of approximately $50,000,000.

No underwriters were involved in the foregoing issuances of securities. The securities described in this section (a) of Item 15 were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(a)(2) under the Securities Act and Regulation D promulgated thereunder relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. The recipients of securities in the transactions described above represented that they were accredited investors and were acquiring the securities for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the instruments representing such securities issued in such transactions.

(b) Stock Option Grants and Option Exercises.

From September 16, 2017 through December 31, 2019, we granted to our employees, directors, consultants and certain employees and affiliates of Paragon options to purchase up to 20,380,283 shares of common stock under our Equity Incentive Plan, at an exercise price of $1.00 per share. 1,161,984 of these options were terminated, expired without being exercised or were otherwise forfeited.

As of December 31, 2019, we have issued an aggregate of 85,190 shares of common stock pursuant to the exercise of stock options by affiliates of Paragon. These issuances were exempt from the registration requirements of the Securities Act pursuant to Section 4(w) of the Securities Act, Rule 701 and/or Regulation S.

From September 16, 2017 through December 31, 2019, we granted stock appreciation rights to certain employees and affiliates of Paragon for up to 380,000 shares of common stock under our Equity Incentive Plan, at an exercise price of $1.00 per share.

No underwriters were involved in the foregoing issuances of securities. The issuances of stock options described in this paragraph (b) of Item 15 were issued pursuant to written compensatory plans or arrangements with our employees, directors, consultants and advisors, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(a)(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

Item 16. Exhibits and financial statements.

Exhibit No.	
1.1*	Form of Underwriting Agreement.
3.1*	Amended and Restated Certificate of Incorporation of the Registrant, as in effect prior to the consummation of this offering.
3.2*	Form of Amended and Restated Certificate of Incorporation of the Registrant, to be in effect upon the consummation of this offering.
3.3*	Form of Amended and Restated Bylaws of the Registrant, to be in effect upon the consummation of this offering.
4.1*	Specimen Stock Certificate evidencing the shares of common stock.
5.1*	Opinion of Latham & Watkins LLP.
10.1*	Credit Agreement, dated as of January 9, 2020, among Harmony Biosciences, LLC, the Lenders from time to time party thereto and OrbiMed Royalty & Credit Opportunities III, LP.
10.2*	Pledge and Security Agreement, dated as of January 9, 2020, among Harmony Biosciences, LLC, the Registrant, OrbiMed Royalty & Credit Opportunities III, LP and the Secured Parties as defined therein.
10.3*†	Harmony Biosciences II, Inc. Equity Incentive Plan and form of agreement.
10.4*†	2020 Incentive Award Plan and form of agreement.
10.5*†	Employment Agreement, dated September 6, 2017, by and between Harmony Biosciences, LLC and John C. Jacobs.
10.6*†	Offer Letter, dated October 10, 2017, by and between Harmony Biosciences, LLC and Jeffrey Dayno.
10.7*†	Offer Letter, dated September 8, 2017, by and between Harmony Biosciences, LLC and Andrew Serafin.
10.8*†	Offer Letter, dated September 29, 2018, by and between Harmony Biosciences, LLC and John Vittoria.
10.9*	Form of Indemnification Agreement between Harmony Biosciences, LLC and each director and executive officer.
10.10*#	License and Commercialization Agreement, dated July 28, 2017, by and between Bioprojet Société Civile de Recherche and Harmony Biosciences, LLC.
10.11*	Amendment No. 1 to License and Commercialization Agreement, dated August 27, 2018, by and between Bioprojet Société Civile de Recherche and Harmony Biosciences, LLC.
10.12*	Management Services Agreement, dated September 22, 2017, by and between Paragon Biosciences, LLC and Harmony Biosciences, LLC.
10.13*	Right of Use Agreement, dated November 1, 2019, by and between Paragon Biosciences, LLC and Harmony Biosciences, LLC.
10.14*	Second Amended and Restated Investors' Rights Agreement, dated August 9, 2019, by and among the Registrant and the other parties thereto.
21.1*	List of Subsidiaries of the Registrant.
23.1*	Consent of Deloitte & Touche LLP, independent registered public accounting firm.
23.2*	Consent of Latham & Watkins LLP (included in Exhibit 5.1).
24.1*	Power of Attorney (included on signature page).

* To be filed by amendment.
† Indicates a management contract or compensatory plan or arrangement.
Certain confidential portions of this Exhibit were omitted by means of marking such portions with brackets ("[***]") because the identified confidential portions (i) are not material and (ii) would be competitively harmful if publicly disclosed.

***

Item 17. Undertakings.

(a) The undersigned registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

(b) Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction, the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

(c) The undersigned hereby further undertakes that:

(1) For purposes of determining any liability under the Securities Act the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial *bona fide* offering thereof.

SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, as amended, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized in the City of Plymouth Meeting, State of Pennsylvania, on , 2020.

HARMONY BIOSCIENCES HOLDINGS, INC.

By: _____
Name: John C. Jacobs
Title: Chief Executive Officer and Director

POWER OF ATTORNEY

Each of the undersigned officers and directors of Harmony Biosciences Holdings, Inc. hereby constitutes and appoints John C. Jacobs and Susan L. Drexler, and each of them any of whom may act without joinder of the other, the individual's true and lawful attorneys-in-fact and agents, each with full power of substitution and resubstitution, for the person and in his or her name, place and stead, in any and all capacities, to sign this registration statement on Form S-1, and any other registration statement relating to the same offering (including any registration statement, or amendment thereto, that is to become effective upon filing pursuant to Rule 462(b) under the Securities Act of 1933, as amended), and any and all amendments thereto (including post-effective amendments to the registration statement), and to file the same, with all exhibits thereto, and all other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents or any of them, or their substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, as amended, this registration statement on Form S-1 has been signed by the following persons in the capacities set forth opposite their names and on the date indicated above.

Signature	Title	Date
_____ John C. Jacobs	Chief Executive Officer and Director (Principal Executive Officer)	, 2020
_____ Susan L. Drexler	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	, 2020
_____ Jeffrey S. Aronin	Chairman of the Board	, 2020
_____ Martin Edwards	Director	, 2020
_____ Antonio Gracias	Director	, 2020

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Signature	Title	Date
_____ Jack Bech Nielsen	Director	, 2020
_____ Aaron Royston	Director	, 2020
_____ Juan A. Sabater	Director	, 2020
_____ Dr. Andreas Wicki	Director	, 2020