



Q4 and Full Year 2023 Financial and Business Update

February 22, 2024



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Strong Momentum in Execution of Our Growth Strategy

Continued Strong Growth For WAKIX® in Adult Narcolepsy

- FY 2023 WAKIX Net Revenue of \$582.0M **+33% Year-over-Year Growth**
- **~6,150** average number of patients on WAKIX in Q4 2023
- **Continued strong growth** in average number of patients & WAKIX prescriber base
- **Demonstrated durability of the brand** entering year five on the market; **2024 Net Revenue guidance of \$700-\$720M**

Strong Momentum in Advancing and Expanding the Pipeline

- **FDA granted Priority Review for pediatric narcolepsy sNDA**; PDUFA date of June 21, 2024
- **Meeting with the FDA** to discuss Idiopathic Hypersomnia development program scheduled for March 2024
- **FDA granted Orphan Drug designation** to Pitolisant for PWS; Phase 3 TEMPO study expected to initiate in Q1 24
- **Reported positive topline results** from DM1 Phase 2 POC study in EDS and fatigue
- **Advanced Next-Gen pitolisant based** formulations into the clinic; on track to report pharmacokinetic data in 1H 24
- **Expanded the pipeline and diversified the portfolio** with acquisition of Zynerba; ZYN002 in Phase 3 pivotal trial for Fragile X syndrome and Phase 3 ready for 22q deletion syndrome

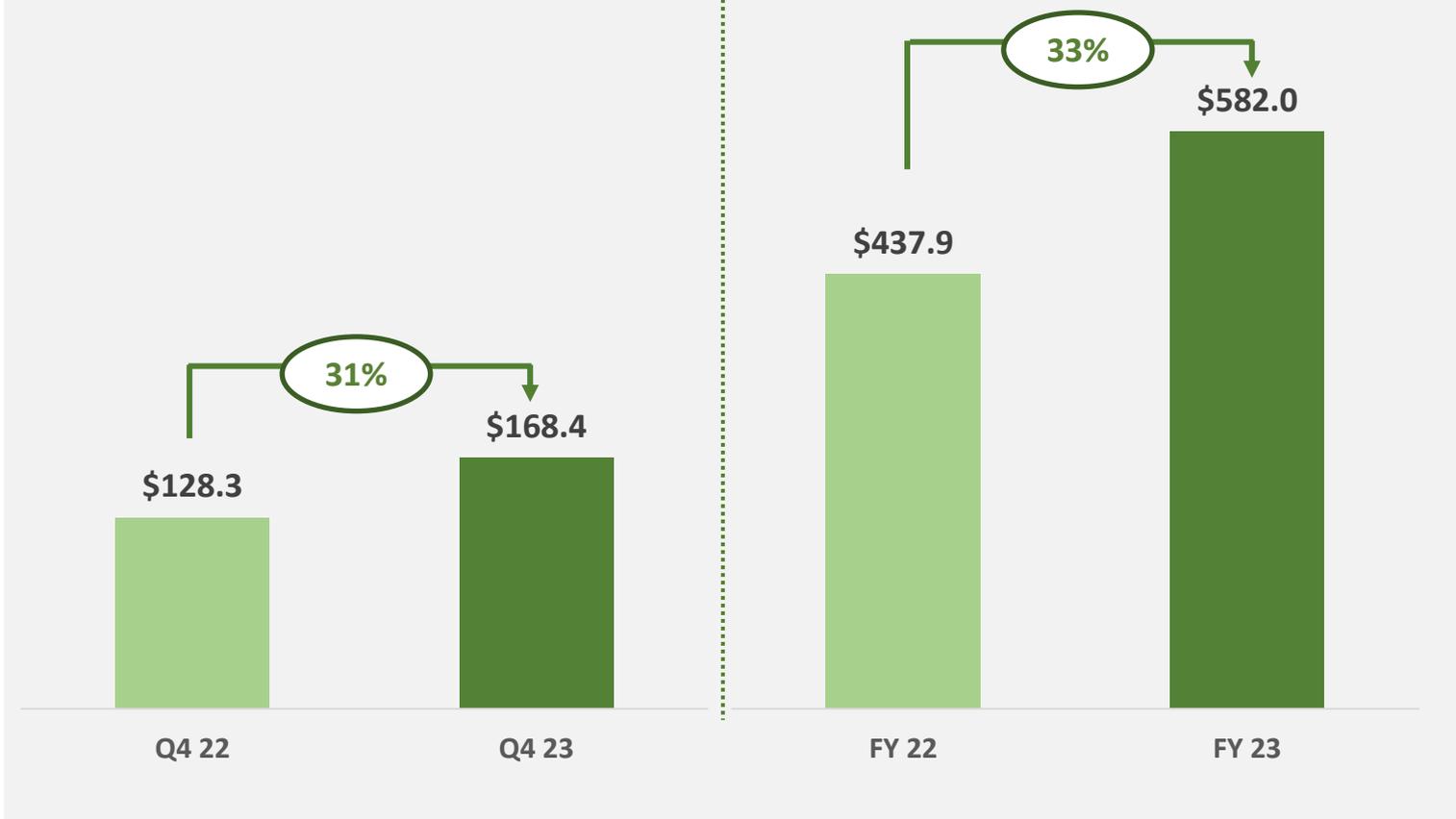
Disciplined Capital Allocation to Maximize Shareholder Value

- **Profitable, cash generating** with **\$425.6M** on the balance sheet as of December 31, 2023
- **Share repurchase program**: Repurchased ~3.2M shares of common stock at an aggregate cost of \$100M during 2023; remaining authorization of \$150M
- **Well positioned** to execute on business development to build out robust pipeline

WAKIX® Net Revenue Performance

CONFIDENT IN WAKIX BEING A POTENTIAL \$1B+ OPPORTUNITY IN ADULT NARCOLEPSY ALONE WITH THE POTENTIAL TO CONTRIBUTE UP TO AN ADDITIONAL \$1B, IF APPROVED IN OTHER CURRENT PITOLISANT LIFECYCLE MANAGEMENT PROGRAMS

WAKIX NET REVENUE (\$M)

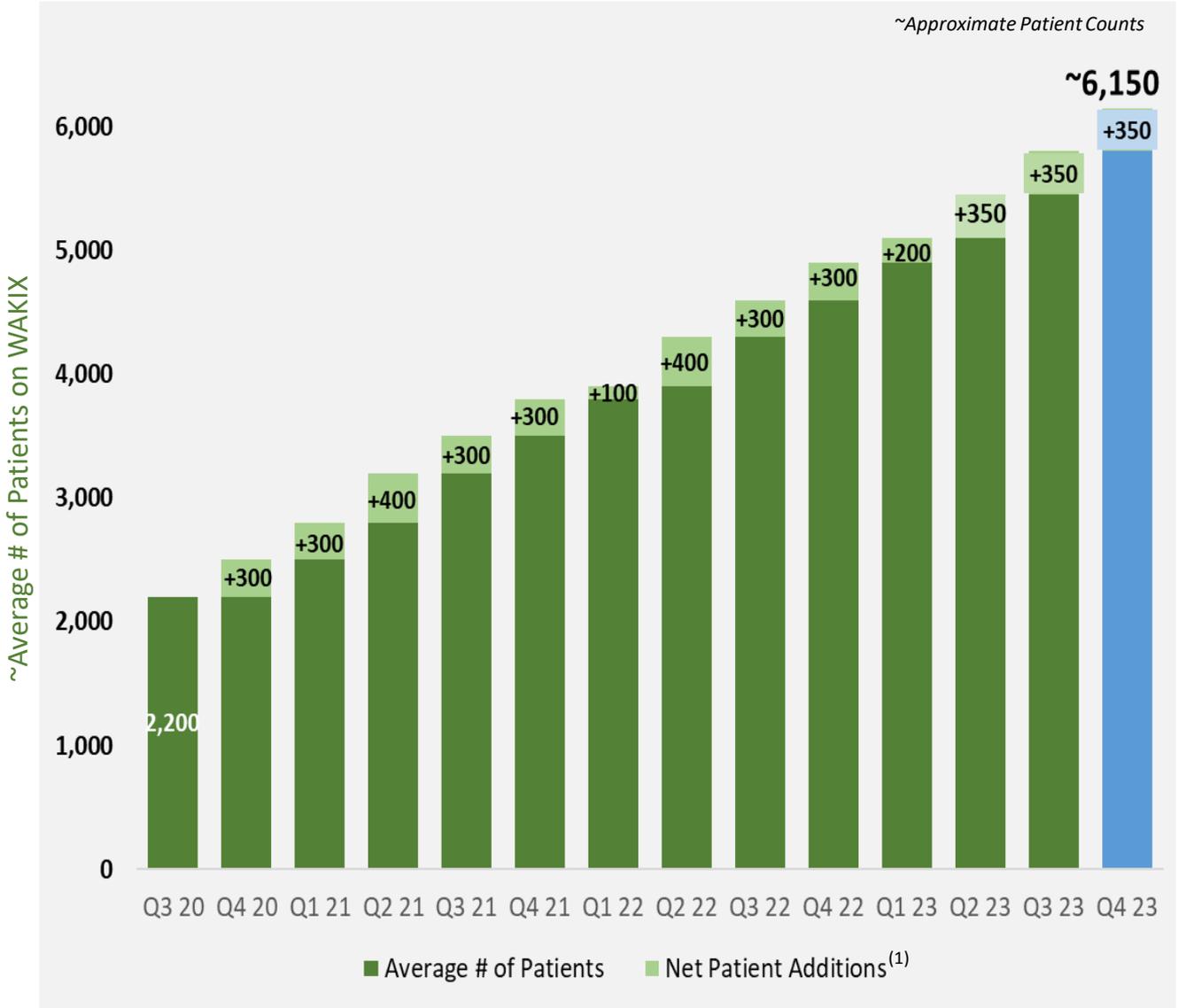


HIGHLIGHTS

- Q4 23 represented the highest quarter of Net Revenue in our history (\$168.4M)
- FY 23 Net Revenue of \$582.0M
- Underlying demand drove continued double-digit revenue growth
 - Strong patient interest
 - Continue to add new prescribers and grow WAKIX prescriber base

Solid Business Fundamentals Driving Growth

Continued Strong Performance in 2023 - Year 4 of Commercialization



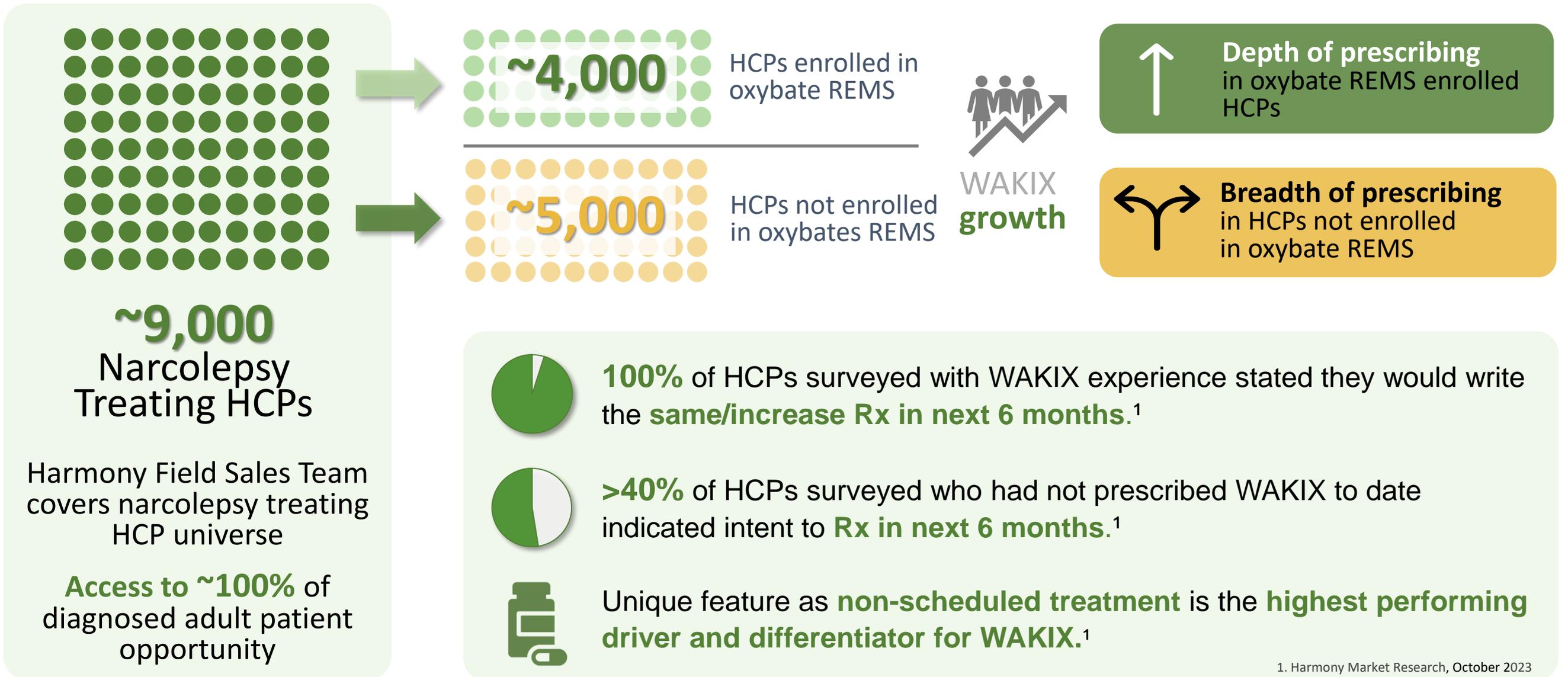
2023 Highlights



More unique prescribers of WAKIX than sodium oxybate
Strong market access coverage (~84%) - even with the launch of generic and new oxybate options

(1) Net Patient Additions based on previously disclosed quarterly average number of patients on WAKIX

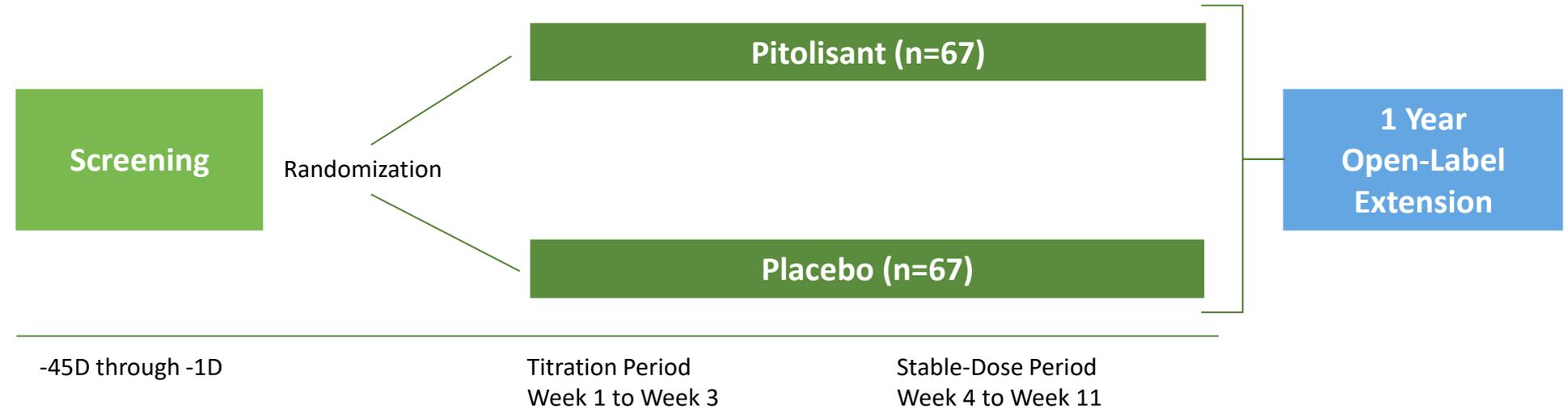
Prescriber Dynamics Support Continued WAKIX® Growth in Adult Narcolepsy



Development Pipeline: Continues to Grow

Product / Indication	Pre-IND	Phase 1	Phase 2	Phase 3	Regulatory Filing	Marketed Product	Milestone
WAKIX®							
EDS in Narcolepsy (Adults)							
Cataplexy in Narcolepsy (Adults)							
Pitolisant							
Pediatric Narcolepsy ⁽¹⁾							PDUFA Date June 21, 2024
Idiopathic Hypersomnia (IH)							FDA Meeting March 2024
Prader-Willi Syndrome (PWS)							Initiate Ph3 Trial 1Q2024
Myotonic Dystrophy (DM)							Positive Topline Data 4Q2023
Next Gen Pitolisant Formulations							PK Data 1H2024
ZYN002 (Cannabidiol Gel)							
Fragile X Syndrome (FXS)							Topline Data Mid-2025
22q11.2 Deletion Syndrome (22q)							Ph 3 Prep Ongoing
HBS-102							
PWS							Preclinical POC Data 1H2024

TEMPO: Global Phase 3 Trial of Pitolisant in PWS



Trial Design:

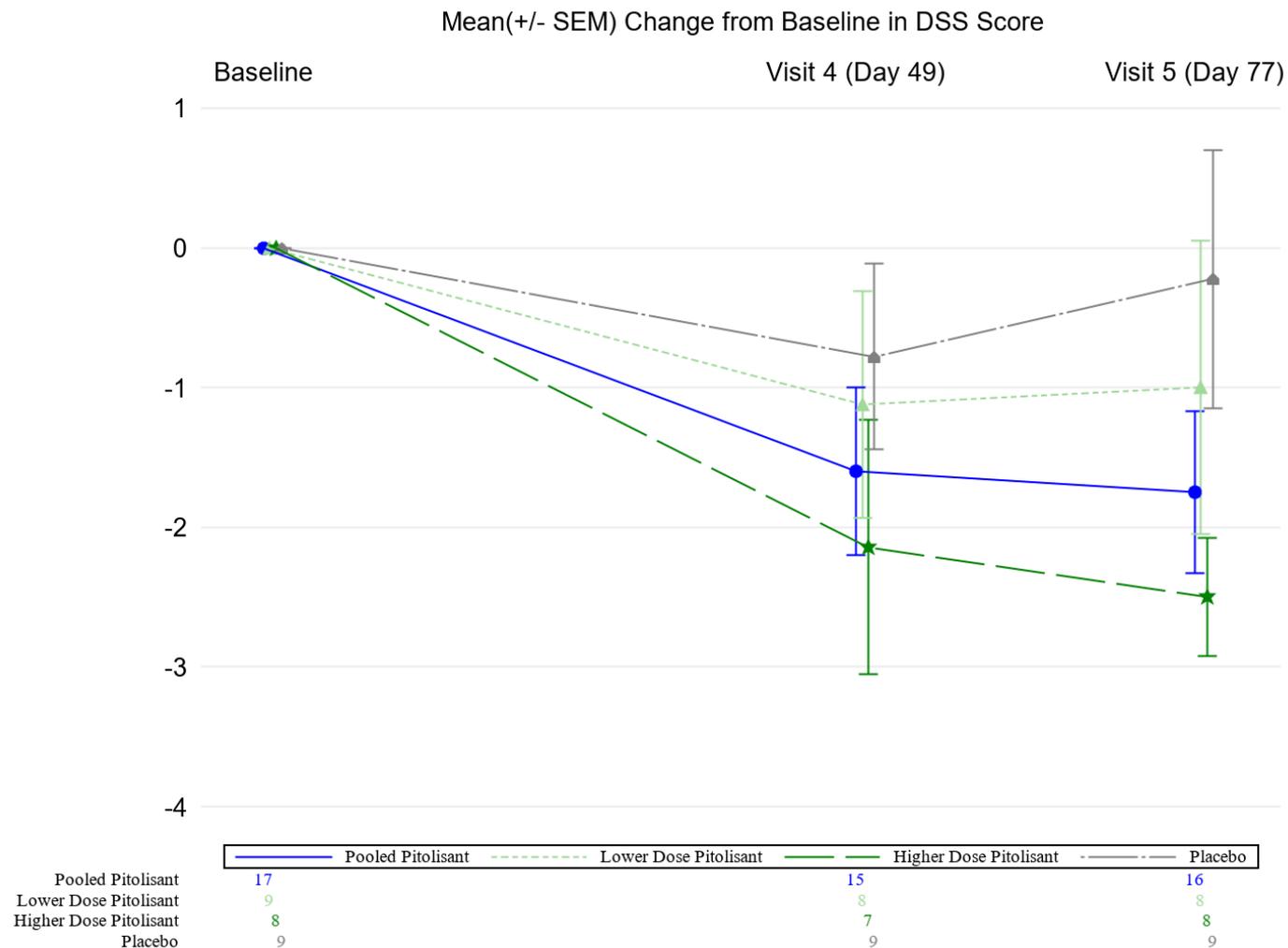
- Randomized, double-blind, placebo-controlled, parallel-group study
- 1:1 pitolisant : placebo
- 134 patients; ages 6 and older

Objectives / Endpoints:

- **Primary objective:** to evaluate the efficacy of pitolisant on EDS in patients with PWS
- **Primary endpoint:** change in severity of EDS as measured by PROMIS-SRI T-score from Baseline to the end of the Double-Blind Treatment Period (Day 77)
- **Secondary objectives:** to evaluate the efficacy of pitolisant on irritability, hyperphagia and behavioral problems in PWS
- **Secondary endpoints:** ABC-C Irritability domain, HQ-CT, ABC-C Other domains

DM1 Phase 2 POC Study Topline Data

Change in Daytime Sleepiness Scale (DSS) from Baseline to End of Treatment Period



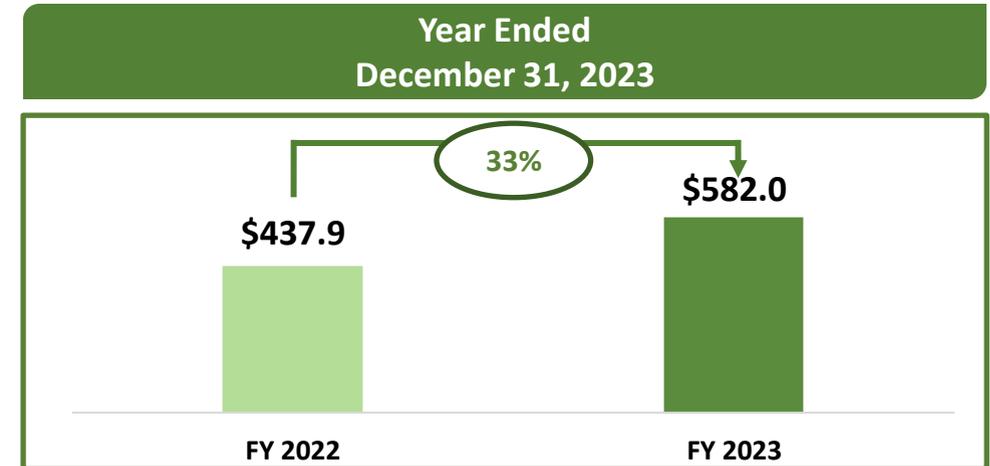
Topline Data Highlights

- **Clinically meaningful signal in EDS (DSS, ESS and CGI-S)**
- **Clinically meaningful signal in Fatigue (FSS)**
 - Mean change from baseline of -0.86 and -0.36 for high-dose and low-dose pitolisant, respectively, compared to -0.13 for placebo
- **A clear and consistent dose-response** was demonstrated across the efficacy outcomes
- **Well tolerated** with an overall safety/tolerability profile consistent with the known profile of pitolisant
- **Next Steps:** Evaluate full data set and assess opportunity. Potentially pivot to next-gen formulations of pitolisant to advance program

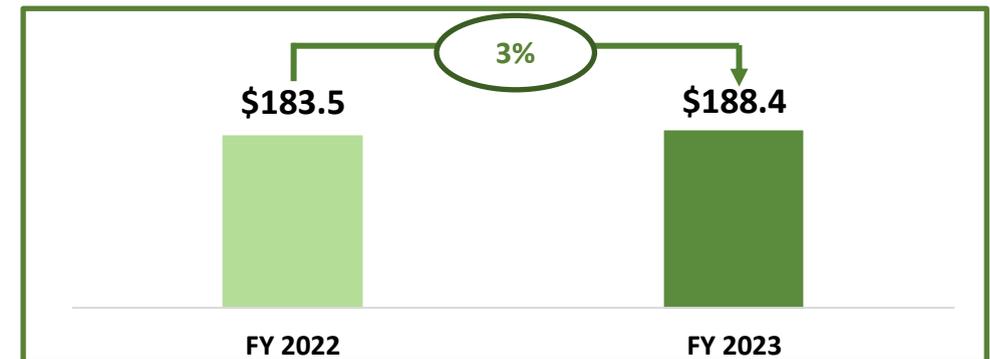
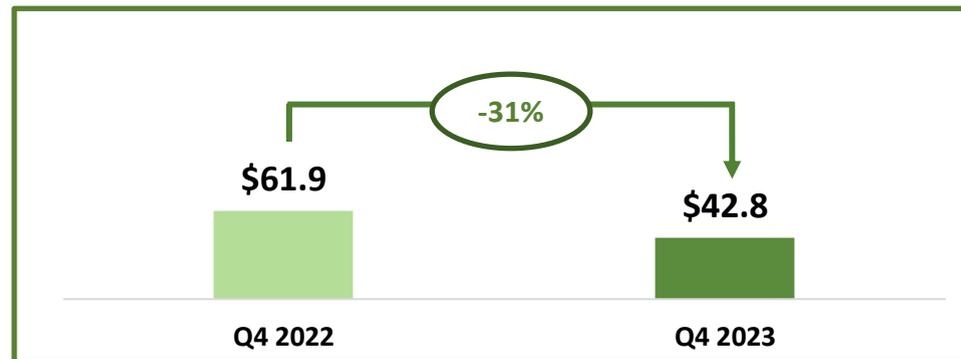
Financial Highlights

(In millions, USD)

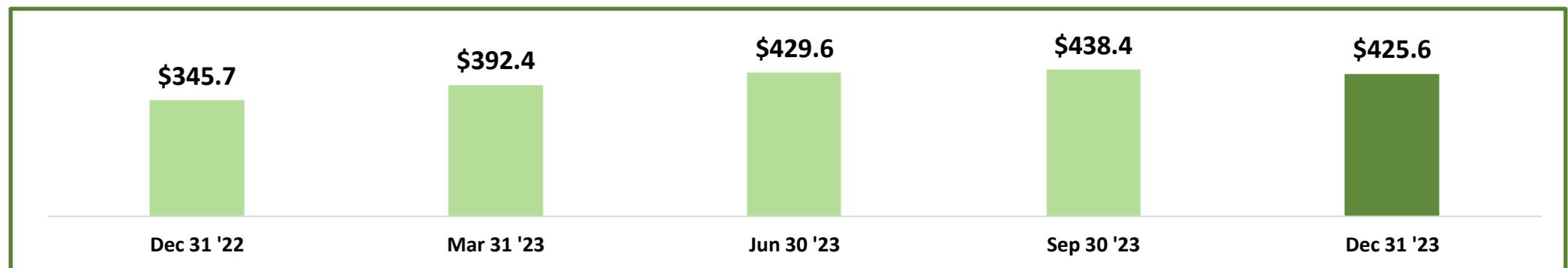
Net Product Revenue



Non-GAAP Adjusted Net Income⁽¹⁾



Cash, Cash Equivalents & Investment Securities



(1) Non-GAAP Adjusted Net Income= GAAP Net Income excluding non-cash interest expense, depreciation, amortization, stock-based compensation, other non-operating items and tax effect of these items

Financial Summary

<i>(In millions, USD)</i>	Three Months Ended December 31,		% Change	Year Ended December 31,		% Change
	2023	2022		2023	2022	
Totals may not foot due to rounding						
Net Product Revenue	\$168.4	\$128.3	31%	\$582.0	\$437.9	33%
Cost of Product Sold	43.2	26.9	61%	121.2	83.5	45%
Total Operating Expenses	\$85.1	\$53.8	58%	\$268.8	\$234.2	15%
R&D Expense ⁽¹⁾	30.3	10.1	NM	76.1	70.9	7%
S&M Expense	26.9	21.1	28%	97.4	79.3	23%
G&A Expense ⁽²⁾	27.9	22.6	23%	95.3	84.0	13%
Net Income	\$26.6	\$48.5	(45%)	\$128.9	\$181.5	(29%)
Cash, cash equivalents & investment securities				\$425.6	\$345.7	23%

NM denotes not meaningful % change

(1) Includes one-time Zynerva transaction related costs of \$6.0M for the three months and year ended December 31, 2023

(2) Includes one-time Zynerva transaction related costs of \$3.8M for the three months and year ended December 31, 2023

GAAP vs NON-GAAP Reconciliation

<i>(In millions, USD)</i>	Three Months Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
Totals may not foot due to rounding				
GAAP net income	\$26.6	\$48.5	\$128.8	\$181.5
Non-cash interest expense ⁽¹⁾	0.2	0.4	3.2	1.7
Depreciation	0.2	0.1	0.5	0.4
Amortization ⁽²⁾	6.0	6.0	23.8	23.0
Stock-based compensation expense	8.9	7.7	31.2	26.9
Transaction related costs ⁽³⁾	9.8	-	9.8	-
Loss on debt extinguishment	-	-	9.8	-
Licensing fees and milestone payments ⁽⁴⁾	-	-	0.8	30.0
Valuation allowance release	-	-	-	(74.5)
Income tax effect related to Non-GAAP adjustments ⁽⁵⁾	(8.8)	(0.7)	(19.6)	(5.4)
Non-GAAP adjusted net income	\$42.8	\$61.9	\$188.4	\$183.5
GAAP net income per diluted share	\$0.45	\$0.79	\$2.13	\$2.97
Non-GAAP adjusted net income per diluted share	\$0.73	\$1.01	\$3.12	\$3.00
Weighted average number of shares of common stock used in non-GAAP diluted per share	58,853,292	61,620,712	60,372,397	61,097,045

(1) Includes amortization of deferred finance charges.

(2) Includes amortization of intangible asset related to WAKIX.

(3) Includes costs associated with the acquisition of Zynerva in October 2023. There were \$2.3M of IPR&D charges and \$3.7M of severance recorded in research and development expenses and \$3.8M of severance recorded in general and administrative expenses.

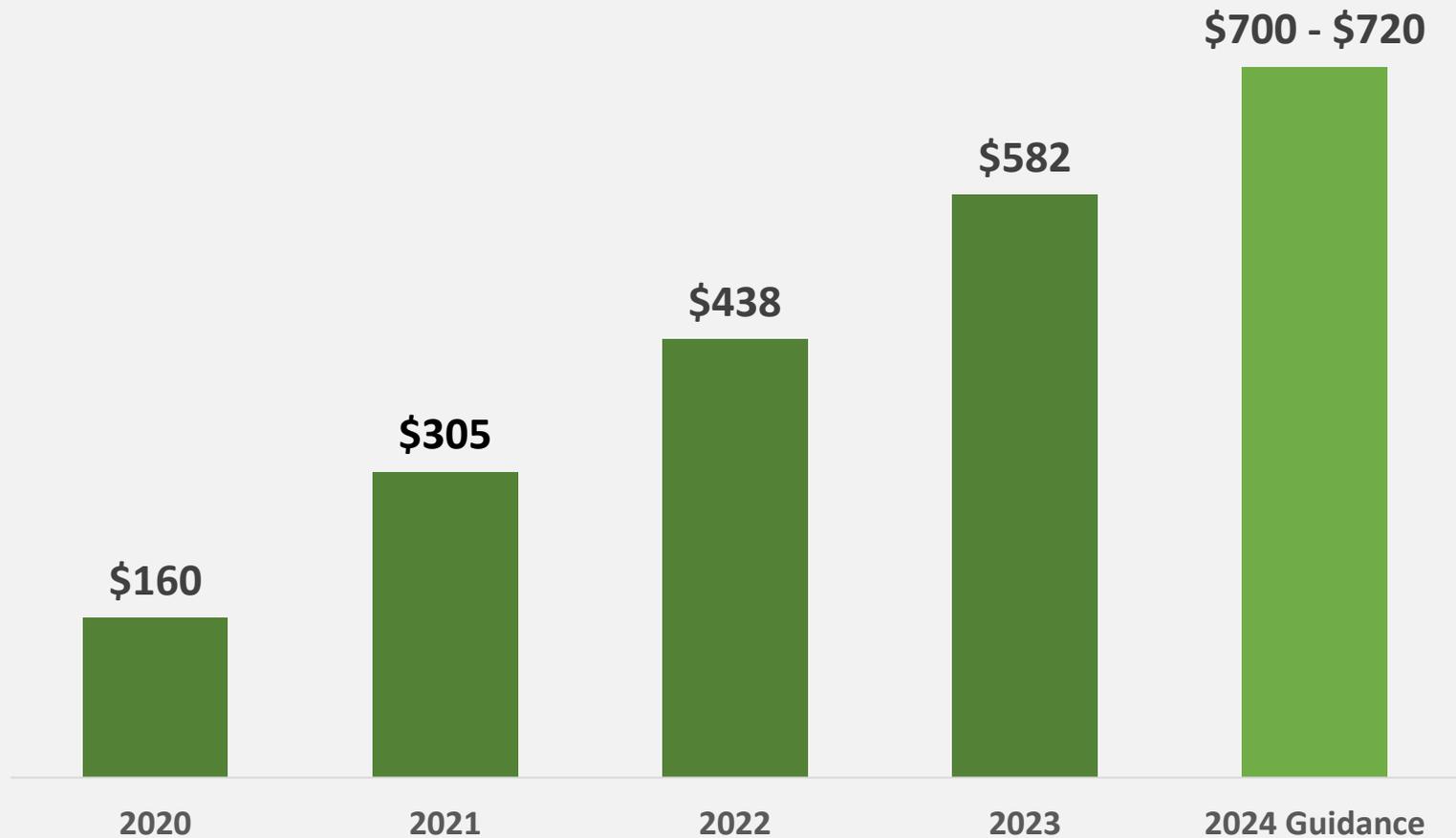
(4) Includes a \$0.8M milestone payment related to HBS-102 preclinical milestone in March 2023 and \$30M licensing fee incurred upon closing the 2022 Licensing and Commercialization Agreement with Bioprojet in August 2022.

(5) Calculated using the reported effective tax rate for the periods presented less impact of valuation allowance release and discrete items.

2024 Net Revenue Guidance

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WAKIX ANNUAL NET REVENUE (\$M)



\$582M

2023
Net Revenue

33% Growth vs. 2022

\$700-\$720M

2024
Guidance



Thank You

