

41st Annual J.P. Morgan Healthcare Conference

January 2023



Forward Looking Statements

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HARMONY BIOSCIENCES

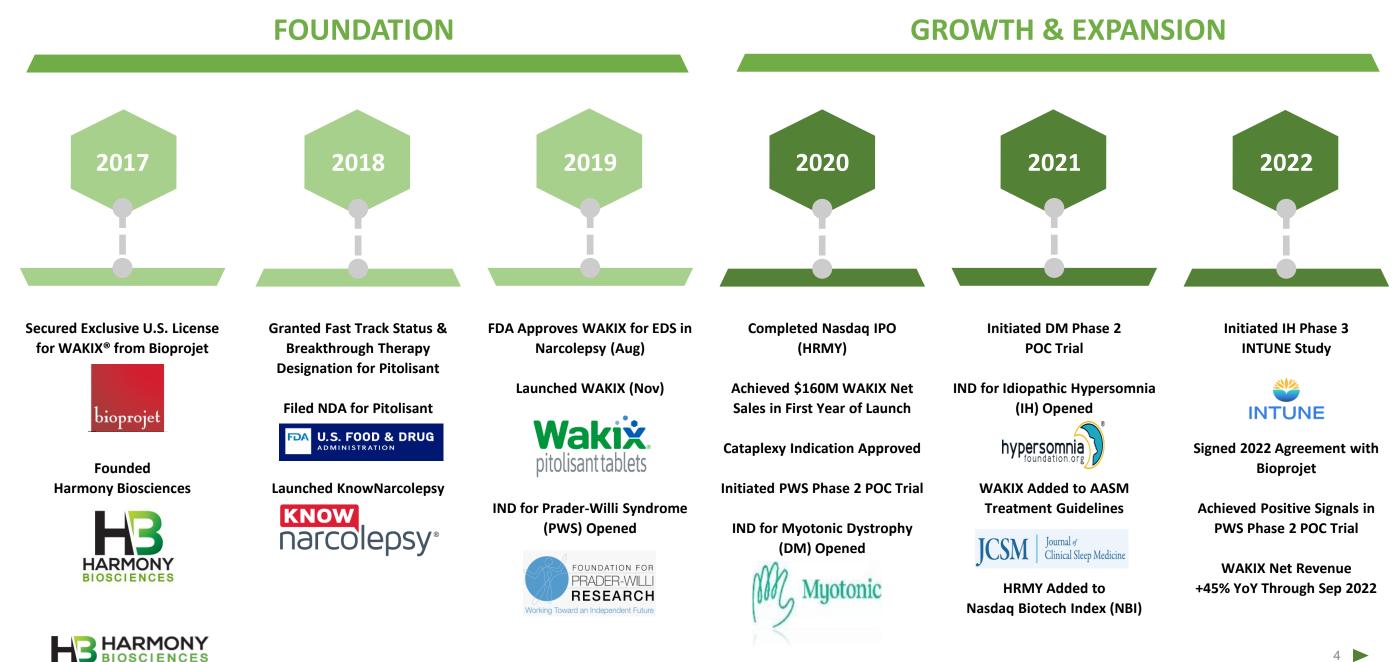
OUR MISSION

At Harmony Biosciences, we specialize in developing and delivering treatments for rare neurological diseases that others often overlook. We believe that where empathy and innovation meet, a better life can begin for people living with neurological diseases.





Our Journey



Harmony Today A Rapidly Growing, Emerging Leader in the Neurology Arena



Strong Launch Success With WAKIX[®] in Narcolepsy

>\$1B+ Net Revenue Opportunity Via Narcolepsy and Additional Indications Established Infrastructure and Proven Capabilities Including Clinical Development, Regulatory Affairs, and Commercial





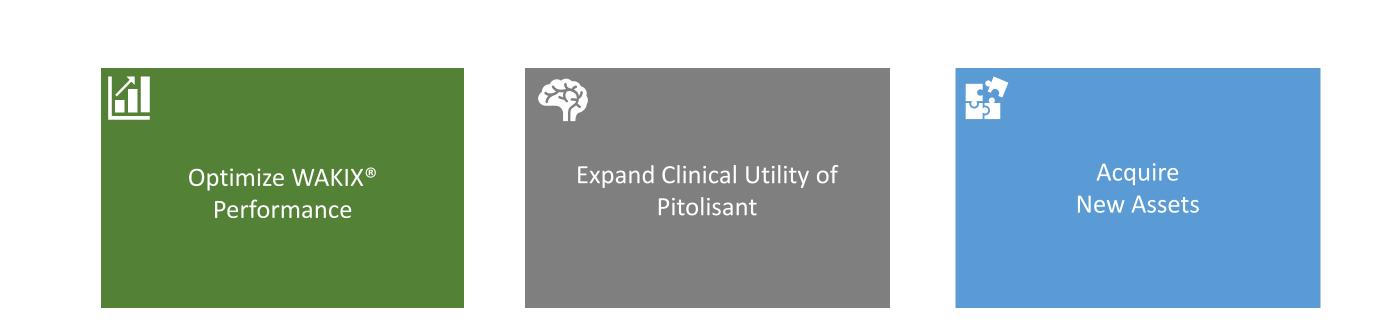
Multiple Phase 2/3 Programs Underway to Expand Utility of Pitolisant Beyond Narcolepsy

Award Winning Team and Culture With Deep Expertise in Rare-Orphan Neurology





Our Three-Pillar Growth Strategy



DESIGNED TO SUPPORT LONG TERM SUSTAINABLE GROWTH



Our Three-Pillar Growth Strategy 2022 Accomplishments

Expand Clinical Vility of	beyond Narcolepsy
	hase 3 INTUNE Study in patients with IH positive signals in Phase 2 POC study in patients with PWS
Acquire New Assets Signed new	ortfolio beyond WAKIX w agreement with Bioprojet for up to two new pitolisant based assets ancial position

Our Three-Pillar Growth Strategy A Look Forward to 2023

HARMONY BIOSCIENCES

Optimize WAKIX [®] Performance	 Continue strong commercial execution for WAKIX Continue growing average number of patients on WAKIX Expect to surpass \$1B in cumulative net revenue since launch
Expand Clinical Utility of Pitolisant	 Continue patient enrollment in Phase 3 INTUNE registrational trial Engage with FDA on next steps to advance the PWS development program Anticipate topline data from Phase 2 POC study in DM1
Acquire New Assets	 Acquire assets to expand portfolio beyond WAKIX Focused on rare/orphan neurology assets and/or assets in other neurological diseases Continue strengthening our financial position

Our Three-Pillar Growth Strategy



DESIGNED TO SUPPORT LONG TERM SUSTAINABLE GROWTH

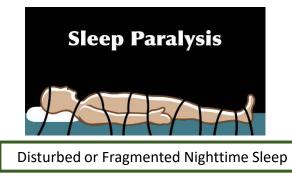


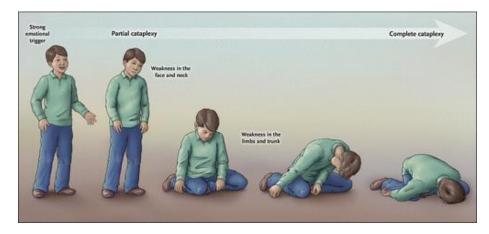
Narcolepsy: A Chronic Debilitating Neurological Disease



- Orphan/rare disease thought to affect up to 165,000 Americans¹
- All patients with narcolepsy have excessive daytime sleepiness (EDS), which is the primary symptom of the disorder
- Type 1 (narcolepsy with cataplexy) and Type 2 (narcolepsy without cataplexy) differentiated by cataplexy as an additional symptom beyond EDS







Hypnagogic and Hypnopompic Hallucinations



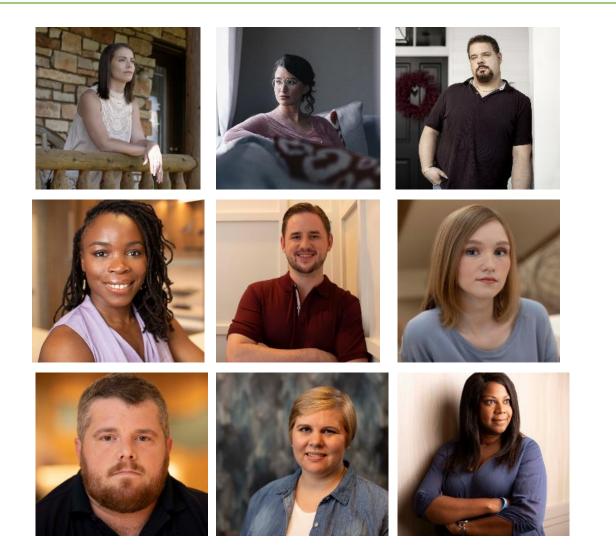
"These hallucinations can be very vivid, and sometimes disturbing. Especially if you see creatures appearing in your room, voices saying unpleasant things or feeling something touch you."

86% of people living with narcolepsy surveyed reported narcolepsy is a life-changing disorder³



Sources: 1. National Institutes of Health. 2017; https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Narcolepsy-Fact-Sheet#3201_Accessed May 2018; 2. Scammel, Thomas. *Narcolepsy.* The New England Journal of Medicine 2015 3. Versta Research, Know Narcolepsy Survey ("Know Narcolepsy"), October 2018 (a three-part survey conducted by Versta Research on behalf of Harmony Biosciences, LLC, 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)

Patient Perspectives: The Impact of Living with Narcolepsy



"Narcolepsy severely impacts not only me, but my family and friends. It's a daily battle fought over a lifetime."

"My biggest accomplishment every day is getting out of bed."

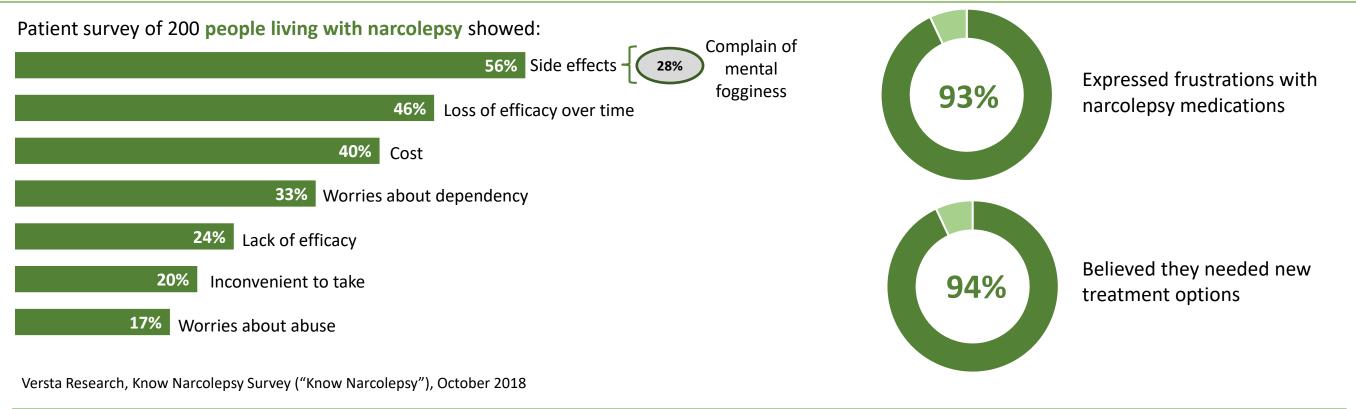
"Narcolepsy is an ugly life stealing disease – it makes you feel useless and labeled as lazy and unmotivated. I couldn't hold a job or drive a car for years."

"I lost my best friend, my spouse and my daughter for a number of years because I couldn't function. I'll never get that time back with them."

- Excerpts from patient stories on <u>www.KnowNarcolepsy.com</u>
- 10x Award winning disease education website designed to advance the understanding of the disorder and the impact of narcolepsy



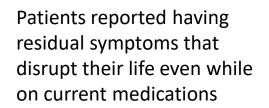
Market Research Supports the Need for Novel Treatment Options for People Living with Narcolepsy



A retrospective, electronic chart review of 97 treated narcolepsy patients conducted at Rush University Medical Center found the majority of patients reported unresolved symptoms even while on treatment

McCullough et al. Novel treatments options in narcolepsy, Chicago Rush Memorial Center - SLEEP 2019 Abstract





75%

Optimize WAKIX Performance

WAKIX[®] – Unique & Meaningfully Differentiated Product Profile



- First in class molecule with a novel MOA
 - The only selective H3 receptor antagonist/inverse agonist approved by the FDA
- First and only FDA-approved non-scheduled treatment for narcolepsy
- **Not a stimulant** no evidence of drug tolerance or withdrawal symptoms
- Can be used as monotherapy or administered concomitantly with other narcolepsy treatments (modafinil and sodium oxybate)
- Once-daily oral tablet administered in the morning upon wakening

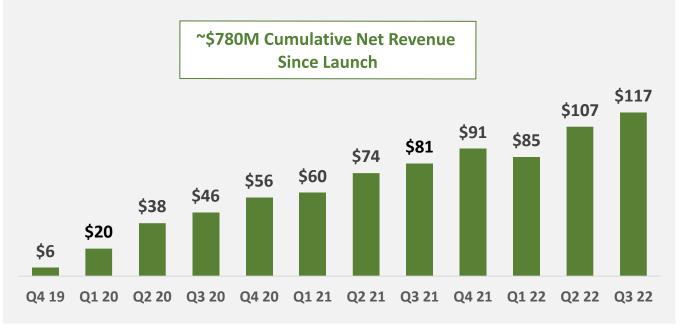


Optimize WAKIX Performance

Strong Track Record of Performance

CONFIDENT IN WAKIX BECOMING A POTENTIAL >\$1B+ FRANCHISE VIA NARCOLEPSY AND ADDITIONAL INDICATIONS

WAKIX QUARTERLY NET REVENUE (\$M)



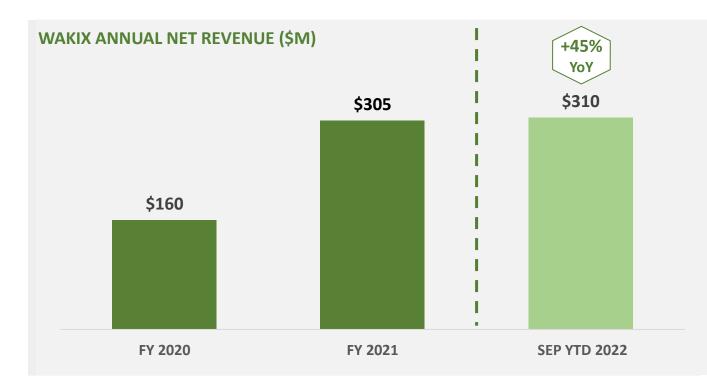
~4,600¹ Average # of WAKIX Patients



& Breadth of Prescriber Base

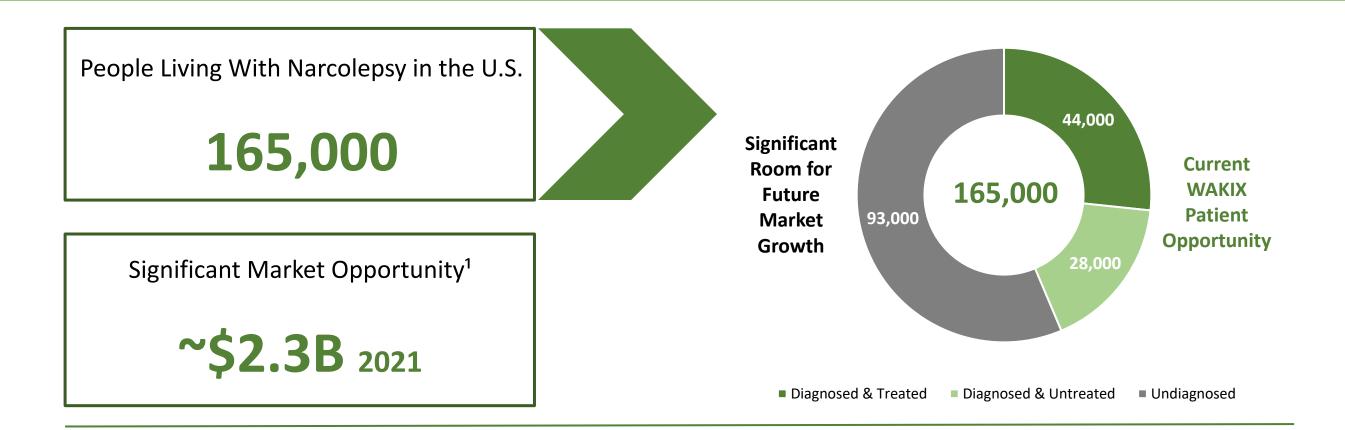


U.S. Covered Lives With Formulary Access





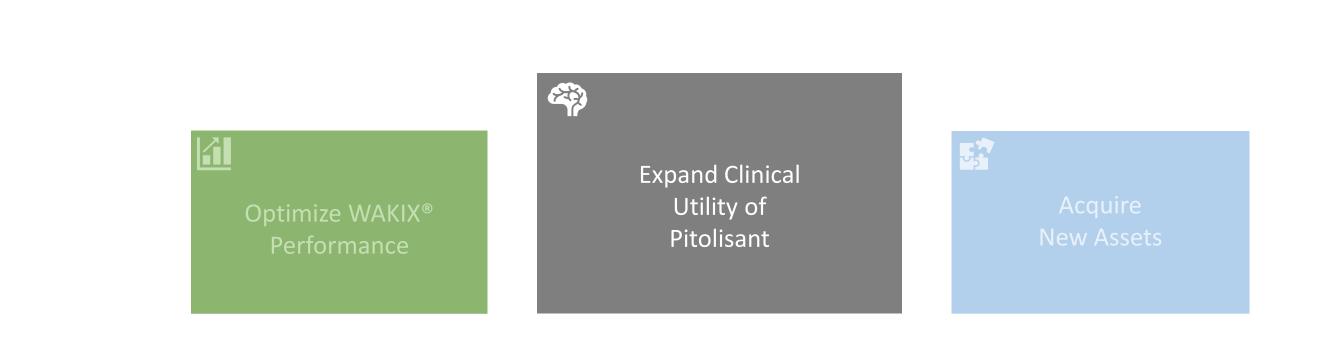
Narcolepsy: Significant Market Opportunity



Factors contributing to market growth

- Growth in diagnosis rates in recent years
- Increased investment in education
- Introduction of new treatments
- Low satisfaction with traditional treatment options

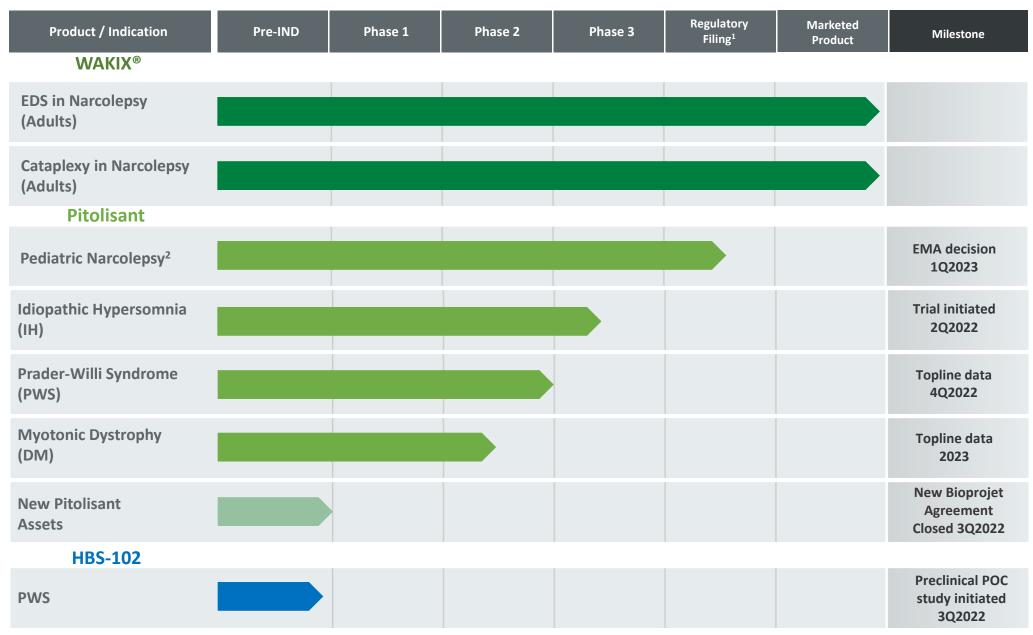
Our Three-Pillar Growth Strategy



DESIGNED TO SUPPORT LONG TERM SUSTAINABLE GROWTH



Development Pipeline





HARMONY 1.
 Includes New Drug Applications and supplemental New Drug Applications.

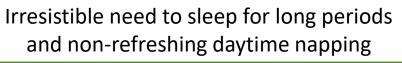
 2.
 Trial conducted by Bioprojet and Bioprojet submitted regulatory package to EMA.

Pitolisant: Key Clinical Programs

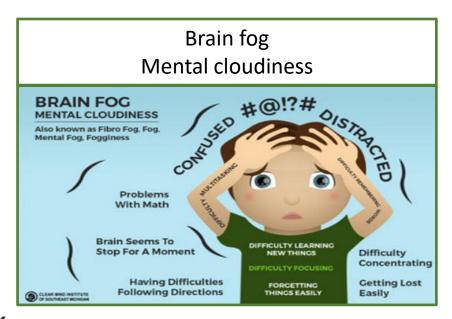
Idiopathic Hypersomnia (IH)	 Rare, chronic, neurological disease characterized by EDS despite sufficient or long sleep; other key features include sleep inertia after waking and impaired cognition, attention, and alertness (known as 'brain fog')¹⁻⁵ Like narcolepsy, another central disorder of hypersomnolence Estimated number of diagnosed patients in U.S. currently about 30,000 to 40,000⁶; epidemiological data suggest about 80,000 people living with IH in U.S. Only one approved treatment for patients with IH
	 Prader-Willi Syndrome (PWS) Rare, genetic multi-system, neurodevelopmental disorder characterized by hypothalamic dysfunction Decreased hypocretin levels in some patients^{7,8} ~15,000-20,000 patients in U.S. and more than 50% have EDS⁷ No approved treatments for EDS in patients with PWS; unmet medical need
Myotonic Dystrophy (DM)	 Rare, genetic multi-system disease; myotonia and progressive muscle weakness hallmark symptoms; EDS most common non-muscular symptom (~80% - 90% of patients) Decreased hypocretin levels in some patients^{9,10,12} ~160,000 people in the US with genetic defect for DM1; of those, ~50% symptomatic and of those, ~50% diagnosed (~40,000 patients)¹³ No approved treatments for DM; significant unmet medical need
	Sources: 1. AASM, 2014; 2. Sateia, Chest, 2014; 3. Arnulf et al., Sleep Med Clin, 2019; 4. Trotti, Sleep Med Clin, 2017; 5. Trotti et al., Sleep Med, 2020; 6. Acquavella et al., J Clin Sleep Med Sleep Med, 2020; 7. Camfferman, Sleep Medicine Reviews, 2008; 8. Omokawa et al, American Journal of Medical Genetics, 2015; 9. Dauvilliers et al, Sleep Medicine Reviews, 2012; 10.

Med, 2020; 7. Camfferman, Sleep Medicine Reviews, 2008; 8. Omokawa et al, American Journal of Medical Genetics, 2015; 9. Dauvilliers et al, Sleep Medicine Reviews, 2012; 10. Hagerman et al, Muscle Nerve, 2019; 11. Heatwole et al. Muscle Nerve, 2016; 12. Martinez-Rodriguez et al. Sleep; 2003; 13. https://www.myotonic.org/; accessed June 2020.

Idiopathic Hypersomnia: A Debilitating Neurological Disorder With Significant Unmet Need







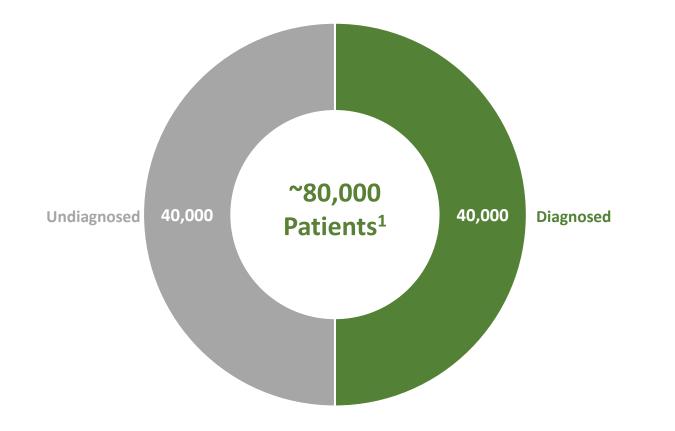






Idiopathic Hypersomnia: Large Market With Significant Unmet Need

Market Opportunity

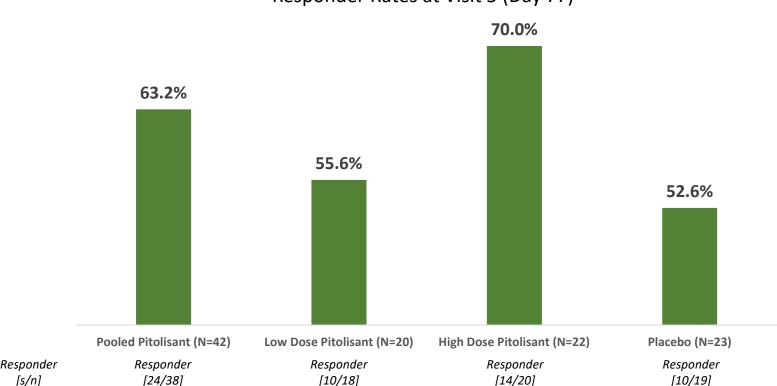


- Significant unmet need
- Only one FDA approved treatment for IH
- Amphetamine, methylphenidate, modafinil and armodafinil, are used off-label for treatment
 - Efficacy of these treatments for the symptoms of IH has not been established
 - Most patients still suffer with residual symptoms
- Pitolisant being investigated in IH with a unique MOA, working through histamine to improve wakefulness
- If approved, opportunity for significant synergy with existing commercial infrastructure



PWS Phase 2 POC Study Topline Data: Responder Analysis

Higher Responder Rate for Pitolisant vs. Placebo: Driven by High Dose Group



Responder Rates at Visit 5 (Day 77)

Topline Data Highlights

- Clinically meaningful reduction in the ESS-CHAD Parent/Caregiver scores in all age groups and across both low-dose and high-dose treatment groups
- Higher responder rate for Pitolisant vs Placebo
- Evidence of a dose-response, favoring high dose group
- Well tolerated with an overall safety/tolerability profile consistent with the known profile of pitolisant

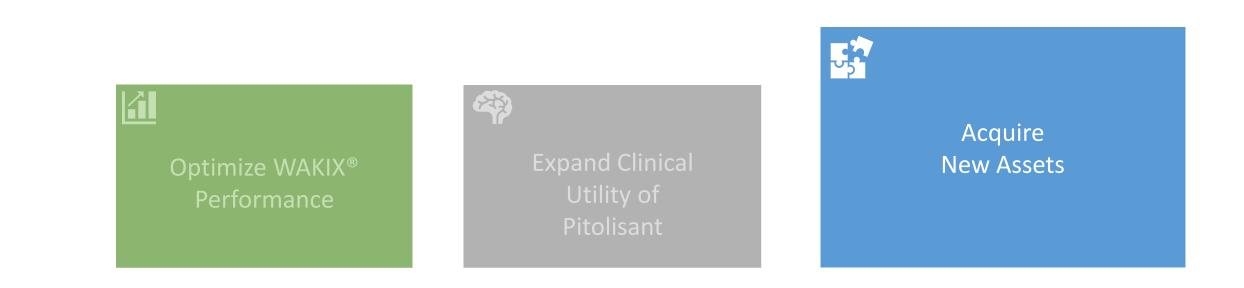
s = Number of responders

n = Number of subjects with baseline assessment and post-baseline assessment at the visit

A responder was defined as a subject with an improvement of \geq 3 points from Baseline or a score \leq 10 at EOT on the ESS-CHAD



Our Three-Pillar Growth Strategy



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Business Development



- Intend to acquire a portfolio of rare/orphan neurology assets and/or assets in other neurological diseases
 - Leverage existing expertise and infrastructure
 - Potential synergies with WAKIX and our current footprint
- Focus across development stages, both early and later stage assets
- Dedicated BD team and internal capabilities across clinical development, regulatory affairs and commercial launch and execution



Financial Flexibility

- Strong financial position
- \$316M in cash, cash equivalents and investments as of September 30, 2022
- Additional \$100M under Blackstone delayed draw debt facility
- Profitable and cash generating
- Access to capital markets

Evolve into a leading pharmaceutical company focused on developing and commercializing innovative therapies for patients living with rare neurological diseases who have unmet medical needs



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Thank You

