



Harmony Biosciences Q4 & FY 2020 Financial and Business Update

March 25, 2021



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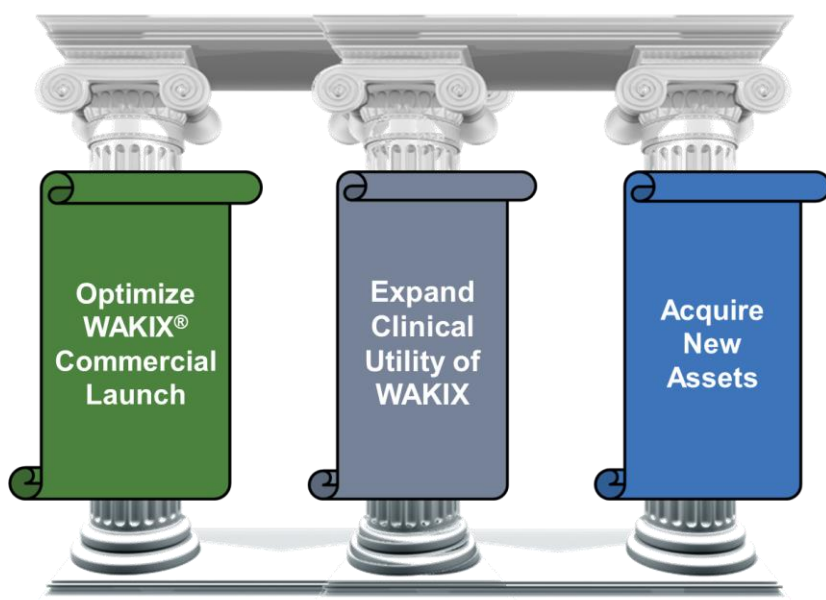
Corporate Highlights

- Commercial-stage US Pharma company focused on treatments for patients living with rare, neurological disorders who have unmet medical needs
- Opportunity to expand existing \$2B narcolepsy market with WAKIX® (pitolisant)
 - First-in-class molecule with a novel mechanism of action (MOA)
 - Approved for treatment of EDS and cataplexy in adult patients with narcolepsy
 - Only FDA-approved non-scheduled treatment option for narcolepsy
 - Differentiated product profile
 - Convenient, once-daily dosing
 - \$160M net revenues in 2020
- WAKIX Life Cycle Management opportunities
 - *Portfolio-in-a-product* opportunity with pitolisant
 - Novel MOA supports mechanism-based approach to LCM drug development
 - New indications being pursued in additional rare neurological disease patient populations
- Strong financial position



2020 Achievements on our Growth Strategy

Harmony's Three Pillars of Growth Strategy



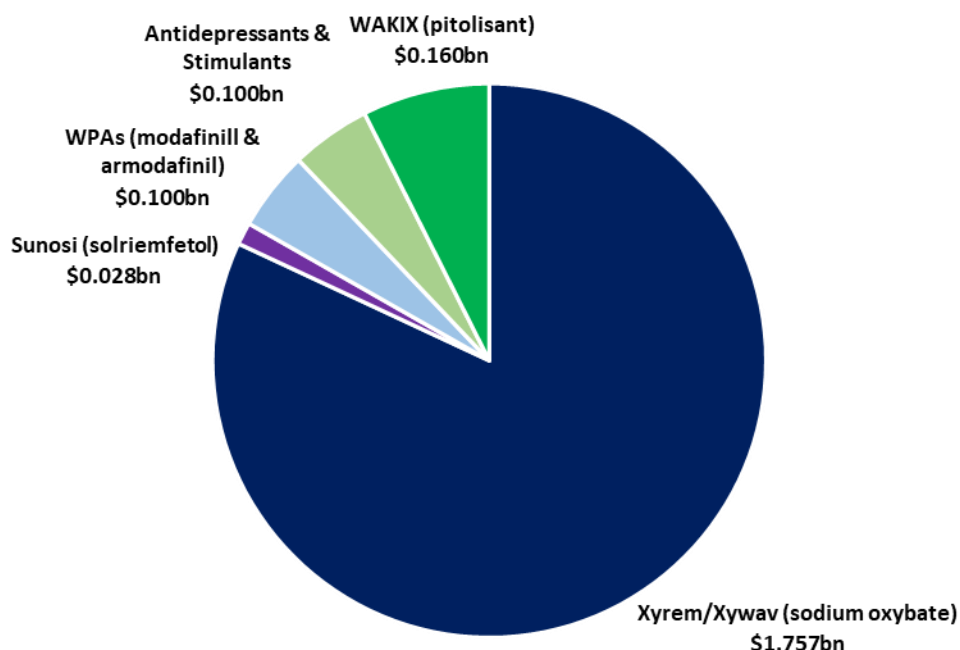
2020 Achievements

- ☑ Raised \$147M in IPO
- ☑ Added to Russell 2000 and 3000 Indices
- ☑ WAKIX generated \$160M in first full year of sales
- ☑ Received FDA approval for cataplexy indication
- ☑ Enrolled first patient in Phase 2 trial in patients with Prader-Willi Syndrome
- ☑ Submitted IND for Myotonic Dystrophy development program
- ☑ Data presentations at medical conferences

Significant Adult Narcolepsy Market Value Opportunity

~\$2.1B

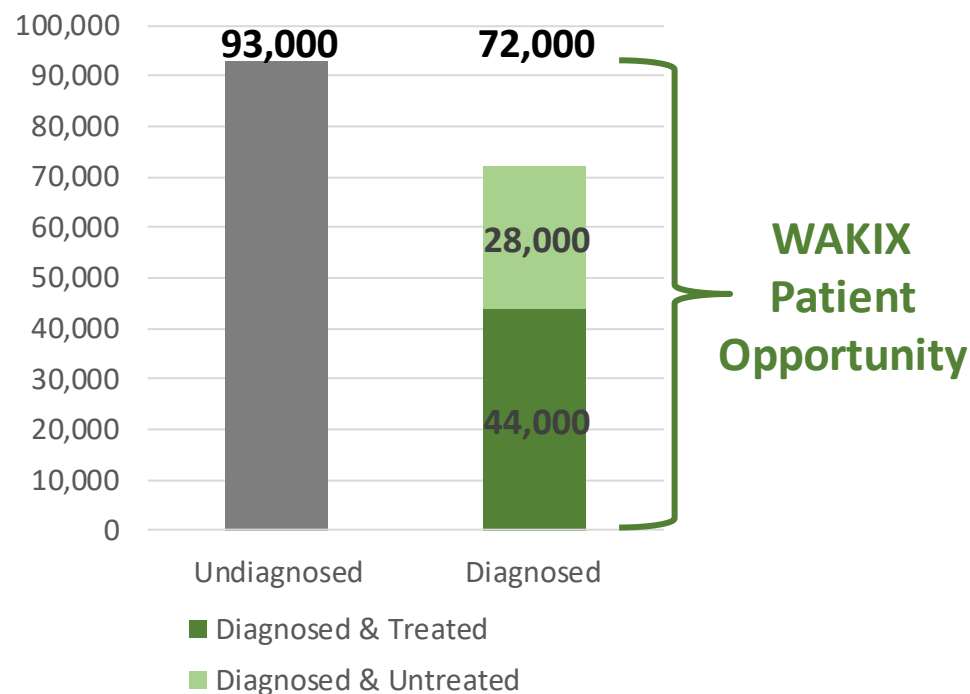
Net Sales



U.S. Narcolepsy Market (2020)

165,000

People Living With Narcolepsy in the U.S.

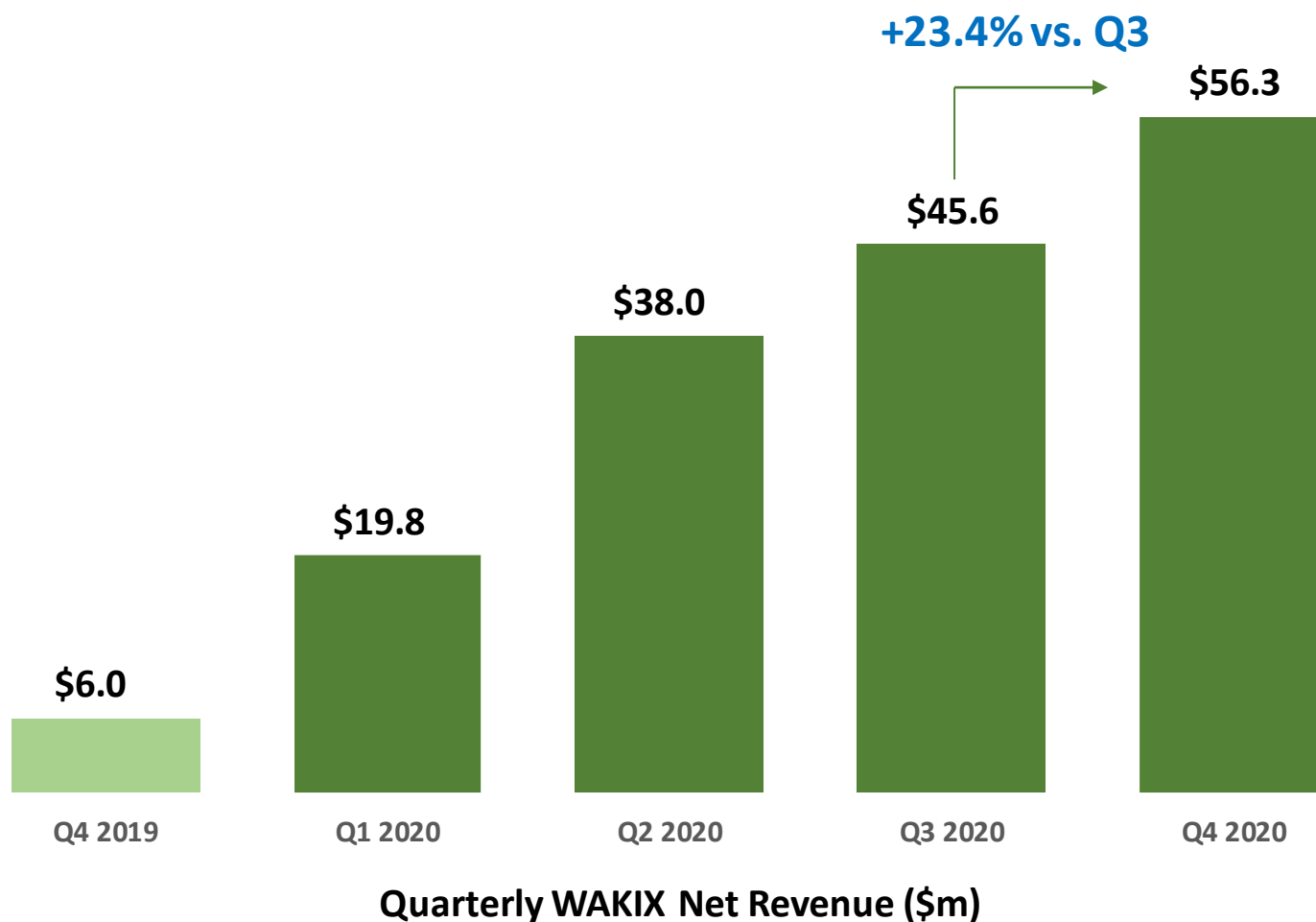


Factors contributing to market growth

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

2020 WAKIX Revenue Performance

Continued Growth with Q4 Revenue of \$56.3M



Driving Growth Through Our Launch For WAKIX

Q4 2020 Performance



Patient Outreach
Programs & Support

~2,500
Average # of
WAKIX Patients



Expanded Virtual
Engagement Programs



Healthcare Professional
Educational Initiatives

>2,400 Unique HCP Prescribers
Since Launch



Managed Care
Educational & Outreach

~80% U.S. Covered Lives With Formulary Access

Multiple Opportunities for Pitolisant

Indication	Pre-IND	Phase 1	Phase 2	Phase 3	Regulatory Filing ¹	Marketed Product	Upcoming Milestones
APPROVED INDICATIONS							
EDS in Adult Patients with Narcolepsy							
Cataplexy in Adult Patients with Narcolepsy							
LABEL EXPANSION IN NARCOLEPSY							
Pediatric Narcolepsy ²							Initiation of Phase 3 trial 2H2021; top line data 1H2023
NEW INDICATIONS							
Prader-Willi Syndrome (PWS)							Top line data 1H2022
Myotonic Dystrophy							Initiation of Phase 2 trial 1H2021; top line data 2H2022

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

2. Current trial being conducted by Bioprojet. We plan to initiate a Phase 3 clinical trial in 2H2021 in pursuit of pediatric indications for both EDS and cataplexy as well as pediatric exclusivity.

Prader-Willi Syndrome (PWS)



Rare, genetic multi-system disease characterized by hypothalamic dysfunction; decreased hypocretin levels in some patients^{1,2}



~15,000-20,000 patients in U.S. and more than 50% have Excessive Daytime Sleepiness (EDS) due to sleep-wake state instability of central origin and other factors¹



Other symptoms include behavioral issues and cognitive impairment which could be related to, or exacerbated by, EDS



No approved treatments for EDS in patients with PWS and significant unmet medical need



Phase 2 clinical trial initiated in 2020; top-line results anticipated in 1H 2022

Rationale for Pitolisant in Patients with PWS



A disorder of hypothalamic dysfunction^{1,2}

- *Abnormalities have been found in the hypothalamus in patients with PWS*
- *Many of the features of PWS are the result of hypothalamic dysfunction (growth hormone deficiency, hypogonadism, hyperphagia, sleep-wake abnormalities)*



The hypothalamus regulates sleep-wake state stability via hypocretin and histamine^{3,4,5}

- *Decreased levels of hypocretin have been found in patients with PWS^{6,7}*
- *Mouse models of PWS (MAGEL2 & SNORD116) have demonstrated impaired hypocretin systems and sleep-wake state instability^{8,9,10}*



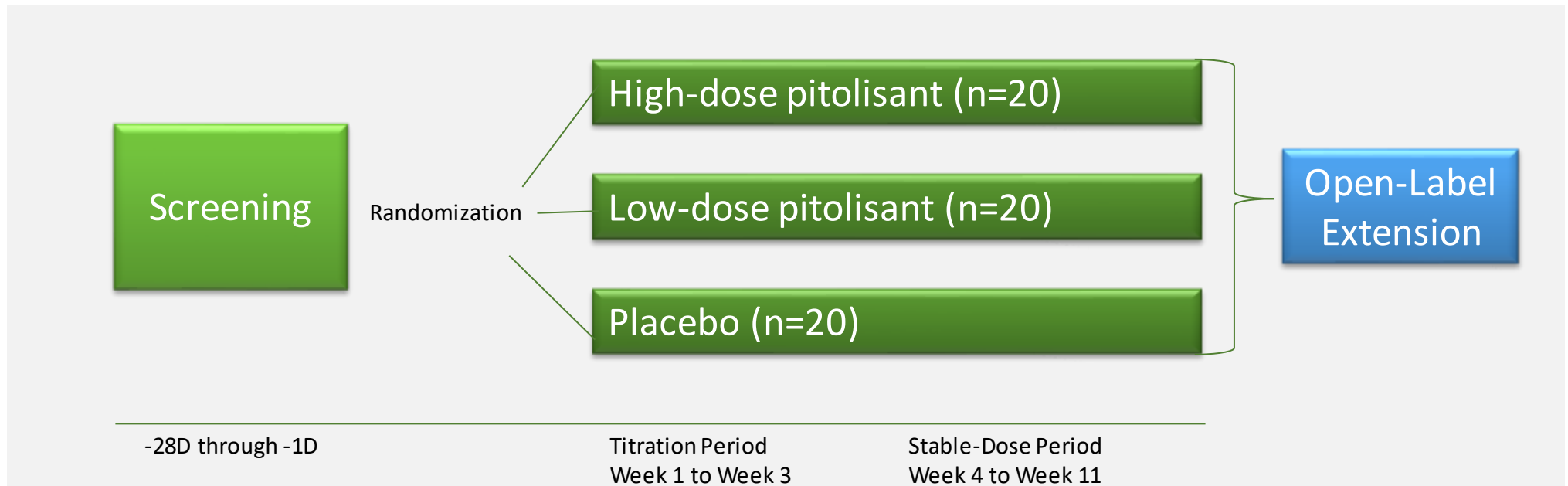
Pitolisant increases histamine levels in the brain and has demonstrated significant improvement in EDS in adult patients with narcolepsy



Studies have suggested potential role of histamine and benefit of pitolisant in attention, vigilance, and cognitive function^{11,12}

- *Preliminary evidence from both animal and human studies*
- *Further studies are needed in patients with PWS*

Phase 2 Clinical Trial of Pitolisant in Patients with PWS: Trial Design



Trial Design:

- Randomized, double-blind, placebo-controlled, parallel-group followed by open-label extension
- ~60 – 70 patients; ages 6 – 65
- Randomized-Controlled Phase
 - 11-week treatment period: 3-week titration period followed by 8-weeks of stable dosing
- Open-label extension planned to run throughout development program

Phase 2 Clinical Trial of Pitolisant in Patients with PWS: Study Objectives



Primary Objective: To evaluate the safety and efficacy of pitolisant compared to placebo in treating EDS in patients with PWS



Secondary Objectives - Impact of pitolisant on:

- Caregiver assessment of severity based on EDS
- Clinician assessment of severity based on overall PWS symptoms
- Behavioral assessments
- Cognitive function
- Caregiver burden
- Long-term safety and effectiveness



Exploratory Objectives – Impact of pitolisant on:

- ESS-CHAD as rated by caregivers
- Hyperphagia as measured by the HQCT
- Ghrelin levels

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)^{1,2}



Two forms: DM1 (~140,000 US patients) more common than DM2 (~3,000 - 29,000 US patients); earlier onset and more severe symptoms in DM1 patients compared to DM2^{1,2}



EDS and fatigue second only to muscle weakness in symptom prevalence and impact; impaired cognitive function another prominent symptom; decreased hypocretin levels in some patients^{1,2}



No approved treatments and significant unmet medical need



IND open; on-track to initiate Phase 2 clinical trial in 1H 2021

FY 2020 Financial Summary *(in millions, USD)*

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
Net Product Revenues	\$ 56.3	\$ 6.0	\$ 159.7	\$ 6.0
Total Operating Expenses	\$ 38.6	\$ 38.1	\$ 115.0	\$ 150.3
R&D Expense	7.6	5.3	19.4	69.6
S&M Expense	17.5	16.8	55.8	44.3
G&A Expense	13.5	16.0	39.7	36.4
Net Income (Loss)	\$ (0.2)	\$ (36.4)	\$ (36.9)	\$ (152.0)
Cash & cash equivalents			\$ 228.6	

Totals may not foot due to rounding

GAAP vs Non-GAAP Reconciliation *(in millions, USD)*

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
GAAP reported net loss	\$ (0.2)	\$ (36.4)	\$ (36.9)	\$ (152.0)
Interest expense / income	8.0	2.7	28.2	6.1
Taxes				
Depreciation	0.1	0.1	0.4	0.4
Amortization	4.3	1.9	9.8	2.8
EBITDA	12.1	(31.7)	1.5	(142.7)
Stock-based compensation expense	2.9	8.8	5.2	9.9
Loss on debt extinguishment			22.6	
Warrant expense			3.1	
Non-GAAP adjusted net income (loss)	15.1	(22.9)	32.5	(132.8)
Accumulation of yield on preferred stock		(9.6)	(26.9)	(35.2)
Non-GAAP adjusted net income (loss) available to common stockholders	\$ 15.1	\$ (32.5)	\$ 5.5	\$ (168.0)
GAAP reported net loss per diluted share	\$ (0.00)	\$ (5.92)	\$ (2.48)	\$ (24.07)
Non-GAAP adjusted net income (loss) per diluted share	\$ 0.25	\$ (4.18)	\$ 0.21	\$ (21.60)
Weighted average number of shares of common stock used in non-GAAP diluted per share	59,128,981	7,778,453	26,982,978	7,777,441

Totals may not foot due to rounding



Thank You

March 25, 2021

