



## HARMONY BIOSCIENCES TO PRESENT NEW WAKIX® (PITOLISANT) SAFETY AND EFFICACY DATA AT SLEEP 2023 ANNUAL MEETING

May 10, 2023 12:05 PM EDT

*Late-breaking Phase 2 proof-of-concept study in Prader-Willi syndrome accepted for poster presentation*

PLYMOUTH MEETING, Pa., May 10, 2023 /PRNewswire/ -- Harmony Biosciences Holdings, Inc. ("Harmony") (Nasdaq: HRMY), a pharmaceutical company dedicated to developing and commercializing innovative therapies for patients with rare neurological diseases, today announced that new safety and efficacy data from a Phase 2 proof-of-concept study evaluating pitolisant for the treatment of excessive daytime sleepiness in patients with Prader-Willi syndrome (PWS) will be highlighted as a poster presentation during a late-breaking abstract session at the 37th Annual Meeting of the Associated Professional Sleep Societies (APSS), known as "SLEEP 2023," in Indianapolis from June 3-7.



**HARMONY**  
**BIOSCIENCES**

Additional presentations by Harmony include an oral presentation covering a pooled analysis of clinically meaningful improvements in adults with narcolepsy, as well as poster presentations detailing the results of a pharmacokinetics study of WAKIX in breast milk and serum in healthy lactating women, and an ongoing prospective observational safety study evaluating WAKIX exposure in pregnant women and their offspring.

"As a company dedicated to developing and delivering treatments for rare neurological disorders, we are pleased to share a broad range of data for pitolisant at this year's SLEEP meeting," said Harmony's Chief Medical Officer, Kumar Budur M.D., M.S. "These data add to the growing body of scientific evidence that has been generated for pitolisant to help further characterize its safety, tolerability and clinical utility."

Dr. Budur added that the company is especially encouraged by the positive results from the Phase 2 proof-of-concept study in PWS in anticipation of an upcoming Phase 3 registration trial.

"Together, the data presented at SLEEP 2023 demonstrate Harmony's commitment to expanding the clinical utility of pitolisant for adult patients with narcolepsy while rigorously pursuing new indications for treating patients with unmet medical needs in other rare neurological disorders."

The following data presented by Harmony will be available in the meeting's gallery:

**Oral and Poster: "Clinically Meaningful Improvements with Pitolisant in Adults with Narcolepsy: Pooled Analysis of Randomized Clinical Trials."**

- **Oral Presentation Date and Time:** Wednesday, June 7, 2023, at 4:30 - 4:45 p.m. EDT

- **Poster Presentation Date and Time:** Tuesday, June 6, 2023, at 5:00 - 6:00 p.m. EDT

Poster Board Number: 294

**Poster: "Pharmacokinetics of Pitolisant in Breast Milk and Serum of Healthy Lactating Women."**

- **Poster Presentation Date and Time:** Tuesday, June 6, 2023, at 5:00 - 6:00 p.m. EDT

Poster Board Number: 232

**Poster: "Pitolisant Pregnancy Registry: An Observational Study of the Safety of Pitolisant Exposure in Pregnant Women and Their Offspring."**

- **Poster Presentation Date and Time:** Tuesday, June 6, 2023, at 12:00 - 1:15 p.m. EDT

Poster Board Number: 231

**Poster: "Primary Efficacy and Safety Results of a Phase 2 Double-Blind, Placebo-Controlled Proof of Concept, Signal Detection Study of Pitolisant in Prader-Willi Syndrome."**

- **Poster Presentation Date and Time:** Tuesday, June 6, 2023, at 5:00 – 6:00 p.m. EDT

Poster Board Number: 510

**About WAKIX® (pitolisant) Tablets**

WAKIX, a first-in-class medication, is approved by the U.S. Food and Drug Administration for the treatment of excessive daytime sleepiness or cataplexy in adult patients with narcolepsy and has been commercially available in the U.S. since Q4 2019. It was granted orphan drug designation for the treatment of narcolepsy in 2010, and breakthrough therapy designation for the treatment of cataplexy in 2018. WAKIX is a selective histamine 3 (H<sub>3</sub>) receptor antagonist/inverse agonist. The mechanism of action of WAKIX is unclear; however, its efficacy could be mediated through its activity at H<sub>3</sub> receptors, thereby increasing the synthesis and release of histamine, a wake promoting neurotransmitter. WAKIX was designed and developed by Bioprojet (France). Harmony has an exclusive license from Bioprojet to develop, manufacture and commercialize pitolisant in the United States.

**Indications and Usage**

WAKIX is indicated for the treatment of excessive daytime sleepiness or cataplexy in adult patients with narcolepsy.

**Important Safety Information**

**Contraindications**

WAKIX is contraindicated in patients with known hypersensitivity to pitolisant or any component of the formulation. Anaphylaxis has been reported. WAKIX is also contraindicated in patients with severe hepatic impairment.

**Warnings and Precautions**

WAKIX prolongs the QT interval; avoid use of WAKIX in patients with known QT prolongation or in combination with other drugs known to prolong the QT interval. Avoid use in patients with a history of cardiac arrhythmias, as well as other circumstances that may increase the risk of the occurrence of torsade de pointes or sudden death, including symptomatic bradycardia, hypokalemia or hypomagnesemia, and the presence of congenital prolongation of the QT interval.

The risk of QT prolongation may be greater in patients with hepatic or renal impairment due to higher concentrations of pitolisant; monitor these patients for increased QTc. Dosage modification is recommended in patients with moderate hepatic impairment and moderate or severe renal impairment (see full prescribing information). WAKIX is not recommended in patients with end-stage renal disease (ESRD).

**Adverse Reactions**

In the placebo-controlled clinical trials conducted in patients with narcolepsy with or without cataplexy, the most common adverse reactions (≥5% and at least twice placebo) for WAKIX were insomnia (6%), nausea (6%), and anxiety (5%). Other adverse reactions that occurred at ≥2% and more frequently than in patients treated with placebo included headache, upper respiratory tract infection, musculoskeletal pain, heart rate increased, hallucinations, irritability, abdominal pain, sleep disturbance, decreased appetite, cataplexy, dry mouth, and rash.

**Drug Interactions**

Concomitant administration of WAKIX with strong CYP2D6 inhibitors increases pitolisant exposure by 2.2-fold. Reduce the dose of WAKIX by half.

Concomitant use of WAKIX with strong CYP3A4 inducers decreases exposure of pitolisant by 50%. Dosage adjustments may be required (see full prescribing information).

H1 receptor antagonists that cross the blood-brain barrier may reduce the effectiveness of WAKIX. Patients should avoid centrally

acting H1 receptor antagonists.

WAKIX is a borderline/weak inducer of CYP3A4. Therefore, reduced effectiveness of sensitive CYP3A4 substrates may occur when used concomitantly with WAKIX. The effectiveness of hormonal contraceptives may be reduced when used with WAKIX and effectiveness may be reduced for 21 days after discontinuation of therapy.

### **Use in Specific Populations**

WAKIX may reduce the effectiveness of hormonal contraceptives. Patients using hormonal contraception should be advised to use an alternative non-hormonal contraceptive method during treatment with WAKIX and for at least 21 days after discontinuing treatment.

There is a pregnancy exposure registry that monitors pregnancy outcomes in women who are exposed to WAKIX during pregnancy. Patients should be encouraged to enroll in the WAKIX pregnancy registry if they become pregnant. To enroll or obtain information from the registry, patients can call 1-800-833-7460. The safety and effectiveness of WAKIX have not been established in patients less than 18 years of age.

WAKIX is extensively metabolized by the liver. WAKIX is contraindicated in patients with severe hepatic impairment. Dosage adjustment is required in patients with moderate hepatic impairment.

WAKIX is not recommended in patients with end-stage renal disease. Dosage adjustment of WAKIX is recommended in patients with moderate or severe renal impairment.

Dosage reduction is recommended in patients known to be poor CYP2D6 metabolizers; these patients have higher concentrations of WAKIX than normal CYP2D6 metabolizers.

Please see the [Full Prescribing Information](#) for WAKIX for more information.

To report suspected adverse reactions, contact Harmony Biosciences at 1-800-833-7460 or the FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

### **About Harmony Biosciences**

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. Established by Paragon Biosciences, LLC, in 2017 and headquartered in Plymouth Meeting, PA, our team of experts from a wide variety of disciplines and experiences is driven by our shared conviction that innovative science translates into therapeutic possibilities for our patients, who are at the heart of everything we do. For more information, please visit [www.harmonybiosciences.com](http://www.harmonybiosciences.com).

### **Forward Looking Statement**

*This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including statements regarding our product WAKIX. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the following: 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 development activities with Bioprojet and plans to explore the therapeutic potential of pitolisant in additional indications; our ongoing and planned clinical trials; the availability of favorable insurance coverage and reimbursement for WAKIX; the impact of the COVID-19 pandemic, including any current and future variants; the timing of and our ability to obtain regulatory approvals for pitolisant for other indications as well as any of our product candidates, including those we are developing with Bioprojet; our failure to achieve the potential benefits under our 2022 LCA with Bioprojet; 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; the impact of government laws and regulations; volatility and fluctuations in the price of our common stock; the significant costs and required management time as a result of operating as a public company; the fact that the price of Harmony's common stock may be volatile and fluctuate substantially; and the significant costs and required management time as a result of operating as a public company. These and other important factors discussed under the caption "Risk Factors" in our Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on February 21, 2023, and our other filings with the SEC could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change.*

### **Harmony Biosciences Media Contact:**

Cate McCanless

202-641-6086


[cmccanless@harmonybiosciences.com](mailto:cmccanless@harmonybiosciences.com)

**Harmony Biosciences Investor Contact:**

Luis Sanay, CFA

445-235-8386

[lsanay@harmonybiosciences.com](mailto:lsanay@harmonybiosciences.com)

 View original content to download multimedia: <https://www.prnewswire.com/news-releases/harmony-biosciences-to-present-new-wakix-pitolisant-safety-and-efficacy-data-at-sleep-2023-annual-meeting-301820237.html>

SOURCE Harmony Biosciences