HARMONY BIOSCIENCES ANNOUNCES US FOOD & DRUG ADMINISTRATION ORPHAN DRUG DESIGNATION FOR PITOLISANT FOR TREATMENT OF IDIOPATHIC HYPERSOMNIA

September 7, 2023 12:05 PM EDT

PLYMOUTH MEETING, Pa., Sept. 7, 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 the U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to pitolisant for the treatment of idiopathic hypersomnia (IH).

The FDA's Orphan Drug designation incentivizes the advancement of promising therapies for rare diseases. Approximately 80,000 people in the U.S. are believed to be affected by IH, with 40,000 currently having been diagnosed. IH is a condition with high unmet medical need.

Harmony is currently evaluating the efficacy and safety of pitolisant in adult patients with IH in the Phase 3 registrational INTUNE study, a double-blind, placebo-controlled, randomized withdrawal study. Topline study results are anticipated in the fourth quarter of 2023 following enrollment completion nine months ahead of plan.

"The FDA's decision to grant Orphan Drug designation reinforces our belief in pitolisant as a promising therapy for adult patients with IH, with the unique added benefit of it being a non-scheduled, once-daily treatment option working through histamine to improve wakefulness," said Kumar Budur, M.D., Chief Medical Officer at Harmony Biosciences. "With the completion of enrollment in our INTUNE study nine months ahead of schedule and topline results expected in Q4, this designation is a significant advance in our clinical and commercial development initiatives. We look forward to working closely with the FDA and the broader IH community to address the unmet medical needs of patients living with this condition."

About Idiopathic Hypersomnia
Idiopathic Hypersomnia (IH) is a rare and chronic neurological disease that is characterized by excessive daytime sleepiness (EDS) despite sufficient or even long sleep time. EDS in IH is usually not alleviated by naps, longer sleep or more efficient sleep. People living with IH experience significant EDS along with the symptoms of sleep inertia (prolonged difficulty waking up from sleep) and 'brain fog' (impaired cognition, attention, and alertness). The cause of IH is unknown, but it is likely due to alterations in areas of the brain that stabilize states of sleep and wakefulness. IH is one of the central disorders of hypersomnolence and, like narcolepsy, is a debilitating sleep disorder that can result in significant disruption in daily functioning.

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.

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 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 of the 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; statements related to our intended share repurchases and repurchase timeframe 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

Harmony Biosciences Investor Contact:
Luis Sanay, CFA
445-235-8386
lsanay@harmonybiosciences.com

View original content to download multimedia:https://www.prnewswire.com/news-releases/harmony-biosciences-announces-us-food--drug-administration-orphan-drug-designation-for-pitolisant-for-treatment-of-idiopathic-hypersomnia-301920012.html

SOURCE Harmony Biosciences