HARMONY BIOSCIENCES RECEIVES U.S. FOOD AND DRUG ADMINISTRATION ORPHAN DRUG DESIGNATION FOR PITOLISANT IN PRADER-WILLI SYNDROME

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PLYMOUTH MEETING, Pa., Feb. 20, 2024 /PRNewswire/ -- Harmony Biosciences Holdings, Inc. (Nasdaq: HRMY), announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to pitolisant for the treatment of Prader-Willi syndrome (PWS).

"The decision to grant Orphan Drug designation to pitolisant indicates that it could be a promising treatment option for people living with Prader-Willi syndrome," said Kumar Budur, M.D., M.S., Chief Medical Officer at Harmony Biosciences. "This designation marks an important step forward in our PWS development program and we are eager to continue working with the FDA and the broader community of PWS patients and caregivers to address their high unmet medical needs."

FDA Orphan Drug designation incentivizes the advancement of promising therapies for rare diseases by providing tax credits for clinical development, waivers for user fees, and seven years of market exclusivity following drug approval. Approximately 15,000 – 20,000 people in the U.S. are living with PWS, the majority experiencing behavioral symptoms and more than half with excessive daytime sleepiness (EDS).

In the upcoming Phase 3 registrational TEMPO study, Harmony will assess the safety and efficacy of pitolisant in treating EDS and behavioral disturbances in PWS. This global study, anticipated to begin in Q1 2024, will be a randomized, double-blind, placebo-controlled trial in patients six years and older with PWS.

Dr. Budur added, "We are excited about our upcoming Phase 3 TEMPO study and the progress we have made to broaden the clinical utility of pitolisant not just in PWS but other rare diseases as part of our life cycle management programs that, if successful, could potentially help over 100,000 patients. On behalf of Harmony, I would like to thank all the patients and family members for participating in our clinical trials, as well as our investigators and site personnel for their commitment to advancing science."

About Prader-Willi Syndrome

PWS is an orphan/rare, genetic neurological disorder with many of the symptoms resulting from hypothalamic dysfunction. The hypothalamus is the part of the brain that controls both sleep-wake state stability and signals that mediate the balance between hunger and satiety, resulting in the main symptoms in patients with PWS, hyperphagia (an intense persistent sensation of hunger accompanied by food preoccupations, an extreme drive to consume food, food-related behavior problems, and a lack of normal satiety), EDS and behavioral symptoms. Other features include low muscle tone, short stature, and cognitive impairment.

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.

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