Harmony Biosciences To Present New Data On Pitolisant In Patients With Narcolepsy At SLEEP 2019 Meeting

June 4, 2019 12:00 PM EDT

Five posters to be presented on a broad range of data, including human abuse potential data and new data on pitolisant related to its use with common treatments for narcolepsy and its overall safety/tolerability profile

PLYMOUTH MEETING, PA, June 4, 2019 —Harmony Biosciences, LLC (Harmony) announced today it will present data on the safety and tolerability of pitolisant, which has been studied for the treatment of both excessive daytime sleepiness (EDS) and cataplexy in adult patients with narcolepsy, at the upcoming 33rd Annual Meeting of the Associated Professional Sleep Societies, known as "SLEEP" in San Antonio, Texas from June 8-12. In addition to integrated data from clinical studies on the safety and tolerability of pitolisant, Harmony's presentations will highlight data from a human abuse potential study of pitolisant, the pitolisant U.S. expanded access program, and an analysis of pitolisant in combination with other medications for the treatment of narcolepsy.

"As a company dedicated to novel treatments for rare 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, Jeffrey Dayno, M.D. "These data are representative of the body of scientific evidence that has been generated for pitolisant, which characterizes its safety/tolerability and efficacy profile in adult patients with narcolepsy with or without cataplexy. Our focus continues to be making pitolisant available as a potential new treatment option for those individuals affected by this rare, debilitating neurologic disorder."

Data being presented by Harmony include:

- **Poster Presentation: The Safety and Tolerability of Pitolisant in the Treatment of Excessive Daytime Sleepiness and Cataplexy in Adult Patients with Narcolepsy: An Open-Label, Expanded Access Program in the United States** (Poster 0611, June 9, 5:15 p.m. - 6:15 p.m.) *E Bauer, C Davis, A Patroneva, J Dayno, M Thorpy*.
    - Results are being presented from an interim data collection of the Pitolisant Expanded Access Clinical Evaluation (PEACE) Program, which provides adult patients in the U.S. living with narcolepsy access to treatment with pitolisant.
- **Poster Presentation: Evaluation of Abuse Potential of the Narcolepsy Medication Pitolisant** (Poster 0612, June 9, 6:15 p.m. - 7:15 p.m.) *J Dayno, C Scart-Grès, P Robert, J-C Schwartz, B Setnik*.
    - Results reported are from a randomized, double-blind, active- and placebo-controlled, single-dummy, four-sequence four-way crossover study evaluating the abuse potential of single-dose pitolisant compared with the stimulant phentermine HCl (C-IV) and placebo in nondependent recreational stimulant users.
- **Poster Presentation: Safety and Tolerability of Pitolisant in the Treatment of Adults with Narcolepsy: Integrated Data from Clinical Studies** (Poster 0614, June 9, 6:15 p.m. - 7:15 p.m.) *C Scart-Grès, C Momah, M Roy, K Maski, S Piris, R Bogan*.
    - This presentation reports on an analysis of integrated safety data from four randomized, placebo-controlled, clinical studies of pitolisant in adult patients with narcolepsy.
- **Poster Presentation: Pitolisant in Combination with Other Medications for the Management of Narcolepsy** (Poster 0615, June 9, 5:15 p.m. - 6:15 p.m.) *K Doghramji, C Davis, A Patroneva, J-C Schwartz, C Scart-Grès, P Robert, S Wanaski, A Krystal*.
    - Current standard of care in narcolepsy often involves polypharmacy. This poster presentation reports results from a drug-drug interaction study that evaluated the pharmacokinetics of pitolisant when administered with sodium oxybate or modafinil. It also includes results from an open-label long-term safety study of pitolisant with analyses of effectiveness and adverse events in patients with narcolepsy treated with pitolisant as monotherapy, as well as in patients treated with pitolisant and concomitant psychostimulants, anticataplectics, and both psychostimulants and anticataplectics.
- **Poster Presentation: Burden of Narcolepsy: A Survey of Patients and Physicians** (Poster 0592, June 9, 6:15

p.m. - 7:15 p.m.) *M Thorpy, J Hopper, A Patroneva*.

- This presentation will report results from a survey on perceptions of healthcare professionals and patients living with narcolepsy regarding the burden, symptoms and treatment of narcolepsy. Discrepancies between patient and healthcare professional responses will also be described.

About Pitolisant
Pitolisant is an investigational medication in the U.S. that is not approved by the FDA. It was granted orphan designation for the treatment of narcolepsy, Fast Track designation for the treatment of excessive daytime sleepiness (EDS) and cataplexy in patients with narcolepsy, and Breakthrough Therapy designation for the treatment of cataplexy in patients with narcolepsy. Pitolisant, a first-in-class medication, is a potent and highly selective histamine 3 (H_3) receptor antagonist/inverse agonist; it enhances the activity of histaminergic neurons in the brain that function to improve a patient's wakefulness and inhibit attacks of cataplexy. It was designed and developed by Bioprojet, who has marketed the product in Europe since its approval by the European Medicines Agency in 2016. Harmony's goal is to obtain FDA approval to market this new medication in the U.S. in 2019. If approved, pitolisant would represent the first new therapy in the U.S. in over 15 years for the treatment of both EDS and cataplexy in adult patients with narcolepsy.

About Narcolepsy
Narcolepsy is a rare, chronic, debilitating neurologic disorder of sleep-wake state instability that impacts up to 200,000 Americans and is primarily characterized by EDS, cataplexy, and other manifestations of REM sleep dysregulation, which intrude into wakefulness. In most patients, it is caused by the loss of hypocretin, a neuropeptide in the brain that supports sleep-wake state stability. This disorder affects men and women equally, with typical symptom onset in adolescence or young adulthood; however, it can take up to a decade to be properly diagnosed. Narcolepsy can cause significant burden for patients and their families, affecting their ability to perform routine tasks, limit achievement at school and work, impact social relationships and cause impairment in overall quality of life.

About Cataplexy
Cataplexy is one of several symptoms of narcolepsy that represent elements of REM sleep state intruding into wakefulness, characterized by sudden temporary loss of muscle tone. Cataplexy can be subtle, such as drooping of eyelids, or severe, such as knee buckling or total body collapse. Often times, symptoms of cataplexy may go unrecognized because of the subtle nature of the symptoms in some patients, variability of how cataplexy is expressed, and/or lack of patient complaints or physician recognition of the symptoms as manifestations of cataplexy. This symptom of narcolepsy can often cause significant impact on a person's ability to carry out normal daily functions. Up to two-thirds of all patients with narcolepsy have cataplexy (known as Type 1 narcolepsy); cataplexy is one of the most debilitating symptoms of this chronic, rare neurologic disorder.

Harmony Biosciences, LLC
Harmony Biosciences, LLC, is a private biopharmaceutical company headquartered in Plymouth Meeting, PA. The company was established in October 2017 with a vision to provide novel treatment options for people living with rare and orphan diseases, with an emphasis on central nervous system disorders, starting with patients living with narcolepsy. Harmony is committed to advancing the understanding of narcolepsy and providing information and resources to individuals who live with, and healthcare professionals who treat patients with, this disorder. For more information on Harmony Biosciences, visit www.harmonybiosciences.com.

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