Harmony Biosciences To Present Data On Pitolisant In Patients With Narcolepsy At 2019 American Academy Of Neurology Meeting

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Highlights include oral presentation on long-term efficacy and safety of pitolisant and key data analyses from pivotal trials

PLYMOUTH MEETING, PA, April 29, 2019 —Harmony Biosciences, LLC (Harmony) today announced it will present data on the efficacy and safety 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 2019 American Academy of Neurology (AAN) annual meeting in Philadelphia, May 4-10. Harmony is a biopharmaceutical company dedicated to developing and commercializing novel treatment options for people living with rare diseases.

“We are pleased to have the opportunity to present data on pitolisant at this year’s AAN annual meeting demonstrating our commitment to investigating treatment options for people living with rare diseases such as narcolepsy,” said Harmony’s Chief Medical Officer, Jeffrey Dayno, M.D. “These data reflect the body of scientific evidence that establishes the efficacy and safety of pitolisant as a first-in-class molecule with a novel mechanism of action and potential new treatment option for patients living with narcolepsy. We also look forward to sharing data on patient and healthcare professional perceptions on the burden of narcolepsy, a rare and chronic debilitating neurologic disorder of sleep-wake state instability that impacts as many as 200,000 Americans.”

Data being presented by Harmony includes:

- **Oral Presentation: Long-Term Evaluation of Safety and Efficacy of Pitolisant in Narcolepsy: HARMONY 3** (Oral Presentation, May 9, 2:28 p.m., by Jeffrey Dayno, M.D.) *Y Dauvilliers, I Arnulf, Z Szakács, C Scart-Grès, I Lecomte, C Caussé, J-C Schwartz*.
    - Harmony III is an open-label, long-term (up to five years) study of pitolisant, the first potent and highly selective histamine 3 (H3) receptor antagonist/inverse agonist, in patients with narcolepsy. This presentation will focus on findings from a one-year analysis that assessed the long-term safety and efficacy of pitolisant in the treatment of excessive daytime sleepiness (EDS) and cataplexy in patients with narcolepsy.
- **Poster Presentation: Efficacy and Safety of Pitolisant in Patients with Narcolepsy: A Review of Clinical Trials** (Poster 6-034, May 7, 5:30-6:30 p.m.) *Y Dauvilliers, J-C Schwartz, C Davis, J Dayno*.
    - Results from Harmony 1 and Harmony CTP, two randomized, double-blind, placebo-controlled pivotal studies that assessed the efficacy and safety of pitolisant for the treatment of EDS (Harmony 1) and cataplexy (Harmony CTP) in adult patients with narcolepsy, will be included in this presentation.
- **Poster Presentation: Burden of Narcolepsy: A Survey of Patients and Physicians** (Poster 6-036, May 7, 5:30-6:30 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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