Harmony Biosciences Presents Clinically Meaningful Open-Label Extension Study Effectiveness Data for EPX-100 in Dravet Syndrome

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Data presented at the 2025 American Epilepsy Society Annual Meeting

PLYMOUTH MEETING, Pa.--(BUSINESS WIRE)--Dec. 8, 2025-- Harmony Biosciences Holdings, Inc. (Nasdaq: HRMY), today announced the presentation of initial open-label extension (OLE) data from the company's ongoing Phase 3 ARGUS trial investigating EPX-100 (clemizole hydrochloride) for the treatment of Dravet syndrome (DS), which showed clinically meaningful reductions in seizure activity in participants with DS along with a favorable benefit-risk profile.

The data from poster #3.353 will be presented at the American Epilepsy Society (AES) Annual Meeting in Atlanta, Ga., on Monday, December 8, from 12:00 pm – 1:45 pm ET.

Data from the eighteen participants in the OLE trial with at least six months' exposure to EPX-100 demonstrated the following:

- A median reduction of approximately 50% in countable motor seizure frequency per 28 days (CMS-28)
- 50% of these participants achieved at least a 50% reduction in CMS-28
- EPX-100 was generally well-tolerated in participants receiving treatment for more than two years and approaching three years in the OLE phase
- The most common treatment emergent adverse events (≥ 5%) were seizures, pyrexia and upper respiratory tract infection
- There were no significant gastrointestinal adverse events (2%) and no additional laboratory testing or special monitoring is being performed in the trial

These data suggest a positive emerging benefit-risk profile for EPX-100, supported by clinically meaningful reduction in seizure frequency and a favorable safety/tolerability profile.

"The ARGUS trial is one of the most advanced development programs in the 5-HT2 (serotonin) agonist class and the effectiveness, safety and tolerability data of EPX-100 dosed BID from the open-label extension study are very encouraging," said Kumar Budur, MD, MS, Chief Medical and Scientific Officer at Harmony Biosciences. "These initial results support the advancement of our epilepsy franchise as we progress toward the topline data readout from the ARGUS trial in 2026."

EPX-100 is being evaluated as an investigational treatment for DS in the Phase 3 ARGUS trial, and for Lennox-Gastaut syndrome in the Phase 3 LIGHTHOUSE trial in global, multicenter, randomized, double-blind, placebo-controlled clinical trials to assess its safety and efficacy as adjunctive therapy.

The ongoing ARGUS and LIGHTHOUSE trials are currently enrolling and more information can be found at argustrial.com and lighthouselgsstudy.com.

About Clemizole Hydrochloride (EPX-100)

EPX-100, clemizole hydrochloride, is an investigational product under development for the treatment of Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS). EPX-100 acts by targeting central 5-hydroxytryptamine 2 (5HT-2) serotonin receptors to modulate serotonin signaling. EPX-100 is administered orally twice a day in a liquid formulation and has been developed based on a proprietary phenotype-based zebrafish drug screening platform. These *scn1Lab* mutant zebrafish replicate the genetic etiology and phenotype observed in the majority of individuals with DS. The *scn1Lab* mutant zebrafish model that expresses voltage gated sodium channels has been used for high-throughput screening of compounds that modulate Nav1.1 in the central nervous system.

About Dravet Syndrome

Dravet syndrome (DS) is a severe and progressive developmental epileptic encephalopathy that causes significant impact on patient functioning. DS begins in the first year of life and is characterized by high seizure frequency and severity, intellectual disability, and an increased risk of sudden unexpected death in epilepsy (SUDEP). Approximately 85% of Dravet syndrome cases are caused by de novo loss-of-function (LOF) mutations in a voltage-gated sodium channel gene, *SCN1A*. DS has an estimated incidence rate of 1:15,700 in the US.

About Lennox-Gastaut Syndrome

Lennox-Gastaut syndrome (LGS) is a rare and drug-resistant epileptic encephalopathy characterized by onset in children between 3-5 years of age. The underlying cause of LGS is unknown and can be related to a wide range of factors including genetic differences and structural differences in the brain. As a result, patients experience multiple seizure types, including atonic seizures, and developmental, cognitive, and behavioral issues. LGS affects approximately 48,000 patients in the U.S.

About Harmony Biosciences

Harmony Biosciences is a pharmaceutical company dedicated to developing and commercializing innovative therapies for patients with rare neurological diseases who have unmet medical needs. Driven by novel science, visionary thinking, and a commitment to those who feel overlooked, Harmony Biosciences is nurturing a future full of therapeutic possibilities that may enable patients with rare neurological diseases to truly thrive. Established by Paragon Biosciences, LLC, in 2017 and headquartered in Plymouth Meeting, Pa., we believe that when empathy and innovation meet, a better future can begin; a vision evident in the therapeutic innovations we advance, the culture we cultivate, and the community programs we foster. For more information, please visit www.harmonybiosciences.com.

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