Harmony Biosciences Acquires Epygenix Therapeutics, Inc., Adding Late-Stage Epilepsy Franchise to Growing Pipeline of Innovative CNS Assets

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Lead Candidate, EPX-100, Holds Orphan Drug and Rare Pediatric Disease Designations

EPX-100 in Pivotal Registrational Trial for Dravet Syndrome; Topline Data Expected in 2026

Plan to Initiate Phase 3 Trial for Lennox-Gastaut Syndrome in Second Half of 2024

Potential Billion Dollar Plus Market Opportunity in Rare Epilepsy

PLYMOUTH MEETING, Pa., April 30, 2024 /PRNewswire/ -- Harmony Biosciences (Nasdaq: HRMY), today announced the acquisition of Epygenix Therapeutics, Inc., accelerating its growth strategy by adding a rare epilepsy franchise to its expanding late-stage pipeline of innovative CNS assets.

"The acquisition of Epygenix gives us three distinct CNS franchises in late-stage development, each with a potential US peak sales opportunity of $1B - $2B," said Jeffrey M. Dayno, M.D., President and Chief Executive Officer at Harmony Biosciences. "We have been strategically building a robust and diverse pipeline of innovative CNS assets for rare diseases that has transformed our business. This acquisition builds on our leadership position in sleep/wake and our franchise in neurobehavioral disorders, allowing us to leverage our expertise in CNS and internal synergies to deliver new therapies to patients with unmet medical needs."

"Harmony shares our team's core values, including a commitment to patients and families living with Dravet syndrome and Lennox-Gastaut syndrome, where a significant unmet need for effective therapies remains," said Alex Yang, Chair and CEO of Epygenix Therapeutics. "Harmony's demonstrated expertise in drug development and commercialization will accelerate the full potential of these treatments and make a real difference in the lives of Dravet and LGS families."

The acquisition includes clemizole hydrochloride (EPX-100), a potent, oral, centrally acting serotonin (5HT2) agonist. It is currently in a pivotal registrational clinical trial for the treatment of Dravet syndrome in children and adults and is poised to enter Phase 3 for the treatment of Lennox-Gastaut syndrome. The proven mechanism of action of clemizole could potentially offer an improved product profile over currently available treatment options and improve daily functioning in patients living with Dravet syndrome and Lennox-Gastaut syndrome.

EPX-100 has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPDD) from the FDA for both Dravet syndrome and Lennox-Gastaut syndrome. A second investigational product, EPX-200, is a potent, oral, centrally active and selective 5HT2C agonist, and is currently in IND-enabling studies. EPX-200 also received ODD from FDA for Dravet syndrome and Lennox-Gastaut syndrome as well as RPDD for Lennox-Gastaut syndrome.

Under the terms of the definitive agreement, Harmony paid $35 million in cash with the potential for payments of up to $130 million based on development and regulatory milestones. In addition, there are potential payments of up to $515 million if certain sales milestones are achieved.

About Clemizole hydrochloride (EPX-100)

EPX-100, clemizole hydrochloride, is under development for the treatment of Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS). EPX-100 acts by targeting central 5-hydroxytryptamine receptors to modulate serotonin signaling. The drug candidate is administered orally twice a day in a

liquid formulation and has been developed based on a proprietary phenotype-based zebrafish drug screening platform.[1] DS is caused by a loss of function mutation in the SCN1A gene, and scn1 mutant zebrafish replicate the genetic etiology and phenotype observed in the majority of DS patients. 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 epileptic encephalopathy that begins in infancy and 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 a risk of sudden unexpected death in epilepsy.[1] Approximately 85% of Dravet syndrome cases are caused by de novo loss-of-function (LOF) mutations in a voltage-gated sodium channel gene, SCN1A1.[2] DS has an estimated incidence rate of 1:15,700[3].

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.[2,4] As a result, patients experience multiple seizure types, including atonic seizures, and developmental, cognitive, and behavioral issues.[3] LGS affects approximately 48,000 patients in the U.S[5].

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.

About Epygenix Therapeutics, Inc.

Epygenix Therapeutics, Inc. is a precision medicine-based biopharmaceutical company focused on genetically screening, discovering, and developing drugs to treat rare and intractable forms of genetic epilepsy in childhood, such as Dravet syndrome (DS). Epygenix is currently focused on developing EPX-100 and EPX-200, drug candidates that were discovered in a zebrafish model of DS which replicates the genetic mutation that causes DS and mimics the human pathology. This mechanism of action has been shown to eliminate both clinical and electrographic seizure activity which could translate into improved patient functioning.

Forward-Looking Statements

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 and our future capabilities following the acquisition of Epygenix, including clemizole hydrochloride. 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 and our other product candidates, including clemizole hydrochloride; the rate and degree of market acceptance and clinical utility of WAKIX, pitolisant in additional indications, if approved, and any other product candidates, including clemizole hydrochloride, 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 and our other product candidates, including clemizole hydrochloride; 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 and those that we have acquired; our failure to achieve the potential benefits of the 2022 LCA with Bioprojet; our ability to recognize the intended benefits of our acquisition of Zynerba Pharmaceuticals, Epygenix and another entity or assets that we acquire; 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, as amended, filed with the Securities and Exchange Commission (the "SEC") on February 22, 2024, 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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[1] EpyGenix Company Presentation: https://www.epygenix.com/news

[2] EpyGenix Poster: https://www.epygenix.com/_files/ugd/4ad619_2db63a277738444c85e70a47b816a67c.pdf

[3] Wu, E., et. al. (2015). Incidence of Dravet Syndrome in a US Population. *Pediatrics 136(5):* 1310-e1315. doi: 10.1542/peds.2015-1807. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4621800/

[4] https://www.epygenix.com/rare-genetic-epilepsy

[5] https://www.lgsfoundation.org/about-lgs-2/how-many-people-have-lgs/

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