Epygenix Therapeutics Receives FDA Orphan Drug Designation for EPX-100 For Treatment of Ohtahara Syndrome

PARAMUS, N.J., UNITED STATES, June 29, 2022 /EINPresswire.com/ -- Epygenix Therapeutics, a clinical-stage biopharmaceutical company developing safe, effective, and patient-friendly drugs for rare and intractable forms of genetic epilepsy, today announces that the U.S. Food & Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to EPX-100 to treat Ohtahara syndrome. EPX-100 was previously granted Rare Pediatric Disease (RPD) designation by the FDA for Ohtahara and has also received FDA ODD and RPD designations for Dravet syndrome and Lennox-Gastaut syndrome (LGS).

"We are encouraged that the FDA recognizes the deep unmet need among Ohtahara families to develop an effective treatment for this devastating and drug resistant disease. The FDA's decision also underscores EPX-100's potential to deliver family-friendly treatments to a broad range of devastating rare genetic epilepsies, including Ohtahara. We look forward to working with the FDA and regulators around the world to further advance development," said Dr. Lorianne Masuoka, Epygenix Therapeutics' Chief Medical Officer.

Under the Orphan Drug Act, Orphan Drug Designation accords sponsors with incentives to facilitate drug development for rare diseases affecting fewer than 200,000 people in the US. These incentives include a potential seven-year market exclusivity if the drug candidate receives FDA approval along with certain tax credits for qualified clinical trial costs, exemptions from certain FDA application fees, and assistance in clinical trial design.

About Ohtahara syndrome

Ohtahara syndrome, also known as early infantile epileptic encephalopathy (EIEE), is a life-threatening rare epilepsy syndrome that has an onset within the first few weeks of life. Ohtahara syndrome is associated with severe cognitive development impairment and persistent seizures resistant to current therapies. While the cause for many cases cannot be determined, Ohtahara syndrome has been associated with mutations in certain genes including STXBP1.

About EPX-100 (clemizole hydrochloride)
EPX-100 (clemizole hydrochloride) is a first-generation antihistamine and 5HT2 agonist in clinical development for rare genetic epilepsies. EPX-100 has been found to be a powerful suppressor of spontaneous convulsive behavior and electrographic seizures in genetically-modified zebrafish disease models and is being developed as a New Chemical Entity. Rigorous nonclinical toxicology and safety studies, and a Phase 1 human safety trial have been completed. EPX-100 is currently being studied in the ARGUS trial, a Phase 2 potentially pivotal study to determine efficacy in the treatment of Dravet syndrome. More information is available at www.clinicaltrials.gov (NCT04462770) and www.argustrial.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. Epygenix is currently focused on developing EPX-100, EPX-200, and EPX-300. These drug candidates reduce convulsive behavior and electrographic seizure activity and were discovered in a zebrafish Dravet Syndrome model which replicates the genetic mutation and mimics the human pathology.

Epygenix's largest shareholder is Mstone Partners, an entrepreneurial biotech incubator in the form of a holding company which owns and manages a portfolio of drug development companies. Mstone focuses on developing novel and repurposed drugs for rare, neurodegenerative disorders. Since 2016, Mstone has invested in two companies in the US and one in Hong Kong which are now in advanced clinical-stages with US FDA. Mstone has also established a number of portfolio companies under the Curestone Platform, which manages a portfolio of drug development companies in a centralized, hub-and-spoke model.

For more information, please visit www.epygenix.com.

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