Zogenix Receives Orphan Drug Designation for FINTEPLA® (Fenfluramine) in Japan

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- *Zogenix plans to submit a new drug application (J-NDA) for FINTEPLA in Japan for the treatment of seizures associated with Dravet syndrome by year end.*
- *Dravet syndrome is a rare, life-long form of epilepsy marked by severe seizures, significant developmental delays, frequent hospitalizations and medical emergencies, and increased risk of premature death*.
- *Seizures associated with Dravet syndrome are highly resistant to existing anti-epileptic medicines, pointing to a major need for new treatment options.*

EMERYVILLE, Calif., Aug. 26, 2021 (GLOBE NEWSWIRE) -- Zogenix (NASDAQ: ZGNX), a global biopharmaceutical company developing rare disease therapies, today announced that the Japanese Ministry of Health, Labour & Welfare (MHLW) has granted Orphan Drug Designation to FINTEPLA® (fenfluramine) oral solution, which Zogenix is developing in Japan as a potential treatment for seizures associated with Dravet syndrome.

Dravet syndrome is a rare infant- and childhood-onset epilepsy marked by frequent and severe treatment-resistant seizures, associated hospitalizations and medical emergencies, significant developmental and motor impairments, and an increased risk of sudden premature death.

The MHLW accreditation of Orphan Drug Designation follows the Japanese Pharmaceutical Affairs & Food Sanitation Council's (PAFSC) First Committee on Drugs agreement on July 28 to grant orphan drug designation for FINTEPLA for Dravet syndrome. The PAFSC is organized under the MHLW and consists of several expert committees from various fields, who serve as the decision-making body for drug approval (J-NDA, s-NDA) as well as Orphan Drug designation. In Japan, Orphan Drug Designation may be granted to drug candidates designed to treat diseases with fewer than 50,000 patients or diseases that are designated as intractable and the need for improved medical care is high.

Zogenix is on track to submit a J-NDA for FINTEPLA for the treatment of seizures associated with Dravet syndrome later this year. If approved, the product will be made available in Japan through an exclusive distribution agreement with Nippon Shinyaku, Co., Ltd., a leading Japanese pharmaceutical product developer and distributor.

"The orphan drug designation of FINTEPLA in Japan is an important milestone in our mission to meet the unmet needs of severe, rare epilepsy patients around the world," said Stephen J. Farr, Ph.D., President and Chief Executive Officer of Zogenix. "We are proud to have worked with patients, physicians, and regulators in Japan to achieve this milestone and reinforce our commitment to working with the MHLW to bring this much-needed potential treatment option to patients and families living with Dravet syndrome in Japan."

The incidence of Dravet syndrome in Japan is estimated to be 1 in 20,000-40,000. There are an estimated 3,000 Dravet syndrome patients in Japan based on the Ministry of Health, Labour and Welfare (MHLW) Patient Survey.

About Dravet Syndrome
Dravet syndrome is a rare, devastating and life-long form of epilepsy that generally begins in infancy and is marked by frequent, treatment-resistant seizures, significant developmental, motor, and behavioral impairments, and an increased risk of sudden unexpected death in epilepsy (SUDEP). Affecting one in 15,700 live births in the U.S. and approximately one in 20,000 to 40,000 live births in Europe, most patients follow a course of developmental delay with cognitive, motor and behavioral deficits that persist into adulthood. Dravet syndrome severely impacts quality of life for patients, families and caregivers due to the high physical, emotional, caregiving, and financial burden associated with the disease.

About FINTEPLA®
FINTEPLA is a prescription medicine used to treat the seizures associated with Dravet syndrome in patients 2 years of age and older. FINTEPLA possesses dual activities to inhibit seizures: as a serotonergic agent, acting as a potent 5-HT releaser with agonist activity at 5-HT1D, 2A, and 2C receptors, and as a positive modulator of Sigma1R. Fenfluramine is approved in the United States and Europe and is in development in Japan for the treatment of seizures associated with Dravet syndrome.

Across multiple clinical studies, FINTEPLA demonstrated significant and sustained reduction of convulsive seizures associated with Dravet syndrome. In two pivotal Phase 3 trials, the reduction in convulsive seizure frequency per 28 days was statistically significantly greater for all dose groups of FINTEPLA compared to placebo. Please see important FINTEPLA prescribing and safety information at www.fintepla.com.

About Zogenix
Zogenix is a global biopharmaceutical company committed to developing and commercializing therapies with the potential to

transform the lives of patients and their families living with rare diseases. The company's first rare disease therapy, FINTEPLA ® (fenfluramine) oral solution, has been approved by the U.S. FDA and the European Medicines Agency and is in development in Japan for the treatment of seizures associated with Dravet syndrome, a rare, severe lifelong epilepsy. The company has two additional late-stage development programs: one in a rare epilepsy called Lennox-Gastaut syndrome and one in a mitochondrial disease called TK2 deficiency. Zogenix also plans to initiate a study of FINTEPLA in a genetic epilepsy called CDKL5 Deficiency Disorder (CDD) and is collaborating with Tevard Biosciences to identify and develop potential next-generation gene therapies for Dravet syndrome and other genetic epilepsies.

Forward-Looking Statements

Zogenix cautions you that statements included in this press release that are not a description of historical facts are forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "indicates," "will," "intends," "potential," "suggests," "assuming," "designed," and similar expressions are intended to identify forward-looking statements. These statements include plans to submit a J-NDA for FINTEPLA in Japan and the estimated timing for that submission. These statements are based on Zogenix's current beliefs and expectations. The inclusion of forward-looking statements should not be regarded as a representation by Zogenix that any of its plans will be achieved. Actual results may differ from those set forth in this release due to the risks and uncertainties inherent in Zogenix's business, including, without limitation: the COVID-19 pandemic may disrupt Zogenix's business operations, impairing regulatory submissions and approvals; unexpected adverse side effects or inadequate therapeutic efficacy of fenfluramine that could limit approval in Japan or commercialization in the United states or Europe, or that could result in recalls or product liability claims; and other risks described in Zogenix's prior press releases as well as in public periodic filings with the U.S. Securities & Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and Zogenix undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

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