New FINTEPLA® (Fenfluramine) Data Show Long-Term Seizure Frequency Reductions in Patients with Lennox-Gastaut Syndrome (LGS)

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- *Median reduction in drop seizure frequency was 39.4% at 3 months (n= 227; p<0.0001) and 51.8% for patients assessed over months 10 to 12 (n=170; p<0.0001).*
- *A Supplemental New Drug Application (sNDA) was recently submitted seeking FDA approval for the use of FINTEPLA in LGS.*

EMERYVILLE, Calif., Sept. 30, 2021 (GLOBE NEWSWIRE) -- Zogenix (NASDAQ: ZGNX), a global biopharmaceutical company developing rare disease therapies, today announced data from an interim analysis of an on-going 12-month Phase 3 open-label extension (OLE) study (Study 1601; Part 2) showing that treatment with FINTEPLA® (fenfluramine) oral solution led to a clinically meaningful and sustained reduction in drop seizures in patients with Lennox-Gastaut syndrome (LGS) on antiseizure medications (ASMs). LGS is a rare, severe childhood-onset form of epilepsy in which seizures are extremely difficult to control and are associated with significant cognitive, behavioral, and motor disabilities. These data were presented today at the Child Neurology Society (CNS) 2021 Annual Meeting, which is being held September 29 - October 2, 2021.

"We are excited to report this study analysis on the long-term treatment effect of FINTEPLA for LGS patients in need of relief from the significant burden associated with life-long, treatment-resistant seizures," said Kelly Knupp, M.D., MSCS, FAES associate professor of Children's Hospital Colorado and principal investigator for the study. "These results highlight the potential of FINTEPLA to reduce the frequency of seizures experienced by LGS patients to meaningfully improve the outcomes associated with the disease long-term."

Study Results
A total of 247 patients (mean age, 14 years) entered the OLE study after completion of the randomized, controlled portion of Study 1601. An interim analysis was conducted when >90% of patient data were available. The median baseline drop seizure frequency prior to study treatment was 75 per month (range 4 - 2943).

Results presented as of the cut-off date showed patients experienced significant improvements in the frequency of drop seizures in the OLE study. During the treatment period, the median reduction in drop seizure frequency was 39.4% at 3 months (n=227; p<0.0001) and 51.8% for those patients assessed at months 10 to 12 (n=170; p<0.0001). Additionally, of the 170 patients assessed at this timepoint, most (51.2%) responded with a clinically meaningful (≥50%) reduction in drop seizures, while 25.3% of patients demonstrated a profound (≥75%) reduction. Also, 49.2% of investigators and 48.8% caregivers rated their patients as being "much improved" or "very much improved" on the Clinical Global Impression of Improvement (CGI-I) scale.

"We are very pleased with the compelling results of this study analysis demonstrating the potential long-term impact and clinical benefit of FINTEPLA for LGS patients, who face challenges in finding effective seizure control," said Gail Farfel, Ph.D., Executive Vice President and Chief Development Officer at Zogenix and one of the study authors. "We are also excited to have recently submitted a supplemental New Drug Application seeking approval of the use of FINTEPLA for the treatment of seizures associated with LGS."

In the long-term trial, FINTEPLA was generally well tolerated with no observed valvular heart disease or pulmonary hypertension. The most common treatment-emergent adverse events were decreased appetite (n=40, 16.2%), fatigue (n=33, 13.4%), nasopharyngitis (n=31, 12.6%) and seizure (n=27, 10.9%). One patient death occurred; the cause was reported as aspiration pneumonia and was considered unrelated to study drug.

The poster, titled, "FINTEPLA (fenfluramine) provides clinically meaningful reduction in frequency of seizures resulting in a drop in patients with Lennox-Gastaut syndrome for up to 1 year: interim analysis of an open-label extension study," (Kelly G. Knupp, Ingrid E. Scheffer, Berten Ceulemans, et al) is available on the Zogenix newsroom.

About the OLE Interim Analysis
Patients with LGS who completed a phase 3 randomized clinical trial and were eligible could enroll in the OLE study (NCT03355209). Effectiveness and safety/tolerability were assessed at Months 1, 2, and 3, and thereafter at 3-month intervals. A total of 247 patients had enrolled in the OLE as of October 19, 2020 and had at least 1 dose of study drug in the OLE.

About Lennox-Gastaut Syndrome
Lennox-Gastaut Syndrome (LGS) is a rare and devastating lifelong childhood-onset epilepsy that can arise from multiple different causes. LGS is characterized by many different seizure types, including many that result in frequent falls and injuries and that often don't respond to currently available seizure medications The intellectual and behavioral problems associated with LGS, as well as around-the-clock care requirements, add to the complexity of life with this disease.

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. In Japan, Zogenix is on track to submit a new drug application (J-NDA) for FINTEPLA for the treatment of seizures associated with Dravet syndrome in late 2021.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 the potential for FINTEPLA to reduce the frequency of seizures and provide clinical benefit to LGS patients, if approved and statements regarding Zogenix's clinical development plans. 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: interim data from the OLE study and other clinical trials may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data; the COVID-19 pandemic may continue to disrupt Zogenix's business operations, including timing of clinical visits in the ongoing OLE study, impairing regulatory submissions and approvals; unexpected adverse side effects or inadequate therapeutic efficacy of fenfluramine that could limit regulatory approval or commercialization, or that could result in recalls or product liability claims; the potential for the FDA to delay timing of review of the sNDA due to the FDA's internal resource constraints or other reasons; 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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