FINTEPLA as adjunctive therapy showed a significant, dose-dependent reduction in convulsive seizure frequency in Dravet syndrome patients versus placebo

EMERYVILLE, Calif., Dec. 17, 2019 (GLOBE NEWSWIRE) -- Zogenix, Inc. (NASDAQ: ZGNX), a global pharmaceutical company developing rare disease therapies, today announced that The Lancet has published the results of its Phase 3 clinical trial, Study 1, of the Company's investigational drug, FINTEPLA® (ZX008, fenfluramine oral solution), in children and young adults with Dravet syndrome, a rare, severe infantile-onset epilepsy characterized by frequent, disabling seizures that are very difficult to treat with existing anti-epileptic drugs. The study showed that both doses of FINTEPLA, when added to the patients' existing treatment regimens, provided a significant reduction in convulsive seizure frequency compared to placebo.

“The results from this study are tremendously encouraging in reducing the magnitude and duration of seizures in our patients with Dravet syndrome,” said Joseph Sullivan, M.D., Director of the Pediatric Epilepsy Center of Excellence at the UCSF Benioff Children’s Hospitals, co-lead author of the manuscript, and Principal Investigator for Study 1. “If future outcomes are as positive, it could help clinicians set new standards of care for a treatment-resistant disease like Dravet syndrome, in which frequent debilitating seizures and significant cognitive and functional impairments are the norm.”

Study 1 was an international, double blind, placebo-controlled Phase 3 study of 119 Dravet syndrome patients ages 2-18 years (mean age 9 years) treated at sites in the U.S., Canada, Europe and Australia. Patients were randomized to one of three treatment groups: FINTEPLA at 0.7 mg/kg/day with a 26 mg maximum daily dose* (n=40), FINTEPLA at 0.2 mg/kg/day (n=39), or placebo (n=40), which were added to the patient’s current antiepileptic drug regimen that excluded use of stiripentol. Following a 6-week baseline observation period, patients were titrated to their target dose over two weeks and remained at that dose for 12 weeks. The mean baseline convulsive seizure frequency across the study groups was approximately 40 seizures per month.

The primary endpoint was the change in mean monthly frequency of convulsive seizures during the treatment period compared with baseline in the 0.7 mg/kg/day group versus placebo. Results showed that patients taking FINTEPLA at 0.7 mg/kg/day achieved a 62.3% greater reduction in mean monthly convulsive seizure frequency compared to placebo (95% CI -47.7 to -72.8, p<0.0001). The same analysis for the group treated at 0.2 mg/kg/day versus placebo was a key secondary endpoint, with results showing a 32.4% greater reduction in mean monthly convulsive seizure frequency compared to placebo (95% CI -6.2 to -51.3, p=0.0209).

During the treatment period, the median reduction in seizure frequency was 74.9% in the 0.7 mg/kg/day group (from median 20.7 per 28 days to 4.7 per 28 days), 42.3% in the 0.2 mg/kg/day group (from median 17.5 per 28 days to 12.6 per 28 days), and 19.2% in the placebo group (from median 27.3 per 28 days to 22.0 per 28 days).

In addition to the seizure frequency data described above, more patients treated with FINTEPLA during the study achieved a clinically meaningful (≥50%) reduction in convulsive seizure frequency compared to placebo: 27 (68%) of 40 patients in the 0.7 mg/kg/day group (p<0.0001) and 15 (38%) of 39 patients in the 0.2 mg/kg/day group (p=0.0091), compared with five (12%) of 40 patients in the placebo group. FINTEPLA also provided significantly longer periods of seizure freedom to patients in the study: the median longest seizure-free intervals were 25 days in the 0.7 mg/kg/day group (p=0.0001) and 15 days in the 0.2 mg/kg/day group (p=0.0352), compared to 9.5 days in the placebo group.

The most common adverse events (occurring in at least 10% of patients, and more frequently in the FINTEPLA groups), were decreased appetite, diarrhea, fatigue, lethargy, somnolence and decreased weight. Echocardiographic examinations revealed normal valve function and morphology in all patients during the trial and no signs of pulmonary arterial hypertension.

“We are proud that the prestigious journal, The Lancet, has published these important results for the international medical community and very much appreciate the investigators, patients and families who made this study possible,” said Stephen J. Farr, Ph.D., President and Chief Executive Officer at Zogenix. “In addition, we are excited to see the continued signs of safety and efficacy of our investigational drug, FINTEPLA, used in Dravet syndrome patients over time in our ongoing open-label extension study and look forward to continuing to work with regulators to advance FINTEPLA as a potential new treatment option for patients suffering from Dravet syndrome.”

*Study 1 data results were previously presented by Zogenix at the December 2017 71st American Epilepsy Society (AES) Annual Meeting. In that and other presentations, Zogenix expressed doses of FINTEPLA (ZX008, fenfluramine) as doses of the HCl salt, with an upper limit of 0.8 mg/kg/day and 30 mg maximum daily dose. Due to current regulatory guidelines, Zogenix has chosen to express study doses as the fenfluramine base-equivalent, with an upper limit dosing of 0.7 mg/kg/day and 26 mg maximum daily
About Zogenix
Zogenix is a global pharmaceutical company committed to developing and commercializing transformative therapies to improve the lives of patients and their families living with rare diseases. The company has two late-stage development programs underway: FINTEPLA® (ZX008, fenfluramine oral solution) for the treatment of seizures associated with Dravet and Lennox-Gastaut syndromes, two rare and often-catastrophic childhood-onset epilepsies, and MT1621, a novel substrate enhancement therapy for the treatment of a rare genetic disorder called TK2 deficiency. Applications for Zogenix’s investigational drug, FINTEPLA, for Dravet syndrome are under review by the U.S. Food & Drug Administration and the European Medicines Agency. Zogenix expects top-line data from its Phase 3 study of FINTEPLA in Lennox-Gastaut syndrome in the first quarter of 2020. FINTEPLA is also in development in Japan.

Forward-Looking Statement
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 benefits FINTEPLA may have, if approved; the impacts that Dravet syndrome has on patients; and the potential timing of top-line data for Zogenix’s Phase 3 study of FINTEPLA in Lennox-Gastaut syndrome (Study 1601). 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 FDA may disagree that the existing safety and efficacy data is sufficient to approve the NDA of FINTEPLA for the treatment of Dravet syndrome; the FDA may require Zogenix to conduct the additional chronic toxicity studies noted in the Refusal to File letter, dated April 3, 2019, or other studies or information in connection with its review of the NDA; the timing of the data from Study 1601 of FINTEPLA in patients suffering from LGS could be delayed; the results of Study 1601 may differ from the results of prior clinical studies in LGS or may demonstrate adverse safety data compared to the prior Phase 3 clinical trials of FINTEPLA; top-line data the Company reports is based on preliminary analysis of key efficacy and safety data, and such data may change following a more comprehensive review of the data related to the clinical trial and such top-line data may not accurately reflect the complete results of a clinical trial, and the FDA may not agree with the Company’s interpretation of such results; later developments with the FDA that may be inconsistent with feedback received at prior meetings with the FDA; additional data from Zogenix’s ongoing studies may contradict or undermine the data submitted in the NDA for FINTEPLA; the uncertainties associated with the clinical development and regulatory approval of product candidates such as FINTEPLA and MT1621; unexpected adverse side effects or inadequate therapeutic efficacy of FINTEPLA that could limit approval and/or commercialization, or that could result in recalls or product liability claims; risks associated with the acquisition of Modis and integration of Modis’ operations into Zogenix’s business, including an increase in near and long-term expenditures, exposure to unknown liabilities and diversion of Zogenix’s management’s time and attention; 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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