Zogenix Phase 2 Study Results Published in Epilepsia Show ZX008 Provides Durable Reduction in Seizure Frequency in Patients With Lennox-Gastaut Syndrome

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67% of Patients Achieved At Least a 50% Reduction in Convulsive Seizures

Global Phase 3 Randomized, Controlled Study in LGS Currently Enrolling Patients

EMERYVILLE, Calif., Sept. 04, 2018 (GLOBE NEWSWIRE) -- Zogenix, Inc. (NASDAQ:ZGNX), a pharmaceutical company developing therapies for the treatment of rare central nervous system (CNS) disorders, today announced that detailed results of the Phase 2, open-label study evaluating its investigational drug, ZX008 (low-dose fenfluramine hydrochloride), for the treatment of refractory patients with Lennox-Gastaut Syndrome (LGS), were published in the September 2018 issue of *Epilepsia*. Consistent with the previously-reported data from this study, the results demonstrated that ZX008 provided sustained, clinically meaningful seizure reduction in the majority of patients and was generally well-tolerated.

“This study was designed to confirm the potential effectiveness of ZX008 for patients with LGS,” said Lieven Lagae, M.D., Ph.D., lead study author and professor at the University of Leuven, Belgium, head of the Pediatric Neurology Department and director of the Childhood Epilepsy Program at the University of Leuven Hospitals. “The clinically meaningful reduction in convulsive seizures in this highly refractory patient cohort emphasizes the importance of the ongoing Phase 3 study of ZX008 as a treatment for LGS patients, who continue to struggle with seizure control despite current therapies.”

The single-center, Phase 2, open-label dose-finding trial was a 20-week core study and a long-term extension option for those patients who were responders in the core study. Results presented in the paper included up to 15 months of treatment for patients in the long-term extension. Patients initiated treatment of 0.2 mg/kg/day ZX008 twice-daily. Patients who were responders (i.e., achieved > 50% reduction in convulsive seizure frequency after 4 weeks) remained at their effective dose, while non-responders were considered for a dose increase up to 0.8 mg/kg/day at 0.2 mg/kg increments per 4 weeks.

Patients enrolled in the study (n=13) had refractory LGS and a baseline median seizure frequency of 61 per month (range: 21-1360) with multiple seizure types (defined as tonic, generalized tonic-clonic, myoclonic, absence, and atonic seizures/drop attacks). Patients failed a median of five anti-epileptic treatments, including vagus nerve stimulation and ketogenic diet (range: 3-7). Patients achieved a 53% median reduction in convulsive seizure frequency during the 20-week treatment period of the core study. A reduction in convulsive seizure frequency of at least 50% was seen in 62% of patients, with a reduction of at least 75% being reported in 23% of patients. The median dose of ZX008 was 0.4 mg/kg/day.

After 15 months in the long-term extension study (n=9), patients achieved a 58% median reduction in convulsive seizure frequency over the entire treatment period compared to baseline. Of these patients, 67% achieved at least a 50% reduction, and 33% achieved at least a 75% reduction in convulsive seizure frequency. At 15 months, the median dose of ZX008 was 0.4 mg/kg/day.

“The compelling results of this study supported commencing a global Phase 3 pivotal trial to evaluate the efficacy and safety of ZX008 for the treatment of seizures associated with LGS,” said Bradley S. Galer, M.D., executive vice president and chief medical officer at Zogenix. “We are especially pleased with the long-term robustness of seizure control given the highly refractory nature of these patients. We believe the publication of these data underscores the significance of these findings for this patient population with a major unmet medical need.”

ZX008 was generally well-tolerated in this study and demonstrated a safety profile consistent with the findings of completed Phase 3 studies of ZX008 in patients with Dravet syndrome. No patient exhibited cardiac valvulopathy or pulmonary hypertension at any time in the study. The most common adverse events were decreased appetite (n=4; 31%) and decreased alertness (n=2; 15%).

LGS is a rare, severe form of epilepsy, marked by frequent and prolonged seizures, with peak onset between ages 3 and 5.[1] LGS impacts approximately 30,000 – 50,000 people in the U.S. and Europe, many of whom are children. While current treatments exist, patients continue to struggle to achieve seizure control.[2]

ZX008 for the treatment of LGS has previously been designated as an orphan drug by both the U.S. Food and Drug Administration and the European Commission. The Phase 3 trial is currently enrolling at sites in North America, Europe and Australia. For additional information, including inclusion/exclusion criteria, please visit https://clinicaltrials.gov, using NCT Identifier # 03355209. ZX008, fenfluramine HCl oral solution, is an investigational compound that is being developed as a potential treatment for seizures in patients with Dravet syndrome and Lennox-Gastaut syndrome. It is currently not approved by any regulatory authority to treat any condition.

About Zogenix
Zogenix, Inc. (Nasdaq:ZGNX) is a pharmaceutical company dedicated to developing therapies for people living with severe central nervous system (CNS) disorders who have limited or no treatment options. Led by a team of experts in rare disease development and CNS conditions, Zogenix is rapidly advancing the clinical investigation and development of ZX008 (low-dose fenfluramine hydrochloride) for patients with severe, rare epilepsies, including Dravet and Lennox-Gastaut syndromes.

For more information, visit www.zogenix.com.

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 are based on the Zogenix's current beliefs and expectations. These forward-looking statements include statements regarding the need for additional therapies for LGS patients; ZX008’s potential to be an effective treatment for LGS; and the timing of enrollment of patients in the ongoing Phase 3 clinical trial of patients with LGS; and Zogenix’s expectations that the Phase 3 clinical trial of patients with LGS will serve as the pivotal trial for submitting applications for regulatory approvals of ZX008. 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 on-going open label nature of the clinical trial of patients with LGS will continue to generate efficacy and safety data, and such data may change compared to the announced data; the FDA may not agree with Zogenix’s interpretation of the results of our clinical trial data; unexpected adverse side effects or inadequate therapeutic efficacy of ZX008 may limit regulatory approval and/or commercialization, or may 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 Securities and 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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