ZOGENIX

Zogenix Announces Positive Top-line Results from Global Pivotal Phase 3 Trial of FINTEPLA® for the Treatment of Lennox-Gastaut Syndrome

- *Primary Endpoint Achieved - Statistically Significant Reduction in Drop Seizures for FINTEPLA 0.7 mg/kg/day versus Placebo*
- *FINTEPLA Also Demonstrated Statistically Significant Improvement on Multiple Secondary Endpoints*
- *Zogenix to Host Conference Call and Live Webcast Today at 4:30 PM Eastern Time/1:30 PM Pacific Time*

EMERYVILLE, California, February 6, 2020 – Zogenix, Inc. (NASDAQ: ZGNX), a global pharmaceutical company developing rare disease therapies, today reported positive top-line results from its global Phase 3 clinical trial (Study 1601) of its lead investigational therapy, FINTEPLA® (ZX008, fenfluramine oral solution) in Lennox-Gastaut Syndrome (LGS), a severe and treatment-resistant childhood-onset epilepsy. The trial met its primary objective of demonstrating that FINTEPLA at a dose of 0.7 mg/kg/day was superior to placebo in reducing the frequency of drop seizures, based on the change between baseline and the titration and maintenance treatment period (p=0.0012). The same dose of FINTEPLA (0.7 mg/kg/day) also demonstrated statistically significant improvements versus placebo in key secondary efficacy measures, including the proportion of patients with a clinically meaningful reduction (≥50%) in drop seizure frequency. A decrease in the frequency of drop seizures between baseline and the treatment period was observed for a lower dose of FINTEPLA (0.2 mg/kg/day) compared to placebo, but this change did not reach statistical significance (p=0.0915). FINTEPLA was generally well-tolerated, with the adverse events consistent with those observed in the Company's two prior Phase 3 studies in Dravet syndrome.

"LGS is a rare and severe form of epilepsy where nearly all patients have highly treatment resistant and lifelong seizures. As a result, the frequent falls and injuries, and also the cognitive impairment, limit the quality of life for patients and caregivers, even with current treatment options," said Associate Professor Kelly Knupp, M.D., MSCS, FAES of Children's Hospital Colorado, Principal Investigator for Study 1601. "The results observed in this placebo-controlled study are indicative of the potential of fenfluramine to treat patients with refractory LGS. If approved, FINTEPLA could represent an important new treatment option for these patients and their families in need."

Study Design

The Phase 3 multicenter, global LGS trial has two parts: Part 1 was a double-blind, placebo-controlled study to assess the safety, tolerability and efficacy of FINTEPLA when added to a patient's current anti-epileptic regimen. The study included a total of 263 patients between the ages of 2 and 35 years whose seizures were currently uncontrolled while on one or more anti-epileptic drugs (AEDs), randomized into three treatment groups: FINTEPLA 0.7 mg/kg/day (26 mg maximum daily dose; n=87), FINTEPLA 0.2 mg/kg/day (n=89), and placebo (n=87). The median age of patients was 13 years, with 29% being 18 years or older. Patients entering the study were taking between one and four AEDs and previously had tried and discontinued an average of seven other AEDs. The median baseline drop seizure frequency across the study groups was 77 seizures per month. After establishing baseline seizure frequency for 4 weeks, randomized patients were titrated to their dose over a 2-week titration period, followed by a 12-week fixed dose maintenance period. Patients who completed Part 1 were eligible to enter

Part 2 of the clinical trial, an ongoing 12-month open-label extension study to evaluate the long-term safety, tolerability and effectiveness of FINTEPLA.

Results

Study 1601 met its primary endpoint of showing a highly statistically significant reduction from baseline compared to placebo in the median percent change in monthly drop seizure frequency. Patients taking FINTEPLA 0.7 mg/kg/day achieved a median reduction of 26.5% compared to a median reduction of 7.8% in patients taking placebo (p=0.0012). Using a parametric analysis, patients taking FINTEPLA 0.7 mg/kg/day demonstrated a 26.5% greater reduction in mean monthly drop seizure frequency compared to placebo (p=0.0034). The median percent reduction in monthly drop seizures between baseline and the treatment period for the lower study dose of FINTEPLA (0.2 mg/kg/day), a secondary endpoint, was 13.2% and did not reach statistical significance compared to placebo (p=0.0915).

Additional secondary endpoints of the study were to compare the proportion of study patients treated with FINTEPLA 0.7 mg/kg/day who achieved a ≥50% reduction in monthly drop seizures versus placebo and to compare Clinical Global Impression of Improvement ratings (CGI-I, a measure of improvement of worsening relative to baseline) as assessed by the investigator. Results are shown in the following table:

	FINTEPLA 0.7 mg/kg/day (N=87)	Placebo (N=87)
Patients with ≥50% reduction in monthly drop seizures (T+M Period)	25.3% (p=0.0165)[1]	10.3%
CGI-I (Proportion of Patients Improved)	48.8% (p=0.0567)[1]	33.8%
CGI-I (Proportion of Patients Much Improved or Very Much Improved)	26.3% (p=0.0007)[1]	6.3%

[1]P-values versus Placebo

FINTEPLA was generally well-tolerated in this study, with the adverse events consistent with those observed in the Company's two prior Phase 3 studies in Dravet syndrome. The incidence of patients who experienced at least one adverse event was 89.7% of patients in the FINTEPLA 0.7 mg/kg/day group, 76.4% in the FINTEPLA 0.2 mg/kg/day group and 79.3% in the placebo group. The most common adverse events (≥10%) in the FINTEPLA-treated groups were decreased appetite, somnolence, fatigue, vomiting, diarrhea, and pyrexia. The incidence of serious adverse events was 11.5% (n=10) in the 0.7 mg/kg/day group, 4.5% (n=4) in the 0.2 mg/kg/day group, and 4.6% (n=4) in the placebo group. Six patients in the 0.7 mg/kg/day group had an adverse event leading to study discontinuation compared to four subjects in the 0.2 mg/kg/day group and one patient in the placebo group; the majority of these were considered treatment-related. There was one death during the trial (0.7 mg/kg/day group) caused by SUDEP (sudden unexpected death in epilepsy), which was assessed by the investigator to be unrelated to the study drug.

No cases of valvular heart disease or pulmonary hypertension have been observed in Study 1601, including both Part 1 and Part 2. A total of 247 (93.9%) patients entered the open-label extension phase.

“On behalf of everyone at Zogenix, I would like to thank the patients, families and investigators who gave their time and effort to participate in this study,” said Stephen J. Farr, Ph.D., President and CEO of Zogenix. “We are pleased with the top-line efficacy and safety results from Study 1601, which highlight FINTEPLA’s potential to be an important new treatment option for one of the most difficult to treat rare epilepsies. We look forward to working with regulatory agencies to potentially bring FINTEPLA to the LGS patient community.”

FINTEPLA for the treatment of LGS has previously been designated as an orphan drug by both the U.S. Food and Drug Administration (FDA) and the European Commission.

The Company’s New Drug Application (NDA) for FINTEPLA for the treatment of seizures associated with Dravet syndrome is under Priority Review by the FDA, with a PDUFA (Prescription Drug User Fee Act) target action date of March 25, 2020. In addition, a Marketing Authorization Application (MAA) for FINTEPLA in Dravet syndrome is under review by the European Medicines Agency. The NDA and MAA are based on data from two pivotal Phase 3 trials (Studies 1 and 1504) of FINTEPLA in Dravet syndrome and an interim analysis from an ongoing open-label extension study, which included 232 patients treated for up to 21 months. FINTEPLA is also under development in Japan.

Conference Call
Zogenix will host a conference call and webcast to discuss the results from Study 1601 today, February 6, 2020, at 4:30 PM Eastern Time. Details to participate in the call are below.

Conference Call Details	
4:30 PM Eastern Time / 1:30 PM Pacific Time	
Toll Free:	1-877-407-9716
International:	1-201-493-6779
Conference ID:	13698945
Webcast (with slides):	http://public.viavid.com/index.php?id=138046

About Lennox-Gastaut Syndrome
Lennox-Gastaut Syndrome (LGS) is a rare and devastating form of childhood-onset epilepsy characterized by many different seizure types, which often don't respond to currently available seizure medications (also known as anti-epileptic drugs, or AEDs). According to the Epilepsy Foundation, LGS accounts for only 2-5% of childhood epilepsies, yet LGS patients are well known to both pediatric and adult neurologists because their seizures are hard to control, and they need life-long treatment. 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 pharmaceutical 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 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.

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 that FINTEPLA, if approved, will be an important new treatment option for LGS patients;; and the timing and results of any decision regarding the NDA or MAA for FINTEPLA for the treatment of seizures associated with Dravet syndrome. 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: 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; the potential for the FDA to delay the PDUFA target action date related to the Dravet syndrome NDA due to the FDA's internal resource constraints or other reasons; additional data from Zogenix’s ongoing studies may contradict or undermine the data submitted in the Dravet syndrome NDA for FINTEPLA or reported for LGS; 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; 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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