Zogenix Announces Positive Top-line Results from Pivotal Phase 3 Clinical Trial of ZX008 in Dravet Syndrome

Primary Endpoint Achieved - Statistically Significant Convulsive Seizure Reduction for ZX008 versus Placebo for Adjunctive Treatment of Seizures

ZX008 Also Demonstrated Statistical Significance in All Key Secondary Endpoints

Zogenix to Host Conference Call Today at 8:30 AM Eastern Time/5:30 AM Pacific Time

EMERYVILLE, California, September 29, 2017 – Zogenix, Inc. (NASDAQ: ZGNX), a pharmaceutical company developing therapies for the treatment of rare central nervous system (CNS) disorders, today reported positive top-line results from its first Phase 3 trial (Study 1) for its investigational drug, ZX008 (low-dose fenfluramine hydrochloride), for the treatment of Dravet syndrome. The trial met its primary objective of demonstrating that ZX008, at a dose of 0.8 mg/kg/day, is superior to placebo as adjunctive therapy in the treatment of Dravet syndrome in children and young adults based on change in the frequency of convulsive seizures between the 6-week baseline observation period and the 14-week treatment period ($p<0.001$). ZX008 0.8 mg/kg/day also demonstrated statistically significant improvements versus placebo in all key secondary measures, including the proportion of patients with clinically meaningful reductions in seizure frequency and longest seizure-free interval. The same analyses comparing a 0.2 mg/kg/day ZX008 dose versus placebo also demonstrated statistically significant improvement compared with placebo.

“Dravet syndrome is a rare, but catastrophic form of epilepsy that can be devastating for patients and their families,” said Joseph Sullivan M.D., director of the Pediatric Epilepsy Center in UCSF Benioff Children’s Hospital San Francisco, and Principal Investigator of Study 1 in the U.S. “These results are truly exciting and demonstrate, in a large multicenter controlled trial, the impressive efficacy of low-dose fenfluramine for patients with Dravet syndrome. If approved, ZX008 could play an important role in treating this devastating condition.”

The randomized, double blind, placebo controlled, Phase 3 study enrolled 119 patients across sites in the United States, Canada, Europe, and Australia. The median age of patients was 8 years (range, 2-18 years). Following a six-week baseline observation period, patients were randomized to one of three treatment groups: ZX008 0.8 mg/kg/day (30 mg maximum daily dose; n=40), ZX008 0.2 mg/kg/day (n=39) and placebo (n=40) in which ZX008 or placebo was added to current regimens of antiepileptic drugs. Patients were titrated to their target dose over two weeks and then remained at that fixed dose for 12 weeks. The mean baseline convulsive seizure frequency across the study groups was approximately 40 seizures per month.

The primary efficacy measure was a comparison of the change in mean monthly convulsive seizure frequency between ZX008 0.8 mg/kg/day and placebo during the 14-week treatment period compared with the 6-week baseline observation period. Patients taking ZX008 0.8 mg/kg/day achieved a 63.9%

reduction in mean monthly convulsive seizures compared to placebo (p<0.001). The median percent reduction in monthly convulsive seizure frequency was 72.4% among ZX008 0.8 mg/kg/day patients compared to 17.4% in placebo patients.

A key secondary endpoint was the same analysis for a comparison of ZX008 0.2 mg/kg/day and placebo. Patients taking ZX008 0.2 mg/kg/day achieved a reduction in mean monthly convulsive seizures of 33.7% compared to placebo (p=0.019). Collectively, these top-line data suggest a dose-response relationship for ZX008 in the adjunctive treatment of convulsive seizures in Dravet syndrome.

Additional key secondary objectives of the study were to compare 0.8 mg/kg/day and 0.2 mg/kg/day ZX008 (independently) with placebo in terms of (1) the proportion of patients who achieved ≥50% reductions in monthly convulsive seizures and (2) the median of the longest convulsive seizure-free interval. These results are shown in the following table. The proportion of patients who achieved ≥75% seizure reductions, a secondary efficacy measure, is also presented.

	ZX008 0.8 mg/kg/day (N=40)	ZX008 0.2 mg/kg/day (N=39)	Placebo (N=40)
Patients with ≥50% reduction in monthly convulsive seizures	70.0% (p<0.001)	41.0% (p=0.001)	7.5%
Patients with ≥75% reduction in monthly convulsive seizures	45.0% (p=0.001)	20.5% (p=0.033)	2.5%
Longest seizure-free interval (median)	20.5 days (p<0.001)	14 days (p=0.011)	9 days

ZX008 was generally well-tolerated in this study with the adverse events consistent with the known safety profile of fenfluramine. The incidence of treatment emergent adverse events was higher in the treatment groups as compared to the placebo group, with 95% (n=38) of patients in the 0.8mg/kg/day group and 94.9% (n=37) of patients in the 0.2 mg/kg/day group experiencing at least one treatment emergent adverse event compared to 65.0% (n=26) of patients in the placebo group. The incidence of serious adverse events was similar in all three groups with 12.5% (n=5) of patients in the 0.8 mg/kg/day group and 10.3% (n=4) of patients in the 0.2 mg/kg/day group experiencing at least one treatment emergent serious adverse event compared to 10.0% (n=4) of patients in the placebo group. Five patients in the 0.8 mg/kg/day group had an adverse event leading to study discontinuation compared to none in the other treatment groups. Prospective cardiac safety monitoring throughout the study demonstrated no clinical or echocardiographic evidence of cardiac valvulopathy or pulmonary hypertension.

"On behalf of everyone at Zogenix, I would like to extend my sincere appreciation to the patients, families and investigators involved in this study. We are extremely pleased with the top-line efficacy and safety results from Study 1 which reinforce the potential of ZX008 to be an important new treatment for seizure control in children with Dravet syndrome. We look forward to presenting further data in future publications and at medical conferences," said Stephen J. Farr, Ph.D., President and CEO of Zogenix. "We expect top-line results from our second pivotal Phase 3 trial, Study 1504, which is nearing full enrollment, in the first half of 2018. We remain on track to submit applications for regulatory approvals in the U.S. and Europe in the second half of 2018."

ZX008 is designated as an orphan drug in both the U.S. and Europe, and has received Fast Track designation in the U.S. for the treatment of Dravet syndrome.

Conference Call Details
Friday, September 29 @ 8:30 AM Eastern Time/5:30 AM Pacific Time

Toll Free:	888-455-2265
International:	719-457-2627
Conference ID:	7770264
Webcast:	http://public.viavid.com/index.php?id=126480

Audio Replays, available through October 13, 2017:

Domestic:	844-512-2921
International:	412-317-6671
Replay PIN:	7770264

About Zogenix

Zogenix (Nasdaq: ZGNX) is focused on developing therapies for patients with rare central nervous system (CNS) conditions that have limited or no treatment options but face a critical need. For more information, visit www.zogenix.com.

About the ZX008 Dravet Syndrome Global Development Program

Study 1 is a prospective merged analysis of two identical double-blind, placebo-controlled studies, ZX008-1501 (US/Canada) and ZX008-1502 (Europe/Australia), designed to enroll approximately 40 patients per treatment group and includes patients from sites in the United States, Canada, Europe, and Australia. A total of 119 patients were randomized into one of two dose groups of ZX008, 0.2 and 0.8 milligrams per kilogram per day, or placebo. After a six-week baseline period, randomized subjects were titrated to their target dose over two weeks, and then held to that fixed dose for 12 weeks of maintenance treatment. The primary objective of the study was to demonstrate that ZX008 at the high dose is superior to placebo in controlling seizures based on the change in the frequency of convulsive seizures between baseline and the combined titration and maintenance periods. The percent of patients who achieve equal or greater than a 50% reduction in convulsive seizures, and measurement of the longest seizure-free interval, were key secondary endpoints. Patients who complete Study 1 are eligible to enter a long-term, open-label extension study.

Zogenix is conducting a second double-blind, randomized, two-arm pivotal Phase 3 trial, Study 1504, in which all patients will be taking stiripentol, valproate and clobazam as part of their baseline standard care. In February 2017, the Company announced the initiation of the safety and efficacy portion of Study 1504, which compares a single dose of ZX008 versus placebo across the titration and 12-week maintenance periods. Study 1504 will enroll 40 patients per treatment group.

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 Company's current beliefs and expectations. These forward-looking statements include statements regarding ZX008's potential as a treatment for seizures associated with Dravet syndrome; the timing of topline results from Study 1504; and regulatory submission timelines for 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 top-line data Zogenix has reported 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 the trial, and the FDA may not agree with Zogenix's interpretation of such results; the uncertainties associated with the clinical development and regulatory approval of product candidates such as ZX008, including potential delays in the enrollment and completion of clinical trials; the potential that earlier clinical trials and studies may not be predictive of future results; Zogenix's reliance on third parties to conduct its clinical trials, enroll patients, manufacture its preclinical and clinical drug supplies; unexpected adverse side effects or inadequate therapeutic efficacy of ZX008 that could limit approval and/or commercialization, or that could result in recalls or product liability claims; Zogenix's ability to fully comply with numerous federal, state and local laws and regulatory requirements, as well as rules and regulations outside the United States, that apply to its product development activities; 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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