Zogenix Announces Initiation of Clinical Efficacy Portion of Study 1504 for ZX008 in Dravet Syndrome

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Study 1504 Part of Phase 3 Program for ZX008 in Dravet Syndrome

Safety and Efficacy Portion of Study Expanded from EU to Include Sites in U.S. and Canada

EMERYVILLE, Calif., Feb. 13, 2017 (GLOBE NEWSWIRE) -- Zogenix, Inc. (NASDAQ:ZGNX), a pharmaceutical company developing therapies for the treatment of orphan and central nervous system (CNS) disorders, today announced the initiation of the clinical efficacy portion of Study 1504 for ZX008 in patients with Dravet syndrome. Study 1504 is a multi-center, two-cohort study to initially assess the pharmacokinetic and safety profile of single doses of ZX008 (fenfluramine hydrochloride), clobazam and valproate when added to stiripentol, followed by a randomized, double-blind, placebo-controlled parallel group evaluation of the efficacy, safety and tolerability of ZX008 as adjunctive antiepileptic therapy to a drug regimen that includes stiripentol, clobazam and valproate. Stiripentol in combination with clobazam and valproate is approved for the treatment of seizures in Dravet syndrome in Europe, Canada, Australia, and Japan. Although stiripentol is not approved in the United States (U.S.), the U.S. Food and Drug Administration (FDA) permits the use of this drug through its Expanded Access regulations and it is a current treatment option for many patients with Dravet syndrome in the U.S.

The primary purpose of Cohort 1 was to identify the appropriate ZX008 dose to then be used in Cohort 2, since the combination of stiripentol and clobazam is expected to impact fenfluramine pharmacokinetics through the inhibition of enzymes responsible for fenfluramine's metabolism. Based on the outcome of the completed pharmacokinetic analysis from Cohort 1, the dose of ZX008 being utilized in Cohort 2 is 0.5 milligrams per kilogram per day, which results in equivalent steady-state exposure to a dose of 0.8 milligrams per kilogram per day in patients whose background medications do not include the combination of stiripentol and clobazam. This dose (0.8 mg/kg/day) is the high dose of ZX008 currently being evaluated in Studies 1501 and 1502, which are two identical ongoing double-blind randomized placebo-controlled studies that are also being conducted as part of Zogenix's Phase 3 program for ZX008 in patients with Dravet syndrome. The two treatment groups in Cohort 2 of Study 1504 will have either ZX008 or placebo added to their stable stiripentol drug regimen. The duration of maintenance treatment is 12 weeks, the same duration as in Studies 1501 and 1502, and the seizure endpoints are also the same, including the primary outcome measure of change from baseline in frequency of convulsive seizures.

Study 1504 was initially to be conducted only in Europe, but was recently expanded to include sites in the U.S. and Canada due to significant investigator interest as they consider stiripentol an important medication in the available treatment armamentarium for seizure control in Dravet syndrome. Importantly, Study 1504 meets the requirements of a Phase 3 pivotal trial in the U.S.

"We are pleased to initiate the efficacy and safety portion of this important Phase 3 study," said Professor Rima Nabbout, M.D. Ph.D., Department of Pediatric Neurology, Reference Center for Rare Epilepsies, Necker Enfants Malades Hospital, and Principal Investigator of Study 1504. "There remains a significant unmet need for improved treatments for Dravet syndrome, and based on the data garnered to date, ZX008 has the potential to address this treatment need for refractory patients. Based on the number of patients in Europe currently on a stiripentol regimen, we expect this trial to enroll quickly, and look forward to the availability of data later this year."

"The initiation of the efficacy portion of Study 1504 represents another key milestone in Zogenix's ZX008 Dravet syndrome Phase 3 program," said Stephen J. Farr, Ph.D., President and CEO. "Study 1504 is important to our Phase 3 development program for several reasons. Most importantly, because it meets the requirements of a pivotal trial, we have increased optionality around our regulatory strategy for ZX008. In addition, Study 1504 will be used to support orphan exclusivity in Europe."

"We are excited to have the opportunity to participate in this study in the U.S.," said Kelly Knupp, M.D., Associate Professor, Pediatrics – Neurology and epileptologist at Children's Hospital Colorado and University of Colorado School of Medicine. "We have seen a significant amount of interest from Dravet families in participating in this study, and look forward to evaluating ZX008 in this underserved patient population. I especially am pleased to assess a potential new medication, ZX008, with the stiripentol regimen that is used in children with Dravet syndrome in our clinic."

About Zogenix
Zogenix, Inc. (Nasdaq:ZGNX) is a pharmaceutical company committed to developing and commercializing CNS therapies that address specific clinical needs for people living with orphan and other CNS disorders who need innovative treatment alternatives to improve their daily functioning.

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 company's current beliefs and expectations. These forward-looking statements include statements regarding the structure of the Study 1504 clinical trial, including the number of patients to be enrolled; the ability to use Study 1504 as a pivotal trial for submission to the U.S. Food and Drug Administration, the timing of top-line results from the Phase 3 clinical trial of ZX008 in Dravet syndrome; the ability of ZX008 to treat patients with Dravet syndrome; the potential commercialization of ZX008; and the timing of any submission of a new drug application to the U.S. Food and Drug Administration or comparable market authorization filing in Europe. 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 uncertainties associated with the clinical development and regulatory approval of product candidates such as ZX008, including potential delays in the commencement, enrollment and completion of clinical trials, including Study 1504; 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 and manufacture commercial supplies of its drug products, if approved; 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; Fast Track designation may not result in an expedited regulatory review process; the potential for distraction of management related to the transition of management responsibilities; 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.

CONTACT:
Investors: Andrew McDonald

Founding Partner, LifeSci Advisors LLC
646-597-6987 | Andrew@lifesciadvisors.com

Primary Logo

Zogenix, Inc.