Zogenix Announces New Efficacy and Safety Data on ZX008 for Treatment of Seizures in Dravet Syndrome

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New Clinical Data Presented at 69th Annual American Epilepsy Society Meeting on the Use of Low-Dose Fenfluramine in Managing Seizures Associated With Dravet Syndrome

SAN DIEGO, Dec. 7, 2015 (GLOBE NEWSWIRE) -- Zogenix, Inc. (Nasdaq:ZGNX), a pharmaceutical company developing therapies for the treatment of central nervous system (CNS) disorders, today announced new data demonstrating sustained effectiveness and cardiovascular-related safety for patients treated with ZX008 (low-dose fenfluramine) as an adjunctive therapy for seizures associated with Dravet syndrome. The data were presented at the 69th Annual American Epilepsy Society Meeting, taking place this week in Philadelphia, PA (see study data here and here). Zogenix expects to initiate a Phase 3 program for ZX008 in 2015. ZX008 is designated as an orphan drug in both the United States and Europe for the treatment of seizures associated with Dravet syndrome.

"In this new cohort of patients, we continue to achieve meaningful seizure control in Dravet syndrome patients using low-dose fenfluramine as adjunctive treatment," said Professor Berten Ceulemans, from the University of Antwerp, Belgium, and one of the authors of the poster regarding the study. "The most recent data corroborate both the effectiveness and the safety observations demonstrated in the original cohort of patients, which were published in 2012. We look forward to the start of Zogenix's ZX008 Phase 3 program evaluating low-dose fenfluramine as a potential adjunctive treatment for seizures associated with Dravet syndrome."

"The improvement in seizures and cardiovascular-related safety data from this ongoing open-label study continues to be quite compelling," said Bradley Galer, M.D., Chief Medical Officer of Zogenix. "These data further support ZX008's potential as a safe and effective adjunct treatment for uncontrolled seizures associated with Dravet syndrome. Dravet syndrome patients and their families remain in need of viable treatment alternatives for this devastating condition that causes frequent, severe and potentially life-threatening seizures that are often unresponsive to standard anti-epileptic medications. As such, we remain focused on advancing our preparations for the start of our Phase 3 program for ZX008, which we expect to initiate in 2015."

The data presented highlight the initial results from a new cohort of 7 Dravet syndrome patients who began add-on treatment with low-dose fenfluramine (5 mg to 15 mg per day) at various starting points between 2010 and 2014. Median treatment duration was 0.9 years (range 0.2 to 3.9 years). During the 90-day run-in period prior to initiating low-dose fenfluramine treatment, the median frequency of tonic-clonic seizures was 3.0 per month (range 0.4 to 39.7). At the 6-month evaluation after starting low-dose fenfluramine treatment, the median frequency of tonic-clonic seizures was 1.2 per month, and the median decrease was 73% (range 48-100%). Over the entire observation period, the median frequency of tonic-clonic seizures was 0.9 per month, and the median decrease was 84% (range 55-100%).

A separate poster reported that during this observation period from 2010 to 2015, treatment with low-dose fenfluramine was generally well-tolerated, and in this new cohort of patients, treatment for periods of 0.9 to 3.9 years did not result in any echocardiographic or clinical signs of cardiac valve abnormalities, pulmonary hypertension or any other cardiovascular abnormalities. The most common treatment-related adverse events were mild-to-moderate somnolence (n=6) and anorexia (n=4). There were no fenfluramine discontinuations due to adverse events or lack of effect.

The observed effectiveness, tolerability and cardiovascular-related safety with add-on, low-dose fenfluramine in this new cohort of Dravet syndrome patients extends the findings previously reported in the original cohort in 2012[1].

In addition, a separate, recently published study (see study data here) evaluated the mechanism of action for fenfluramine as a treatment for Dravet syndrome using a gene knockout zebrafish model. As a result of this study, certain 5-$HT_{subtype}$ receptors that appear to be involved in the mechanism-of-action of fenfluramine were identified. Specifically, the elevation of serotonin levels and interaction with three 5-HT receptor subtypes, 5-HT1D, 5-HT2A and 5-HT2C, were found to be responsible for reducing both abnormal motor behavior and brain activity in this model of Dravet syndrome.

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.

About Dravet Syndrome

Dravet syndrome (also known as Severe Myoclonic Epilepsy of Infancy) is a rare, severe and therapy-resistant form of epilepsy most often caused by an identifiable gene defect that results in abnormal functioning of a sodium channel in the brain. Children with Dravet syndrome experience severe, long-lasting, fever-related seizures in the first year of life. Other seizures typically arise later, including myoclonus (involuntary muscle spasms) and status epilepticus (prolonged seizures), which often result in severe cognitive and developmental impairment. Episodes of status epilepticus require immediate emergency care and can be fatal.

Individuals with Dravet syndrome face a higher incidence of SUDEP (sudden unexplained death in epilepsy) and have associated conditions, which also require proper treatment and management. Children with Dravet syndrome do not outgrow this condition and it affects every aspect of their daily lives.

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 timing of the commencement of Phase 3 clinical studies for ZX008 and ZX008's potential as a safe and effective adjunct treatment for uncontrolled seizures associated with Dravet syndrome. 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; 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; 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.

[1]Ceulemans, Berten, et al. Successful use of fenfluramine as an add-on treatment for Dravet syndrome. EPILEPSIA. July 2012; 53(7):1131-1139.

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

Zogenix, Inc.