Zogenix Receives Orphan Drug Designation from FDA for ZX008 in Treatment of Lennox Gastaut Syndrome

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EMERYVILLE, Calif., June 22, 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 U.S. Food & Drug Administration (FDA) has granted its investigational drug, ZX008 (low-dose fenfluramine), orphan drug designation for the treatment of Lennox Gastaut Syndrome (LGS), a refractory, debilitating childhood-onset epilepsy. The Orphan Drug Designation by the FDA follows the European Union's granting of Orphan Drug Designation for ZX008 in the treatment of LGS earlier this year.

"The receipt of Orphan Drug Designation in the treatment of LGS represents another significant milestone for our ZX008 development program," said Stephen J. Farr, Ph.D., President and Chief Executive Officer of Zogenix. "The Investigational New Drug (IND) Application for our Phase 3 LGS study was approved to proceed by the FDA in April. We intend to initiate this clinical trial in the second half of 2017, following the availability of top-line Phase 3 data in our initial indication, Dravet syndrome. We expect that the first patients will enroll in the planned LGS study in the fourth quarter of 2017."

Under the U.S. Orphan Drug Act, FDA's Office of Orphan Products Development grants Orphan Drug Designation to investigational drugs and biologics that are intended for the treatment of rare diseases that affect fewer than 200,000 people in the U.S. Orphan drug status is intended to facilitate drug development for rare diseases and may provide several benefits to drug developers, including assistance with clinical study design and drug development, tax credits for qualified clinical trials costs, exemptions from certain FDA application fees, and seven years of market exclusivity upon regulatory product approval.

Zogenix is currently conducting a Phase 3 program in the U.S. and internationally for ZX008 in Dravet syndrome, another intractable, severe epilepsy that begins in infancy. The last patient in Zogenix's first Phase 3 pivotal study was randomized in April 2017, and top-line data are expected in the third quarter of 2017. ZX008 for the treatment of Dravet syndrome has orphan designation in both the U.S. and Europe, and the development program has received Fast Track designation in the U.S.

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 ZX008's potential as a treatment for seizures associated with LGS and Dravet syndrome; the timing of the initiation the Phase 3 clinical trial in LGS during the second half of 2017 and the timing of the first patients to enroll in such study; the timing of top line results for the on-going Phase 3 clinical trials in Dravet syndrome; and the expected benefits associated with orphan drug designation. 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: risks that the benefits associated with orphan drug designation may not be realized, including that orphan drug exclusivity may not effectively protect a product from competition and that such exclusivity may not be maintained; 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; top-line data from the Phase 3 clinical trials of ZX008 in Dravet syndrome may not support our NDA for ZX008 in Dravet syndrome; negative top-line data from the ongoing Phase 3 clinical trials may delay or prevent commencement of the Phase 3 clinical trial in LGS; 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; 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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