



Company Presentation

February 2018
LEERINK Annual Global Healthcare
Conference



Forward Looking Statement



Zogenix cautions you that statements included in this presentation 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 and Lennox Gastaut Syndrome (LGS); the timing of results from ongoing and planned clinical trials; the timing and development plan for the Phase 3 clinical program for LGS; regulatory submission timelines for ZX008; the potential commercialization of ZX008; and Zogenix's financial position. 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 presentation due to the risks and uncertainties inherent in Zogenix's business, including, without limitation: the top-line data Zogenix reports from time to time 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 and regulatory submissions; 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, if approved, its commercial 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; Fast Track designation may not result in an expedited regulatory review process; Zogenix's cash burn rate may be greater than anticipated; and other risks described in Zogenix's press releases as well as in public periodic filings with the Securities and Exchange Commission.

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Highly Focused on Developing and Commercializing Therapies for
RARE CNS DISORDERS

ZX008 (Low Dose Fenfluramine) for the
Treatment of Seizures in Severe Childhood
Onset Epileptic Encephalopathies

DRAVET SYNDROME

**LENNOX GASTAUT
SYNDROME**

Expanding global IP portfolio including 4 Issued US Patents
Orphan Drug Designation in US and EU
Global Rights Retained

Recent Milestones

Complete first Phase 3 study for ZX008 in Dravet syndrome



Highly significant efficacy results in Study 1 (Sep 2017)
FDA Breakthrough Therapy Designation (Feb 2018)

Initiate and complete enrollment for second Phase 3 study in Dravet



Study 1504 last patient randomized (Jan 2017)
1504 Top-line data forthcoming (Q2 2018)

Strengthen and Secure Financial Position



~\$290M (gross) Secondary Offering (Oct 2017)

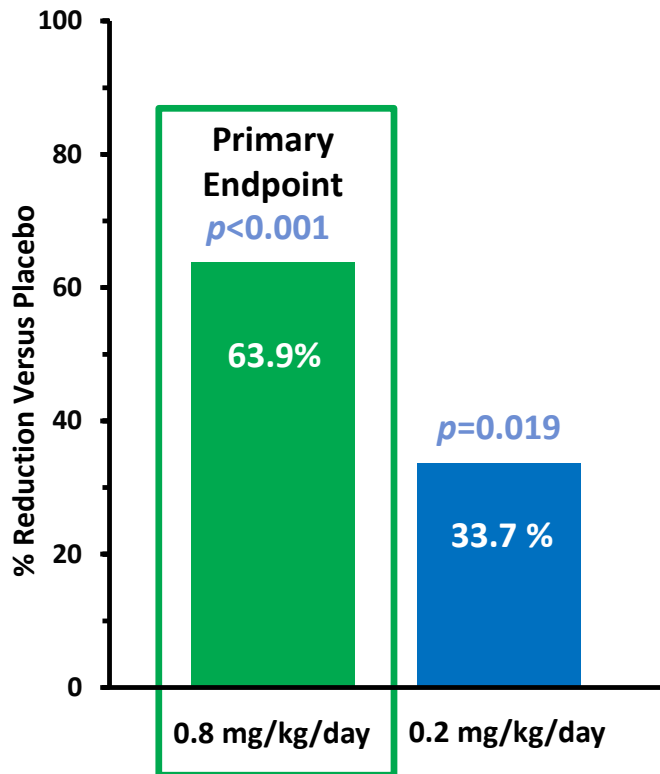
Initiate Global Phase 3 program for ZX008 in Lennox Gastaut syndrome



Positive Phase 2 open-label results
First patient enrolled in Phase 3 study (Nov 2017)

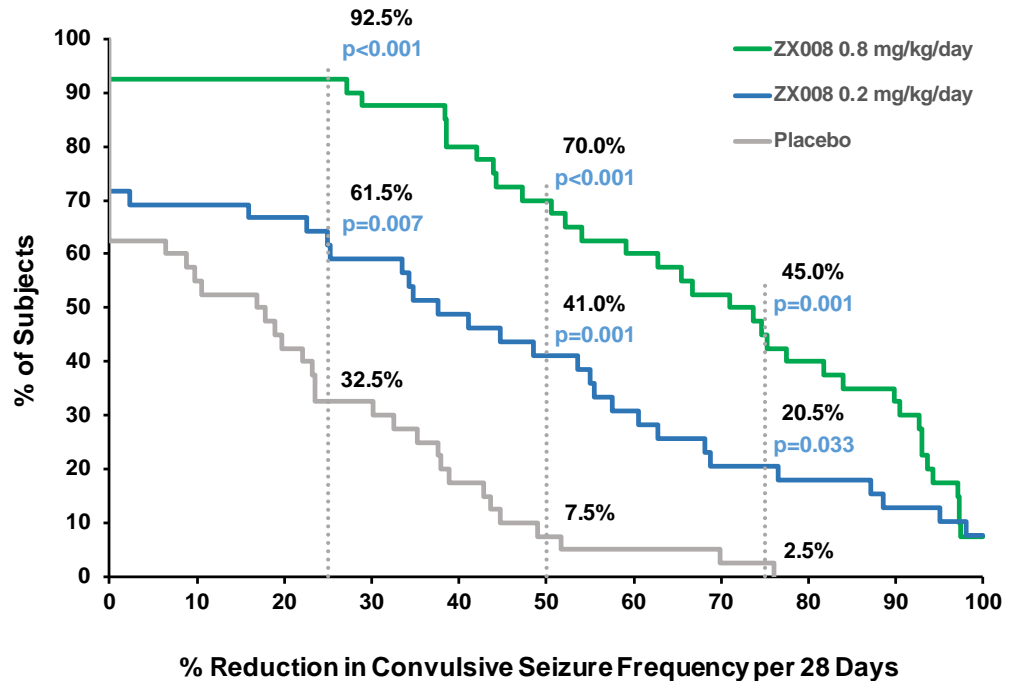
ZX008 in Dravet Syndrome: Study 1 Results

% Reduction in Mean Monthly Convulsive Seizures vs. Placebo



p-values are vs placebo; derived from ANCOVA

Cumulative Responder Analysis



p-values are vs placebo

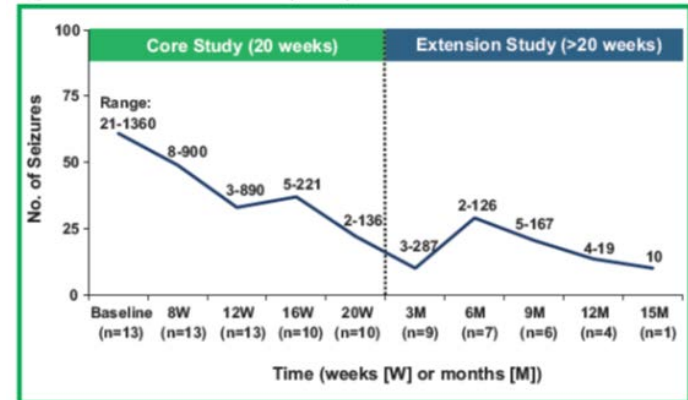
	Placebo (n=40)	ZX008 0.2 mg (n=39)	ZX008 0.8 mg (n=40)
Number of Subjects With at Least One Treatment Emergent Adverse Event (AEs)	26 (65.0%)	37 (94.9%)	38 (95.0%)
Number of Subjects With at Least One Treatment Emergent Serious Adverse Event (SAEs)	4 (10.0%)	4 (10.3%)	5 (12.5%)

- Generally well-tolerated with adverse events consistent with the known safety profile of fenfluramine.
- Prospective cardiac safety monitoring throughout the study demonstrated no clinical or echocardiographic evidence of cardiac valvulopathy or pulmonary hypertension.
- Five subjects in the 0.8 mg/kg/day group had an adverse event leading to study discontinuation, compared to zero in the other treatment groups.

- Positive results of ZX008 in Phase 2 LGS dose finding study as add-on therapy to AEDs⁽¹⁾
- Single global Phase 3 initiated Q4 2017
- Trial design similar to recent trial in Dravet syndrome
- Primary efficacy endpoint: change from baseline in monthly frequency of seizures that result in drops
- Anticipated sNDA (US) and MAA variation (EU) regulatory filing strategy

(1) Anti-Epileptic Drugs

Figure 3. Median seizure frequency/4 weeks for ZX008 treatment.*



*Seizure frequency during the Extension study was determined as total seizures during the 4 weeks prior to each visit vs baseline.
Decreasing n due to not reaching visit time point as yet, not to patient discontinuation.

Results

- 8/13 (62%) Responders⁽²⁾ during Core
- 6/9 (67%) Responders⁽²⁾ during Extension

Safety

- No signs of valvulopathy or pulmonary hypertension
- AEs consistent with known ZX008 profile
- 4 Core discontinuations: (1 ortho surgery, 3 decrease alert, sleepiness)
- 2 Extension discontinuations (loss of efficacy)

(2) Responder defined as $\geq 50\%$ reduction in seizure rate versus baseline

Upcoming 2018 Milestones

- Complete second Phase 3 study for ZX008 in Dravet syndrome
- File NDA and MAA for ZX008 for Dravet syndrome
- Dravet syndrome commercialization preparedness and pre-launch activities
- Enrollment of global Phase 3 study for ZX008 in Lennox Gastaut syndrome



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