



Corporate Presentation

August 2018



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 the projected timing of Zogenix's applications for regulatory approvals of ZX008 in the U.S. and 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 presentation 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; unexpected adverse side effects or inadequate therapeutic efficacy of ZX008 may limit regulatory approval and/or commercialization, or may 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 (the "SEC").

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 presentation 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.



ZX008 (Low Dose Fenfluramine) for the Treatment of Uncontrolled Seizures in Rare Epileptic Encephalopathies

DRAVET SYNDROME

Two positive pivotal Phase 3 trials

Primary endpoint and all key secondary endpoints met with high statistical significance

NDA and MAA submissions targeted for Q4 2018

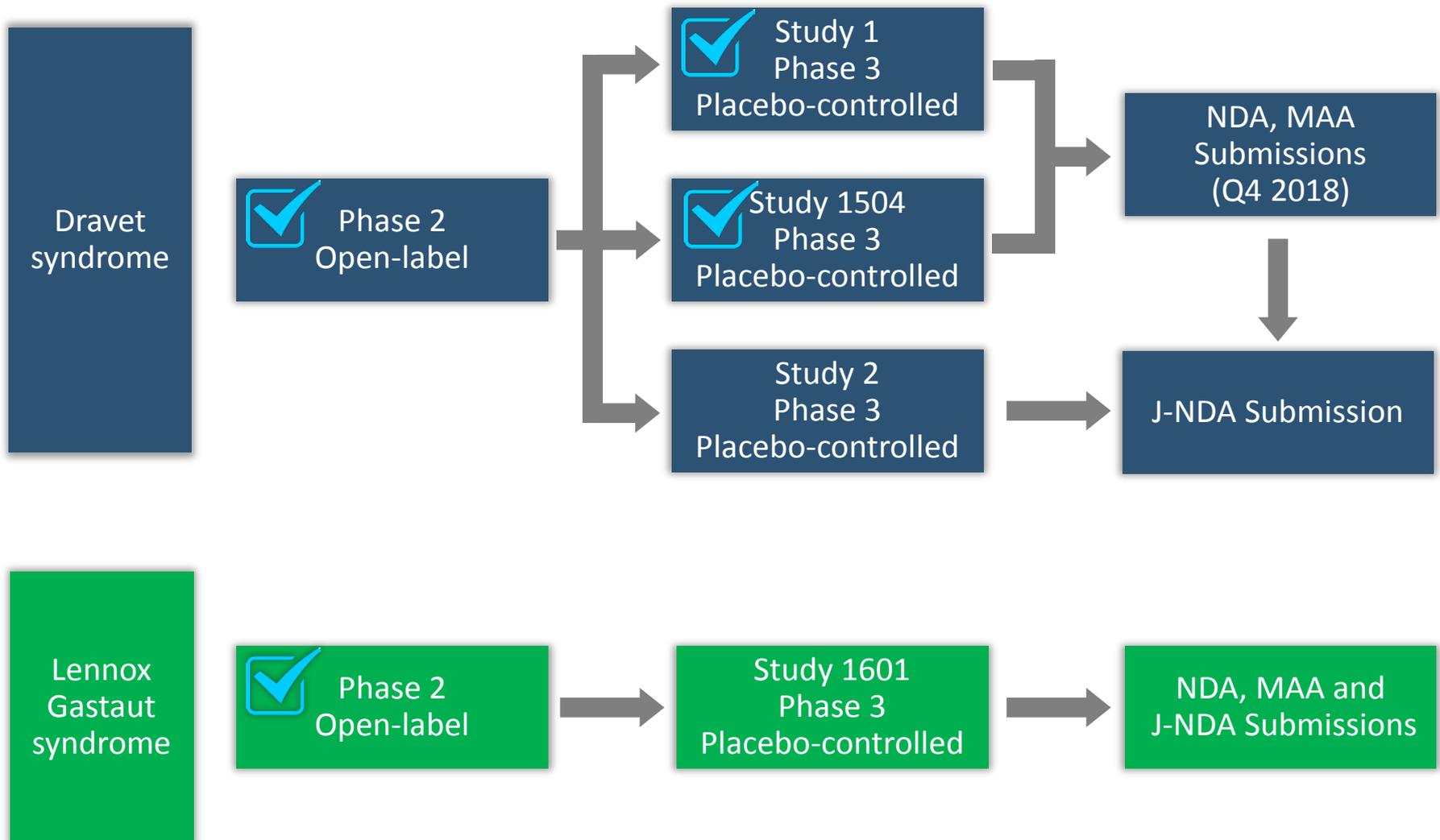
LENNOX GASTAUT SYNDROME

Global Phase 3 trial ongoing; initiated Q4 2017

- Severe Epileptic Encephalopathies
 - Begin in infancy or early childhood
 - Significant intellectual impairment and motor disabilities
 - Frequent and prolonged seizures, multiple types
 - High risk of status epilepticus, SUDEP
- Rare, Orphan Diseases
 - Dravet syndrome: recent incidence study 1/15,700 births in the U.S.
 - Lennox Gastaut syndrome: Approx. 30,000 patients in the U.S.; of which 14,500 – 18,500 are children
- Significant Unmet Needs
 - Pharmaco-resistant; most patients uncontrolled even on multiple AED's
 - Significant need to reduce or eliminate high seizure burden and comorbidities



ZX008 Development Summary



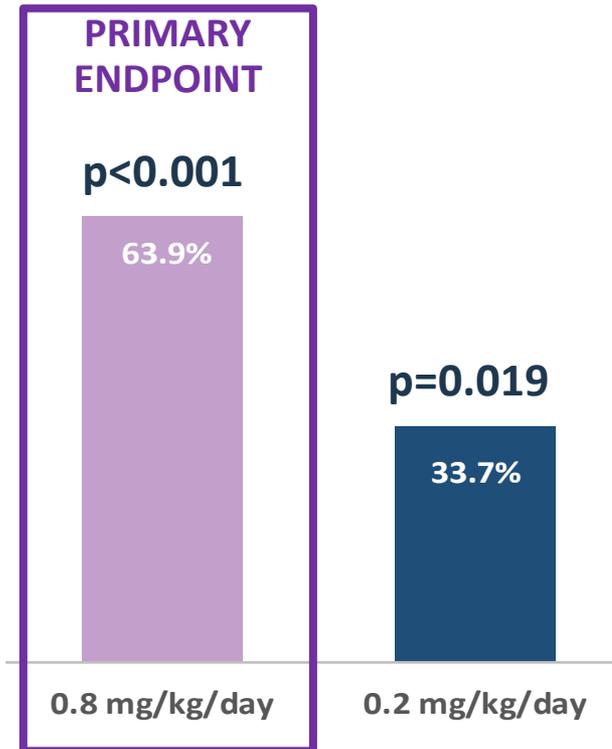
Study 1 Met Primary Efficacy Endpoint

Study 1 met primary endpoint demonstrating ZX008, at a dose of 0.8 mg/kg/day, is superior to placebo as adjunctive therapy in the treatment of Dravet syndrome ($p < 0.001$)

ZX008, at a dose of 0.2 mg/kg/day, also demonstrated superiority to placebo based on the same endpoint ($p = 0.019$)

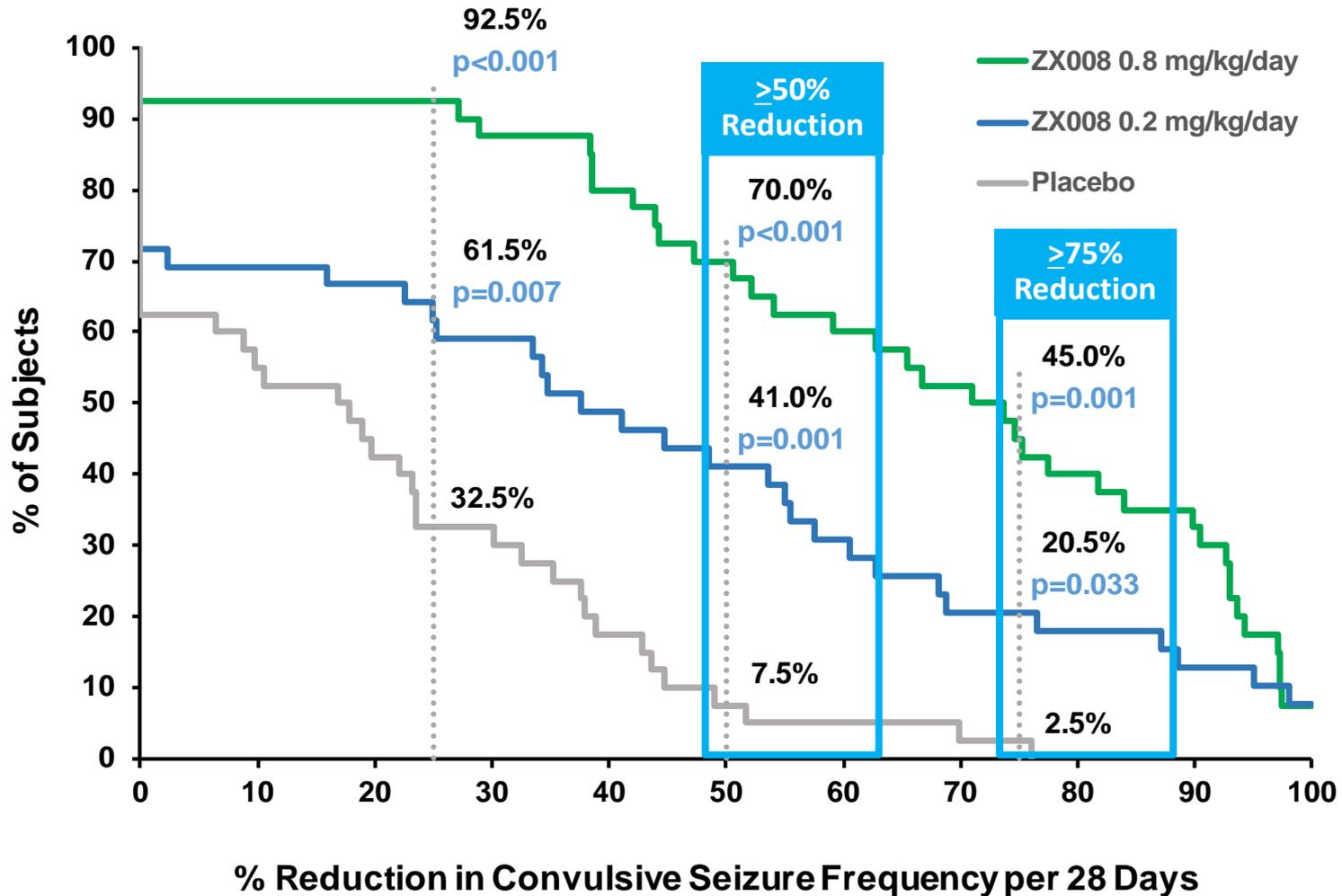
% DIFFERENCE FROM PLACEBO IN REDUCTION
IN MEAN MONTHLY CONVULSIVE SEIZURES

(2 WK TITRATION + 12 WK MAINTENANCE PERIOD =
TREATMENT PERIOD)



p-values are treatment compared with placebo group

Study 1 Responder Analysis



p-values are vs placebo; 50% response is primary endpoint for EMA

Study 1504 Met Primary Efficacy Endpoint

Study 1504 met primary endpoint demonstrating ZX008 at a dose of 0.5 mg/kg/day is superior to placebo as an adjunctive therapy to a stiripentol drug regimen in the treatment of Dravet syndrome ($p < 0.001$)*

* ZX008 dose accounts for drug-drug interaction with stiripentol and was selected to provide similar ZX008 exposure as 0.8 mg/kg/day dose in Study 1

% DIFFERENCE FROM PLACEBO IN REDUCTION
IN MEAN MONTHLY CONVULSIVE SEIZURES
(3 WK TITRATION + 12 WK MAINTENANCE PERIOD
= TREATMENT PERIOD)

$p < 0.001$

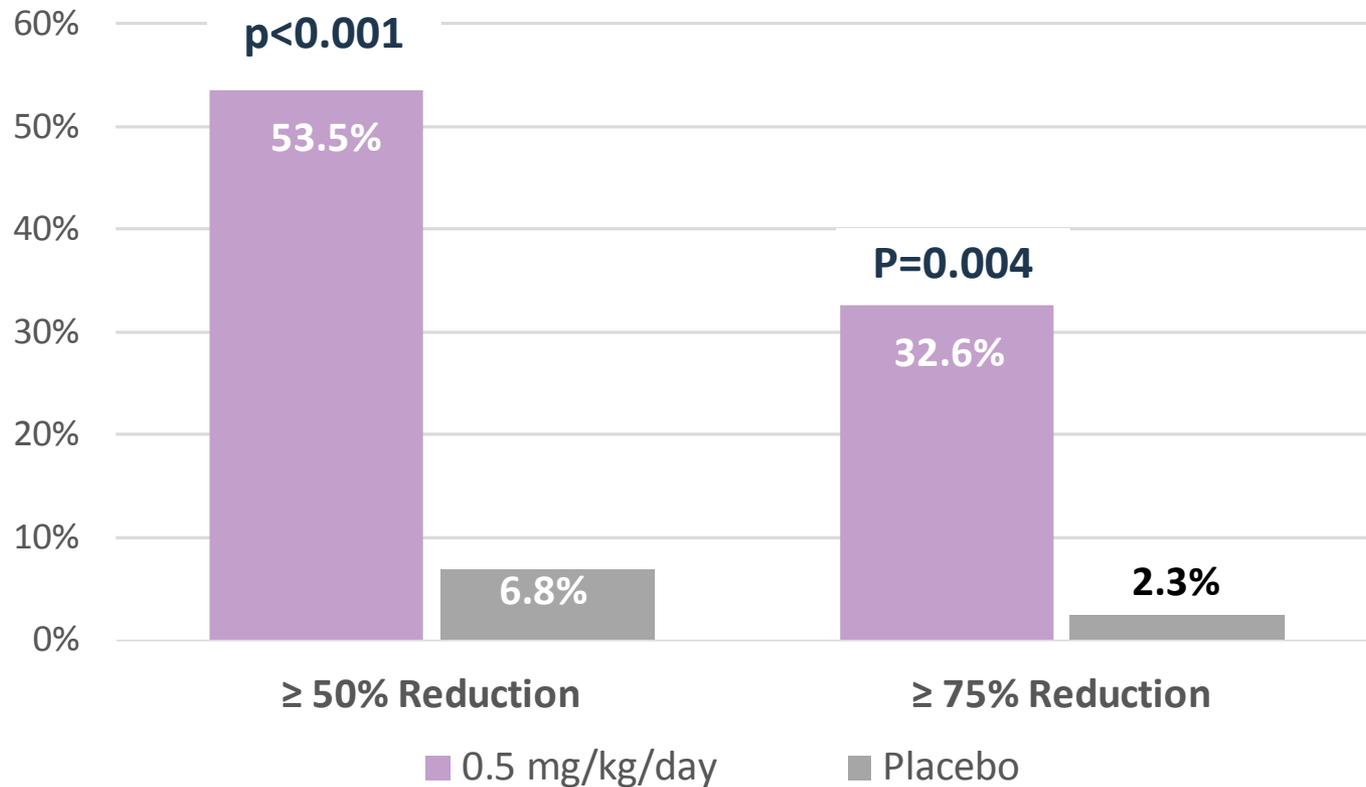
54.7%

0.5 mg/kg/day

p-value is treatment compared with placebo group

Study 1504 Convulsive Seizure Responder Rates Zogenix®

PROPORTION OF PATIENTS WHO ACHIEVED $\geq 50\%$ AND $\geq 75\%$
REDUCTION IN MEAN MONTHLY CONVULSIVE SEIZURES
(3 WK TITRATION + 12 WK MAINTENANCE PERIOD = TREATMENT PERIOD)

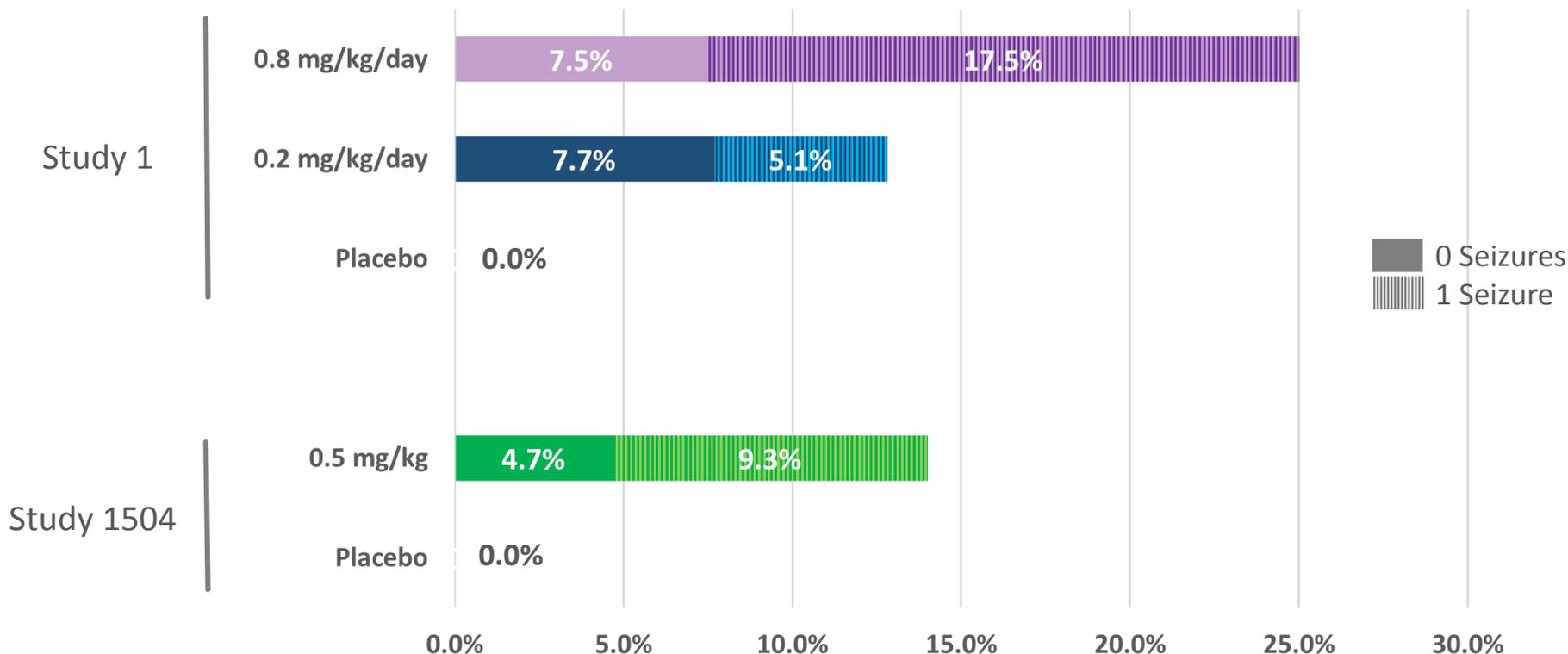


p-values calculated vs. placebo

Phase 3 Trials Seizure Freedom/Near-Freedom

PROPORTION OF PATIENTS WHO EXPERIENCED ZERO (0) SEIZURES OR ONE (1) SEIZURE THROUGHOUT TREATMENT PERIOD

TREATMENT PERIOD = TITRATION + MAINTENANCE PERIODS



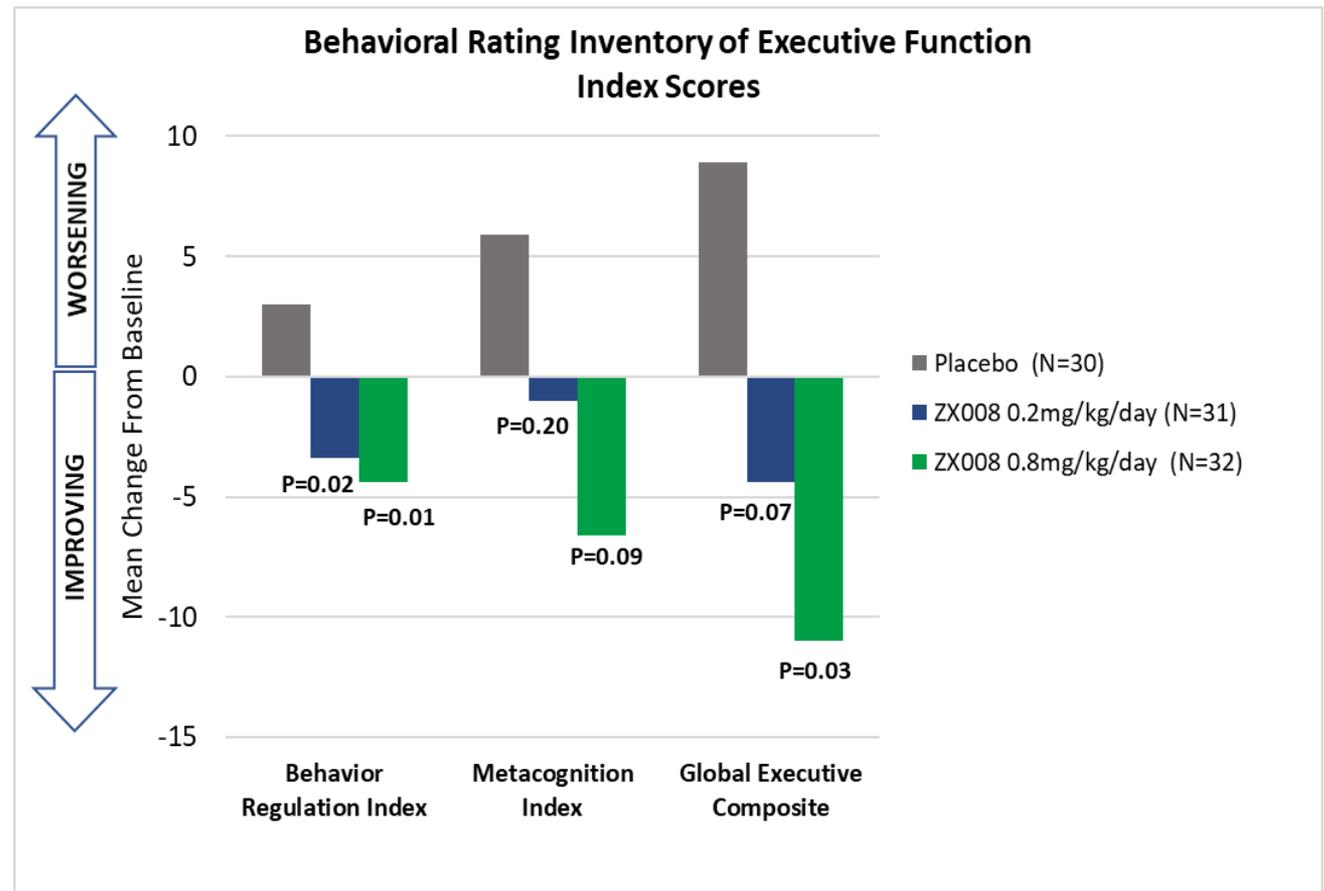
Mean monthly seizure rate at baseline for all patients in Study 1 was ~40

Mean monthly seizure rate at baseline for all patients in Study 1504 was ~25

Study 1: Positive Impact on BRIEF, Clinical Assessment of Executive Function

BRIEF: Behavioral Rating Inventory of Executive Function:

A measure of executive function in home & school; aspects such as control of impulses, modulate emotional responses appropriately, transition from one task to another



P values are compared to placebo

- ZX008 was generally well-tolerated in both pivotal Phase 3 trials
- No clinical or echocardiographic evidence of cardiac valvulopathy or pulmonary hypertension in any patient
- Most common AEs (>15%) were decreased appetite, diarrhea, fatigue, lethargy, pyrexia and nasopharyngitis
- Incidence of serious AEs was comparable across placebo and ZX008 treatment groups
- Low study discontinuation rates due to AEs

ZX008 Open-Label Study 1503 Status

- Over 300 subjects have rolled over from Dravet syndrome clinical trials to Open Label Extension Safety Study; 90% remain in study
- Over 65% of subjects have been dosed for at least 6 months in open label; >100 subjects in 2nd year of open label ZX008 treatment
- Cardiac safety monitoring continuing via echocardiography every 3 months
- No development of cardiac valvulopathy or pulmonary hypertension in any subject

Full Global Commercialization Rights Retained
“High Touch”, Targeted Commercial Effort
Leadership Experienced in Rare Disease and Epilepsy Markets



One recently approved product
(CBD)

Majority of patients
uncontrolled

~2,000 to 3,000 clinicians, most
pediatric neurologists⁽¹⁾

Target sales force of 20-30

Patent portfolio (issued & filed);
7.5 year orphan drug exclusivity



One approved product
(stiripentol)

Majority of patients
uncontrolled

Top 10 countries >90% of
opportunity

Filed patent portfolio;
12 year orphan & pediatric
drug exclusivity



One approved product
(stiripentol)

Majority of patients
uncontrolled

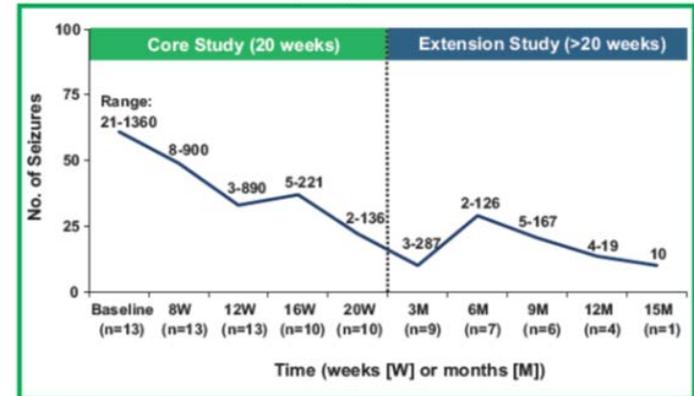
Patient population ~25-30%
of US population⁽¹⁾

Filed patent portfolio;
Seeking orphan drug
exclusivity

(1) Company estimates

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

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⁽¹⁾ during Core
- 6/9 (67%) Responders⁽¹⁾ 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)

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

Study 1601: Global Phase 3 Study in LGS

- **Part 1: Randomized, double-blind, 3-arm trial**
 - N=225, randomized 1:1:1 to 0.2 mg/kg/day or 0.8 mg/kg/day or placebo
 - ZX008 or placebo added to subjects' background AED regimen
 - Ages 2 to 35 years old
 - 4-week baseline, 14 week double-blind treatment period
- **Part 2: 12-month open-label extension**



U.S. and Canada: Screening initiated, 22 sites active and enrolling

EU & ROW: Site activation and screening beginning

ORPHAN DRUG STATUS

Provides 7.5 Years* in U.S. and 12 Years* of Market Exclusivity in EU

** Reflects additional exclusivity due to work in pediatric populations*

PATENT APPLICATIONS

Global, Multi-Family Patent Portfolio Including Method(s) of Use,
Elements of a Future REMS Program, and Novel Drug Synthesis Process

*Four Issued U.S. Patents - Method For the Treatment of Dravet Syndrome with ZX008
(Patent Expiries in 2033)*

Ongoing Clinical and Preclinical Development Work Providing Additional IP Opportunities

PRODUCT SPECIFIC REMS

Will Include Patient Registry and Cardiac Monitoring

Cash (as of Jun 30, 2018 plus net proceeds from Aug 07, 2018 secondary offering)	\$565 M
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Debt	\$0 M
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Common Shares Outstanding (as of Jun 30, 2018 plus shares issued in Aug 07, 2018 secondary offering) ⁽¹⁾	41.8 M
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(1) Excludes the following:

- a) 38K shares of common stock issuable upon exercise of outstanding warrants with a weighted average exercise price of \$76.11 per share;
- b) 3.8 million shares of common stock issuable upon the exercise of outstanding stock options with a weighted average exercise price of \$18.95 per share;
- c) 290K shares of common stock issuable upon the vesting of outstanding restricted stock units; and
- d) 1.8 million shares of common stock reserved for future issuance under our 2010 amended and restated equity incentive award plan, our 2010 employee stock purchase plan and our 2013 employment inducement equity incentive award plan.

Recent and Upcoming Milestones

2H 2017 - TODAY

- ✓ Highly significant efficacy results in first Phase 3 trial in Dravet syndrome
- ✓ FDA Breakthrough Therapy Designation for ZX008 in Dravet syndrome
- ✓ Positive Type B meeting with FDA
- ✓ Highly significant efficacy results in second Phase 3 trial in Dravet syndrome
- ✓ Initiation of global Phase 3 trial in Lennox Gastaut syndrome

2H 2018

- Pre-NDA meeting with FDA
- NDA and MAA submissions for ZX008 in Dravet syndrome
- Multiple publications and presentations of Study 1 and other ZX008 program results
- Dravet syndrome commercialization preparedness and pre-launch activities
- Continue enrollment of global Phase 3 trial in Lennox Gastaut syndrome



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