NASDAQ: ZGNX

Zogenix®

Company Presentation

October 2017

Forward Looking Statement

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Zogenix®

Highly Focused on Developing and Commercializing Therapies for
RARE CNS DISORDERS

ZX008 (Low Dose Fenfluramine) for the Treatment of Uncontrolled Seizures

DRAVET SYNDROME
Phase 3 Program Underway
Announced Positive Top Line Results in Study 1

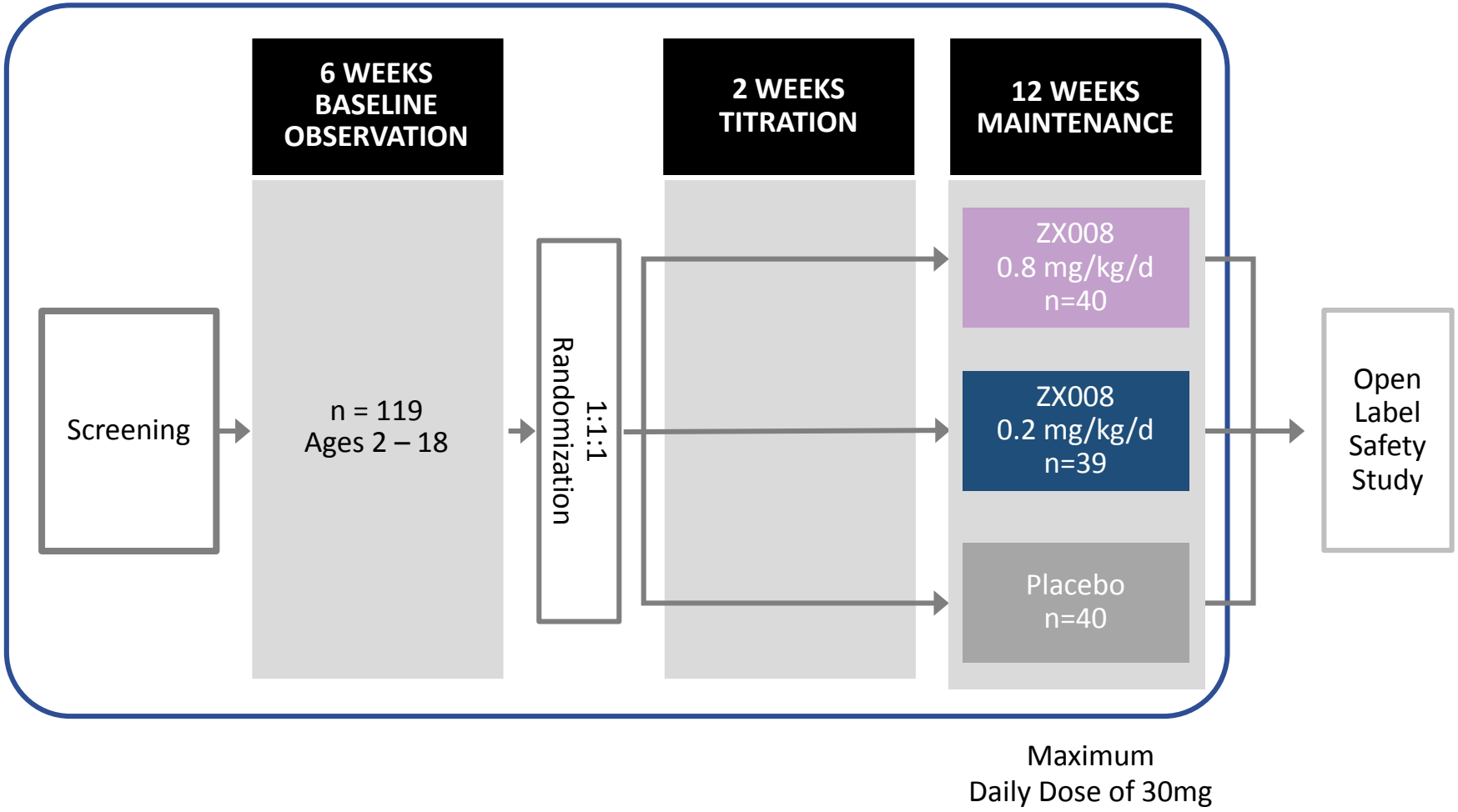
LENNOX GASTAUT SYNDROME
Targeting Phase 3 Trial Initiation Q4 2017

Dravet Syndrome: Catastrophic Illness

- Intractable, Severe Epilepsy
  - Begins in infancy
  - Pharmcoresistant
  - Genetic epilepsy syndrome (SCN1A)
  - Developmental encephalopathy
  - Intellectual disability, ADHD, behavior, crouch gait
  - Higher incidence of status epilepticus, SUDEP
- Rare, Orphan Disease
  - Recent incidence study 1/15,700 births
  - Can go undiagnosed or have late diagnosis
- No Effective Long-Term Treatments Exist
  - Standard of care: Valproate, topiramate, levetiracetam, clobazam, clonazepam, stiripentol (EU)
  - No approved drugs for Dravet syndrome in U.S.
  - Sodium channel blockers (carbamazepine, phenytoin) make seizures worse

ZX008 Phase 3 Study 1 Design

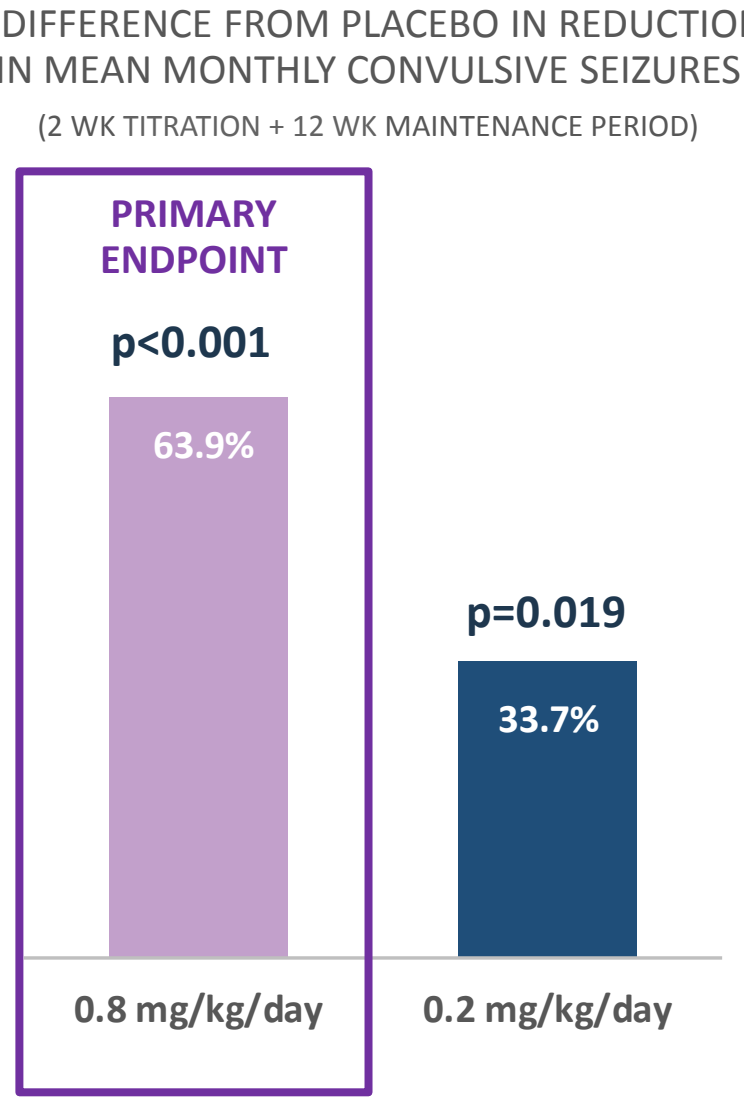
Study 1 is a prospective merged analysis of two identical double-blind, placebo-controlled studies ZX008-1501 (US/Canada) and ZX008-1502 (Europe/Australia)



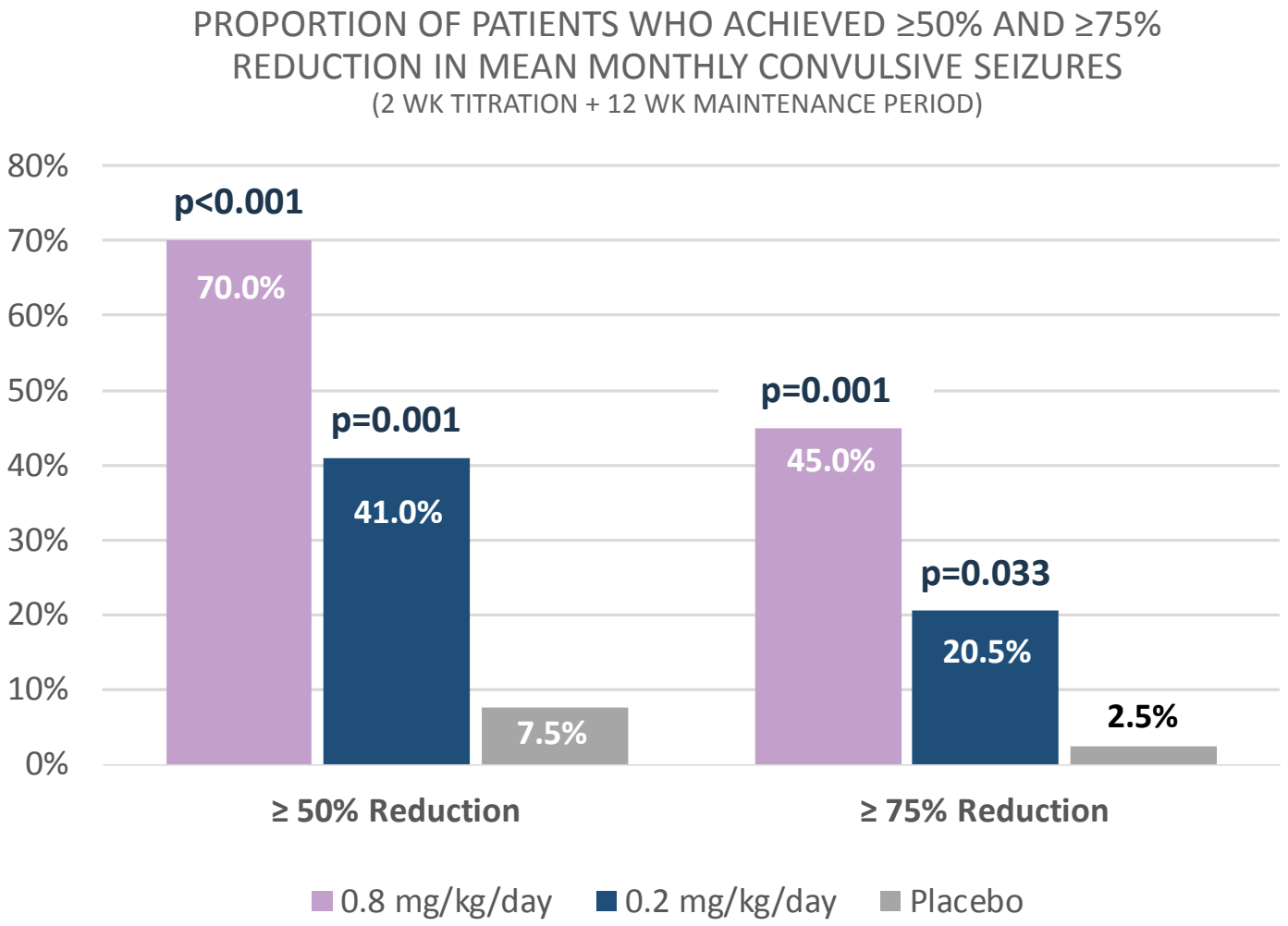
Study 1 Met Primary Efficacy Endpoint

- 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 based on the change in the mean monthly convulsive seizure frequency (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)

p-values are treatment compared with placebo group



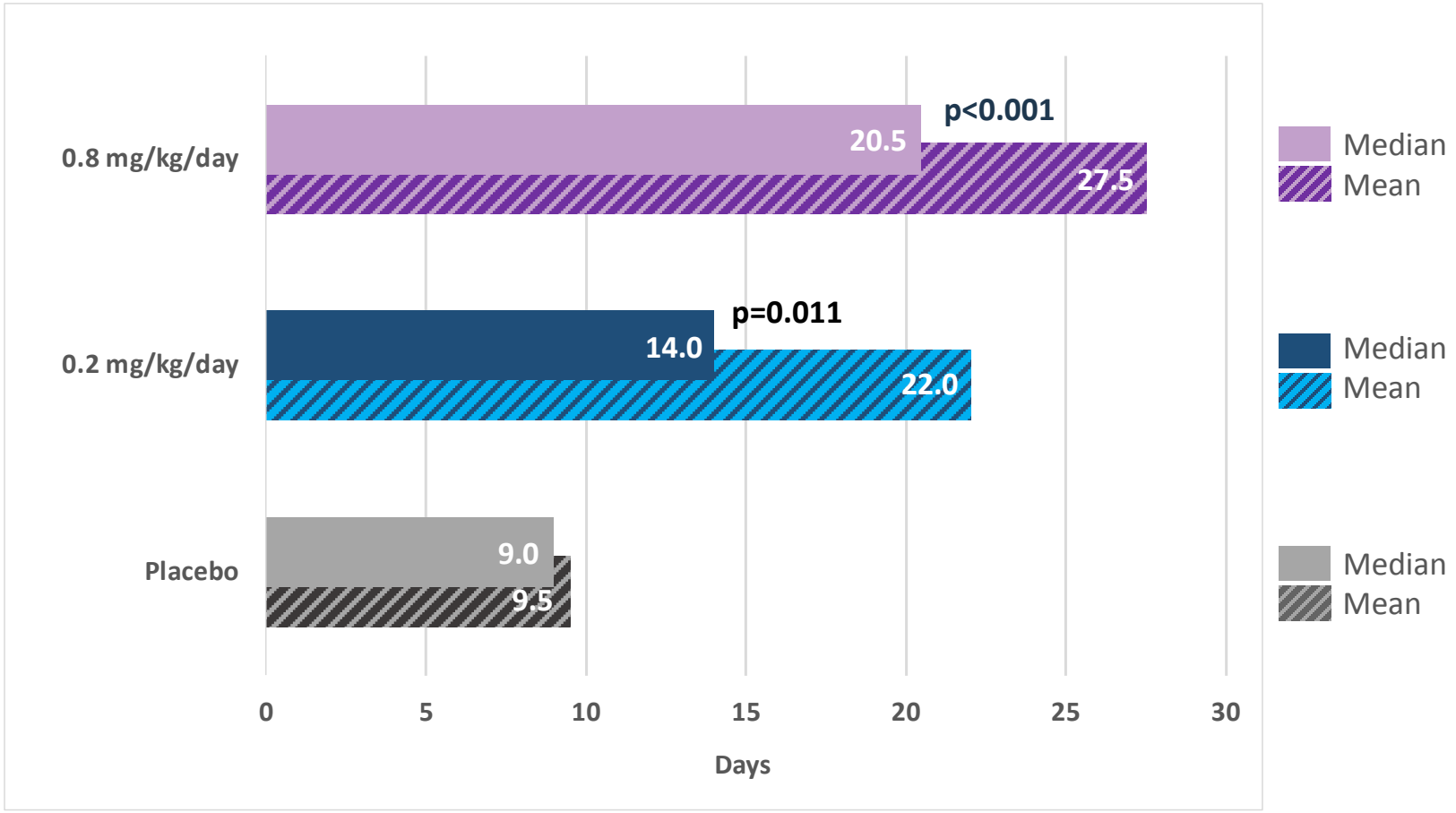
Study 1 Convulsive Seizure Responder Rates



p-values calculated vs. placebo

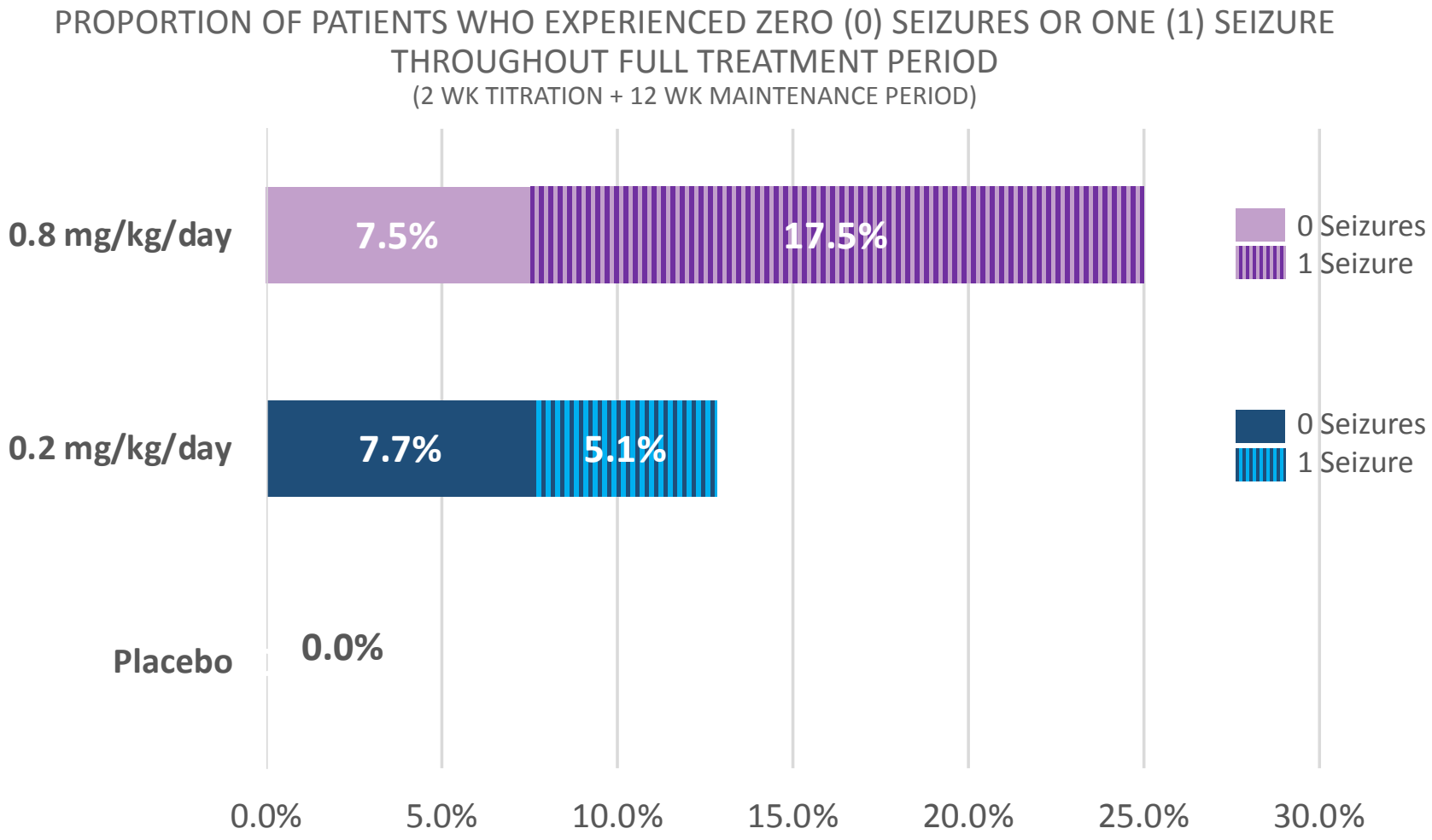
Study 1 Longest Seizure Free Interval

MEDIAN AND MEAN OF EACH PATIENT'S LONGEST SEIZURE FREE INTERVAL
(2 WK TITRATION + 12 WK MAINTENANCE PERIOD)



p-values are for median values vs. placebo

Study 1 Seizure Freedom/Near-Freedom Rates



Note: mean monthly seizure rate at baseline for all patients in Study 1 was 40/month

ZX008 Study 1 Safety

	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%)

- Prospective cardiac safety monitoring throughout the study demonstrated no clinical or echocardiographic evidence of cardiac valvulopathy or pulmonary hypertension.
- Generally well-tolerated with adverse events consistent with the known safety profile of fenfluramine.
- The incidence of treatment emergent adverse events was higher in treatment groups as compared to placebo; however, the incidence of treatment emergent serious adverse events was similar in all three groups.
- 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.

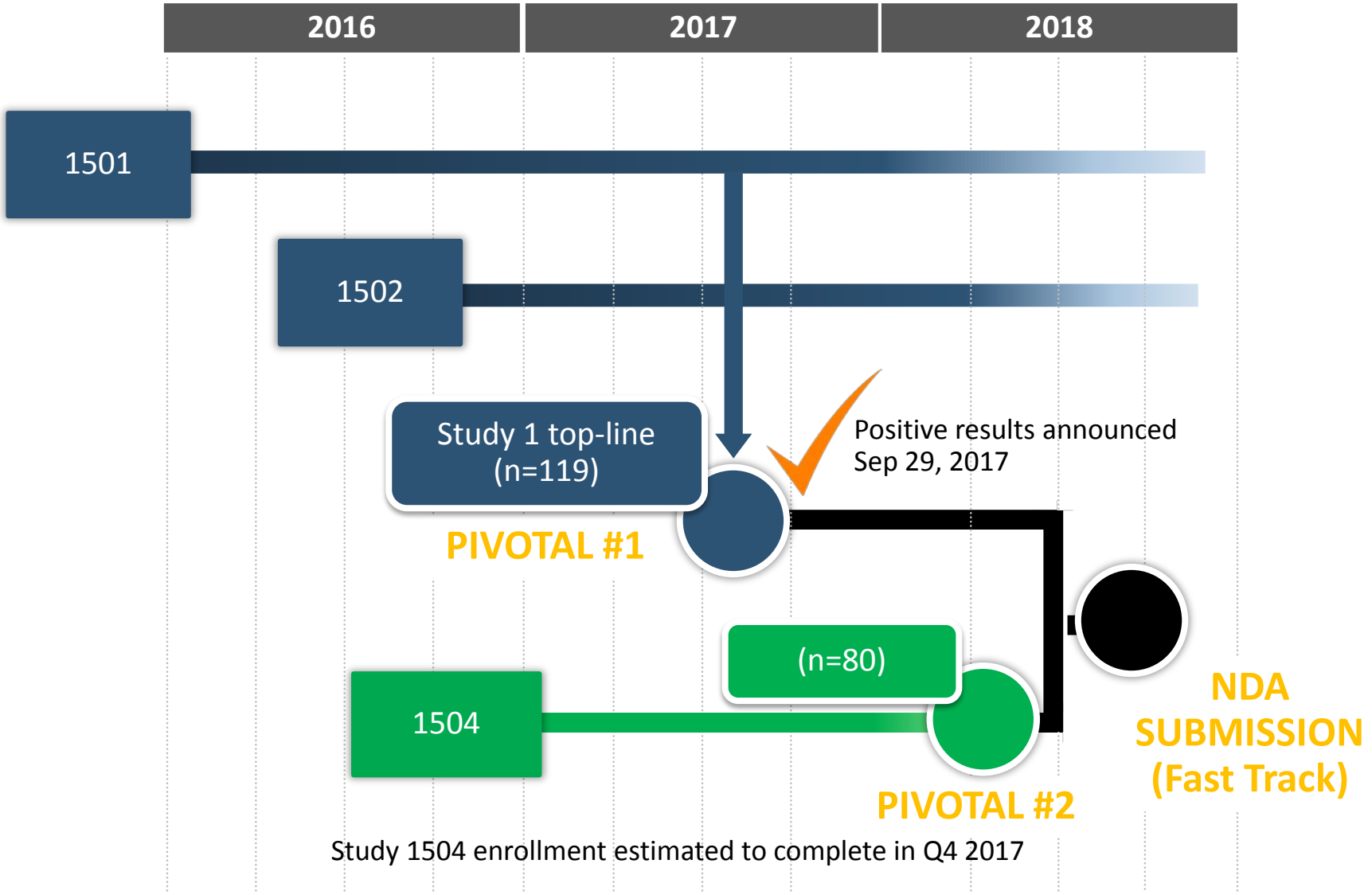
Global Phase 3 Program in Dravet Syndrome

ZX008 as adjunctive treatment for seizures in children and young adults with Dravet syndrome

Study 1501 / Study 1502	Study 1504
Double-blind, randomized, placebo-controlled, 12-week treatment **Two active doses (0.2 mg/kg/day, 0.8 mg/kg/day) & placebo**	Double-blind, randomized, placebo-controlled, 12-week treatment **One active dose (0.5 mg/kg/day) & placebo**
U.S. Standard of Care; **Stiripentol excluded**	EU Standard of Care; **All patients on stiripentol**
Primary outcome: Change from baseline in frequency of monthly convulsive seizures	Primary outcome: Change from baseline in frequency of monthly convulsive seizures
FDA confirmation of adequate and well-controlled pivotal trial design	FDA confirmation of adequate and well-controlled pivotal trial design

All patients eligible to enter Study 1503: long-term, open-label, flexible-dose extension

ZX008 NDA Filing Strategy in Dravet Syndrome



Study 1504 enrollment estimated to complete in Q4 2017

Commercial Manufacturing Readiness

- Room-temperature, oral solution (2.5 mg/mL)
- Proprietary, patent-pending synthesis
- Outsourced production via leading third party CMOs with GMP licensed facilities
- Commercial scale implemented for drug substance and product
- Registration batches completed
- CMC regulatory sections underway and scheduled to be completed in 2017

ZX008 Commercialization in Dravet Syndrome

Full Global Commercialization Rights Retained

"High Touch", Targeted Commercial Effort

Leadership Experienced in Rare Disease and Epilepsy Markets

No approved products

Majority of patients uncontrolled

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

Target sales force of 20-30

7.5 year orphan drug exclusivity; issued patents

One approved product (stiripentol)

Majority of patients uncontrolled

Top 10 countries >90% of opportunity

15-30 key pediatric centers in major countries(1)

12 year orphan & pediatric investigational plan drug exclusivity

(1) Company estimates

Lennox Gastaut Syndrome (LGS)

- Refractory, Debilitating Early-Onset Epilepsy
  - Onset peaks 5-7 years of age
  - Multiple seizure types, including drop seizures
  - Cognitive impairment
  - Characteristic EEG abnormality
  - Underlying etiologies: 66% brain abnormalities, 33% unknown
  - No known genetic abnormality identified

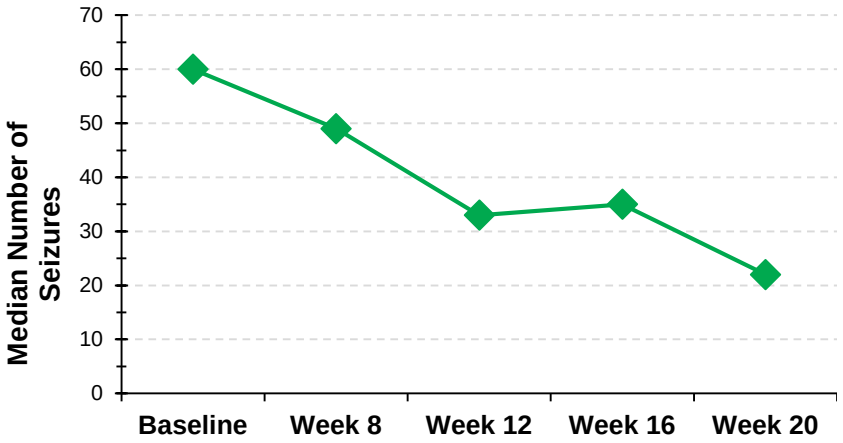
- Rare, Orphan Disease
  - US Prevalence: ~30,000; 14,500 - 18,500 children
  - EU Prevalence: >50,000; ~11,000 children
- No Effective Long-Term Treatment Exists
  - Polypharmacy with AEDs is standard of care
  - Six drugs approved in US but large unmet need persists

ZX008 in LGS: Interim Phase 2 Results

Open Label, 20-Week, Dose Finding Trial of ZX008 as Add-on Anticonvulsive Therapy

- 13 patients to date (average age 11.4 years) with minimum of 12 weeks of treatment
  - Failed a median of 5 antiepileptic therapies
  - Receiving a median of 4 antiepileptic therapies
  - At least 4 documented major motor seizures during 4-week baseline
- Dose-finding study design:
  - All patients start at 0.2 mg/kg/d ZX008
  - Semi-forced step-titration design: patients who tolerate study drug but do not achieve 50% reduction in seizures step to next dose level
  - Dose adjusted after 4 weeks at level: 0.2 mg/kg/day to 0.4 mg/kg/day to 0.8 mg/kg/day; max dose 30 mg/day

Intent-to-Treat (ITT) Population



- 7/13 (54%) Achieved at Least a 50% Reduction in Number of Major Motor Seizures
- Dose-Response is Emerging: 2-Fold Increase in Responders, 0.2 versus 0.4 mg/kg/d
- Most Common AEs: Decreased Appetite (3), Somnolence (3), Insomnia (2)
- Early Termination by 4 Patients
  - 3 discontinued due to AEs
  - 1 withdrew after loss of response following orthopedic surgery

Lagae *et al.* American Epilepsy Society Annual Meeting, Houston, December 2016

ZX008 LGS Development Plan

- Single, global Phase 3 trial leveraging sites participating in the ZX008 Phase 3 program in Dravet syndrome
- IND accepted in April 2017 to enable Phase 3 initiation in Q4 2017
- Trial design consistent with recent positive trial in Dravet syndrome
- Primary efficacy endpoint will be the mean change in the number of seizures that result in drops per 28 days during the treatment titration and maintenance periods compared with baseline
- Anticipated regulatory filing strategy is sNDA (U.S.) and MAA variation (EU) pending Dravet syndrome approval
- Orphan Drug Status received for both U.S. and EU during H1 2017

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ZX008 Phase 3 Study 1 Top-Line Results

Trial met primary endpoint of the change in the mean monthly convulsive seizure frequency

All key secondary endpoints positive and demonstrated statistical significance