INTRODUCTION

Dravet syndrome is a rare, severe, pediatric-onset treatment-resistant epileptic encephalopathy - characterized by various seizure types and neurodevelopmental and psychomotor disabilities - current treatment strategy involves polypharmacy with the use of many antiepileptic drugs (AEDs), although these treatment regimens remain inadequate for most patients.

45% of patients continue to experience ≥4 seizures per month despite multiple AED regimens.

- Stiripentol is approved in Europe, Australia, Canada, and Japan for adjunctive treatment of patients with Dravet syndrome and available via compassionate use in the USA.

- ZX008 has recently been reported to substantially reduce the convulsive seizure burden in subjects with Dravet syndrome compared with placebo in the ZX008 Phase 3 Study (1).

METHODS

- Study design: This was a parallel-group, randomized, double-blind, placebo-controlled, fixed-dose clinical trial.

RESULTS

- 125 subjects were randomized to placebo, ZX008 0.2 mg/kg/day, and ZX008 0.8 mg/kg/day across 3 phases.

- Seizure frequency and tolerability were assessed for 12 weeks across all phases, with a 14-day titration phase and a 10-week maintenance phase.

- The primary efficacy outcome was reduction in major motor seizure frequency as measured by investigator-rated Global Impression of Improvement (GIOI) scores at Phase 2 and Phase 3.

- ZX008 0.8 mg/kg/day demonstrated statistically significant (S) and clinically meaningful (CM) decrease in seizure frequency compared with placebo (P).

- ZX008 0.2 mg/kg/day showed a trend towards S decrease in seizure frequency compared with placebo.

- Seizure-free intervals were longer in the active treatment groups compared with placebo (P).

CONCLUSIONS

- This study was funded by Zogenix, Inc. (Emeryville, CA). Zogenix thanks all of the subjects, their families, and the investigators involved in this study.

ACKNOWLEDGMENTS

Poster presented at the 2018 American Academy of Neurology (AAN) Annual Meeting; April 21-27, 2018; Los Angeles, CA, USA

REFERENCES


DISCLOSURES

- 6. Consultant/advisor, Brabant, Zogenix.
- 10. Research support, Zogenix.
- 17. Zogenix.
- 23. Zogenix.
- 29. Zogenix.