**Effect of Low-Dose Fintepla® (Fenfluramine HCl Oral Solution) on Seizure and Non-Seizure Clinical Outcomes in Dravet Syndrome: A Case Series of 5 Pediatric Patients Receiving Fenfluramine Through Compassionate Use Protocols**

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**INTRODUCTION**

Dravet syndrome (DS) is a severe, treatment-resistant pediatric epilepsy syndrome with an incidence of approximately 1 in 15,700 births. It is characterized by recurrent, treatment-refractory seizures of multiple types, intellectual disability, motor and language impairment, and developmental delays, with increased severity of mortality (35%) if patients live more than 15 years. Caregivers of patients with DS report non-seizure comorbidities (eg, speech/communication impairments, impacts on diet, and cognitive impairment) rate higher than seizures if the patient survives.

Adjuvant fenfluramine (FFA) reduced convulsive seizures (CS) frequency in a recent phase 3, randomized, placebo-controlled clinical study. In the FFA groups, 39.7% of patients showed clinically meaningful improvement in all other non-seizure outcomes except for sensitivity to light/patterns (20%) after 4 weeks of FFA treatment, with fewer episodes of status epilepticus and shorter clusters.

**METHODS**

To present a case series of 5 children and adolescents with DS receiving adjuvant FFA under a compassionate use-investigator-initiated new drug (NID) program.

**RESULTS**

**Seizure Outcomes**

- Reduction in CS frequency during the last 4 weeks of treatment vs 4 weeks prior to FFA initiation ranged from 47%-100% (Figure 1).
- 3 patients were seizure-free; 1 had a 96% reduction, and 1 had a 4% reduction.
- CS were reported to be lessened in severity and duration after FFA, with improvements noted in postictal state (Figure 2).

**Non-Seizure Outcomes**

- Initial no change in myoclonic seizures, but weeks 4 and 8 saw meaningful improvement.
- FFA may be an important and effective new DS treatment option.

**CONCLUSIONS**

- Clinical meaningful reduction in seizure frequency and improvements in both seizure and non-seizure outcomes.
- Clinical meaningful improvements in non-seizure outcomes, including cognition, behavior, speech, alertness, and motor ability, were noted in 40%-60% of patients.
- FFA was generally well-tolerated with no development of cardiac valve disease or pulmonary hypertension.
- FFA may be an important and effective new DS treatment option with efficacy in improving both seizure and non-seizure outcomes.

**REFERENCES**


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