DRAVET ENGAGE

PARENT EXPERIENCES AND PERSPECTIVES INFORMING CLINICAL RESEARCH

OVERVIEW

In 2020, Encoded Therapeutics engaged parents of children living with Dravet syndrome to learn about their experiences with Dravet syndrome and their perspectives, priorities, and desired outcomes of clinical trials of potential of disease modifying therapies.

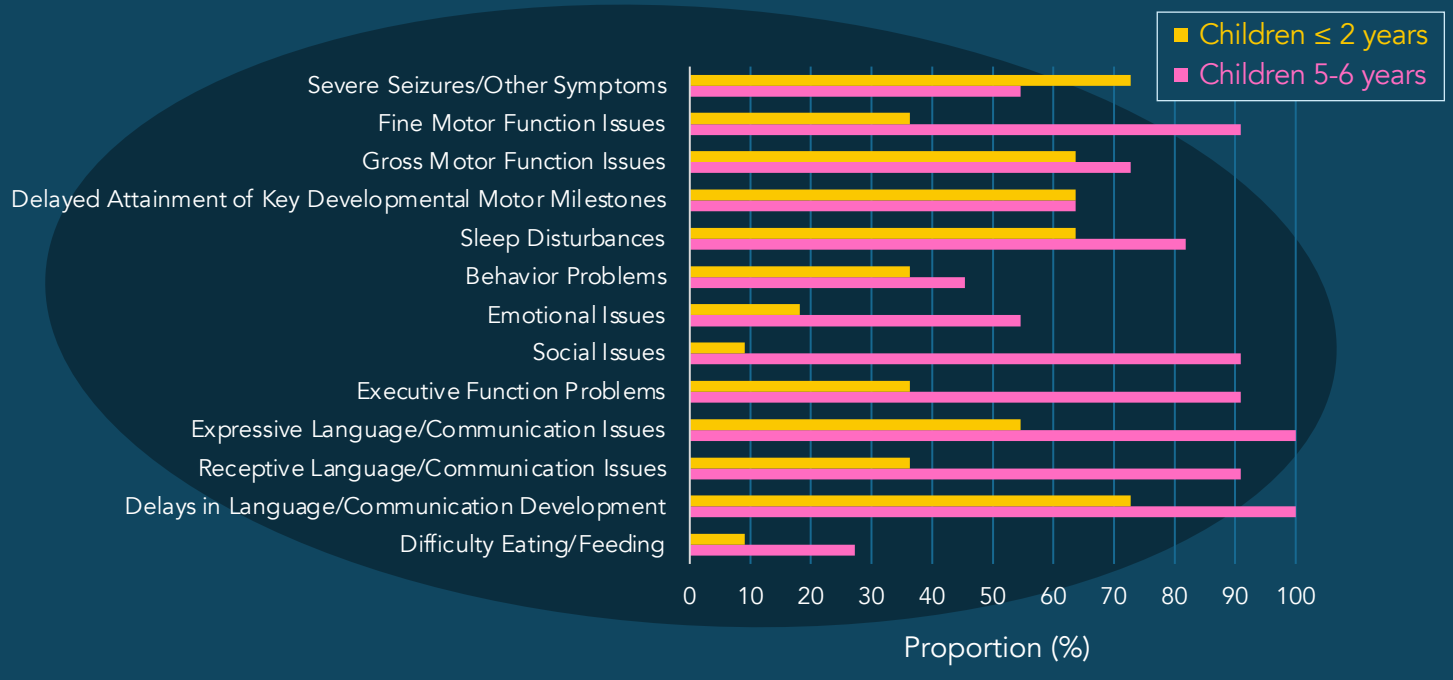
GROUP DISCUSSION WITH 10 PARENTS

INDIVIDUAL INTERVIEWS WITH 6 PARENTS

SURVEY WITH 36 PARENT RESPONDENTS

MANY ASPECTS OF DRAVET SYNDROME BECOME MORE COMMON WITH AGE

SPECIFIC ASPECTS OF DRAVET SYNDROME OCCURING IN THE LAST SIX MONTHS AS REPORTED BY PARENTS



Reference: Juandó-Prats C et al., 2021

Encoded THERAPEUTICS

PARENTS SEEK A THERAPY THAT EASES MULTIPLE ASPECTS OF DRAVET SYNDROME

TOP 5 DESIRED TARGETS OF A POTENTIAL NEW THERAPY

PARENTS ARE CAUTIOUSLY OPTIMISTIC ABOUT POTENTIAL DISEASE-MODIFYING THERAPIES

FOUR THEMES EMERGED FROM GROUP DISCUSSIONS AND INTERVIEWS

1 ENROLLING MY CHILD IN A CLINICAL TRIAL: EXPECTATIONS AND VALUED OUTCOMES

- Outcomes should recognize the impact of Dravet syndrome on whole family.
- Main hopes are to stop regression, along with reducing seizures.

2 IS THE TRIAL RIGHT FOR MY CHILD? AGE RELATED INCLUSION CRITERIA

- Treating children at an early age may provide the greatest opportunity for transformative benefit.
- Strong desire to see clinical trials suitable for all ages, where possible, in the future.

3 VIEWS ON A FIRST-TIME-IN-HUMANS CLINICAL TRIAL DESIGN

- Understanding that optimal distribution of gene therapy in the brain requires ICV injection.
- Delayed-treatment sham controls are acceptable, provided treatment given in a concise timeframe.

4 GENE THERAPY

- The potential positive impact of a one-time disease-modifying therapy is exciting.
- Cautious about the potential long-term implications in the absence of data.

"A few years ago, researchers focused on seizures, not the holistic approach to Dravet syndrome. Dravet is much more than a type of epilepsy."

Thank you to those parents who generously shared their time and their experiences caring for their children, the Dravet Syndrome Foundation (USA) for support with recruitment for the focus group and interview discussions and in the drafting and fielding of the caregiver survey, and Dravet Syndrome UK for support with recruitment for the focus group and interview discussions.

Reference: Juandó-Prats C et al. DRAVET ENGAGE. Parent caregivers of children with Dravet syndrome: Perspectives, needs, and opportunities for clinical research. Epilepsy & Behavior. Sep 2021; 122: https://doi.org/10.1016/j.yebeh.2021.108198

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