



THE VOICE OF DRAVET SYNDROME

Daily Challenges of a Progressive Genetic Epilepsy

The Dravet Syndrome Foundation Voice of the Patient Report



IN FEBRUARY 2022, The Dravet Syndrome Foundation (DSF) hosted an Externally-Led Patient Focused Drug Development (EL-PFDD) meeting to bring together patients, caregivers, FDA representatives, and physicians to share perspectives on the challenges of life with Dravet syndrome.

IN MAY 2022, DSF published the [Dravet Syndrome Voice of the Patient Report](#), with insights from the meeting and survey results from more than 100 caregivers on the daily challenges of the disease, including the need for more effective treatments.

KEY INSIGHTS

1

Dravet syndrome is more than seizures.

Other significant symptoms include developmental and/or intellectual disability, risk of sudden unexpected death in epilepsy (SUDEP), sleep disruptions, and behavioral issues.

91%



of caregivers reported experiencing developmental delays or loss of developmental skills.

2

The unpredictable nature of Dravet syndrome impacts the whole family.

Caregivers and siblings report significant, long-term impacts to their quality of life due to social isolation, strained relationships, and the need to adhere to strict schedules. People with Dravet syndrome often require constant supervision and care for their entire lives.

77%



of caregivers expressed concern about how loved ones with Dravet syndrome will be cared for and maintain access to resources as they get older.

3

All people with Dravet syndrome require polytherapy.

Patients typically try many medications and are on a regimen of multiple anti-seizure medications, in addition to physical therapy and speech therapy. Despite these therapies, they continue to experience seizures and a myriad of non-seizure comorbidities.



4

Families cited that they desperately need new therapies.

Most medicines aim to control seizures, and none address the genetic cause of the disease. Most families reported that they would be willing to consider a variety of disease modifying therapies, and for some caregivers even a partial reduction in seizures is a benefit.

77%



of caregivers reported that current approaches only treat some, and not all symptoms of Dravet.



We need better solutions and better therapies in order to fully succeed in Dravet syndrome...We did our absolute best for [our son] and offered him the best therapies available. The fact that even a well-controlled Dravet syndrome patient is not protected from sudden death is a tragedy.

—Sarah M., mother who lost her 8-year-old son due to Dravet syndrome



Impact on FDA Benefit-Risk Assessment Framework



The FDA uses a Benefit-Risk Assessment Framework, which provides important context for drug regulatory decision-making based on factors such as analysis of condition, current treatment options, benefit, risk, and risk management.

Following analysis of the EL-PFDD and caregiver survey results, DSF has proposed a framework for FDA to consider in their Benefit-Risk Assessment that may enable a more comprehensive understanding of Dravet syndrome among key reviewers responsible for evaluating new treatments.

Conclusion

The Voice of the Patient Report will help advance DSF's mission to aggressively raise funds for Dravet syndrome and related epilepsies, to support and fund research, increase awareness, and to provide support to affected individuals and families.

For more information about **Dravet syndrome** or the **Voice of the Patient Report**, visit [DravetFoundation.org](https://www.DravetFoundation.org).

