Includes Input From More Than 490 Dravet Syndrome Caregivers

The Dravet Syndrome Patient Experience

A study of the real-world experience of patients and caregivers living with Dravet syndrome.

Patient Journeys.

www.PatientJourneys.org

Introduction

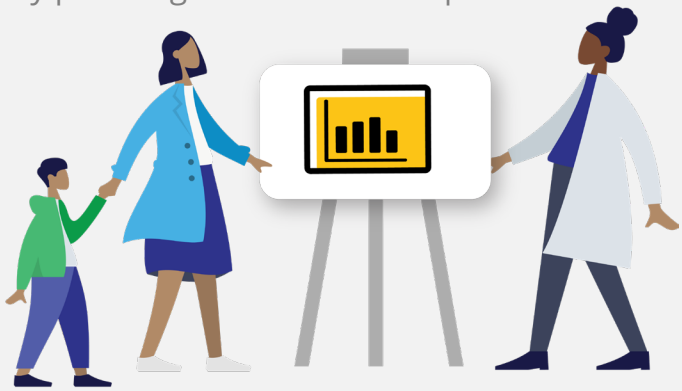
Thanks to the participation of over 490 parents and other caregivers of patients affected by Dravet syndrome, we have compiled this overview of commonly reported experiences of those living with the disease. We hope that the knowledge of your peers' experiences with Dravet aids in your own understanding of the disease and how it may be managed. Armed with this broad understanding of the possible realities of living with Dravet, we hope that you will feel better able to make informed decisions regarding medical care for the person you care for.

It's important to remember that the content in this report is not intended to be a substitute for professional medical advice, diagnosis, or treatment. Always seek the advice of your physician or other qualified health provider with any questions you may have regarding a medical condition.

We sincerely thank you for taking the time to share your experience with many other individuals affected by Dravet, and hope you take comfort in knowing that there is a community of people working alongside you to learn more about this disease.

Study Highlights

- Most of the survey respondents reported symptoms beginning within the first year of the patient's life
- 63% of the patients were diagnosed before they were 3 years of age
- 80% of patients had genetic testing done to confirm their Dravet diagnosis
- 58% of patients were diagnosed with a different or general seizure disorder prior to receiving a confirmed Dravet diagnosis
- More than 27% reported not being satisfied at all with the current quality of life
- Approximately 60% reported low to moderate degrees of seizure control
- 58% said they were starting to pursue or actively pursuing new treatment options



ABOUT THE STUDY

Overview and Methodology

The data for this analysis was collected using an online survey of 492 parents and caregivers of patients living in the United States with a reported diagnosis of Dravet syndrome.

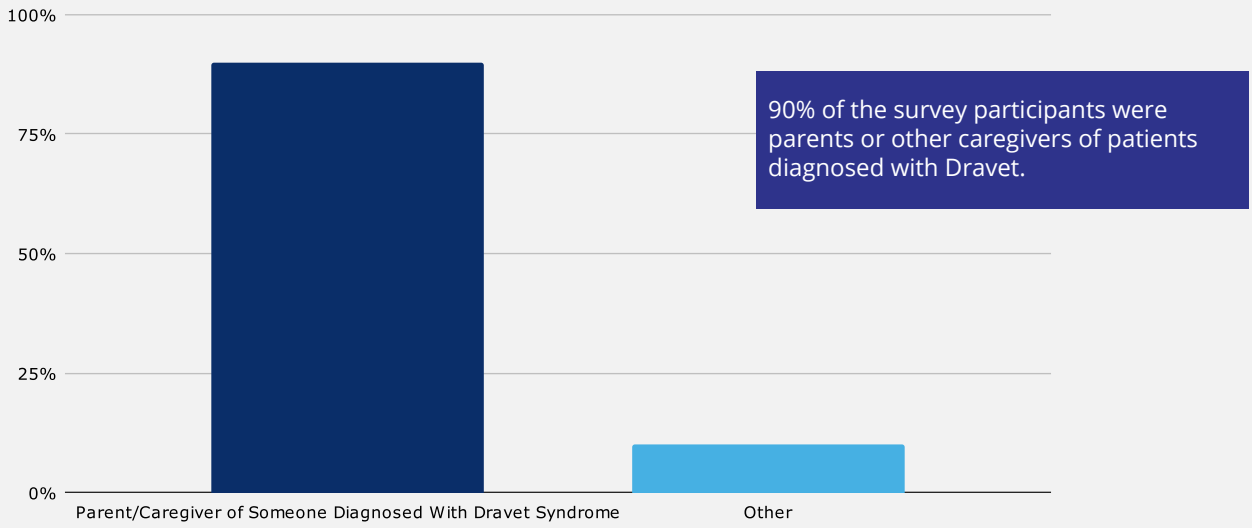
Key Areas of Research

- Symptom history
- Impact of the disease on daily life
- Experiences obtaining a confirmed diagnosis
- Familial history of seizures
- Seizure “triggers”
- Challenges, frustrations, and support needs
- Valued sources of information
- Experience with various treatments
- Seizures and how well they are controlled

ABOUT THE STUDY

A Closer Look at Who Participated in This Study

The Dravet survey respondents were a blend of parents, other caregivers, and healthcare professionals.

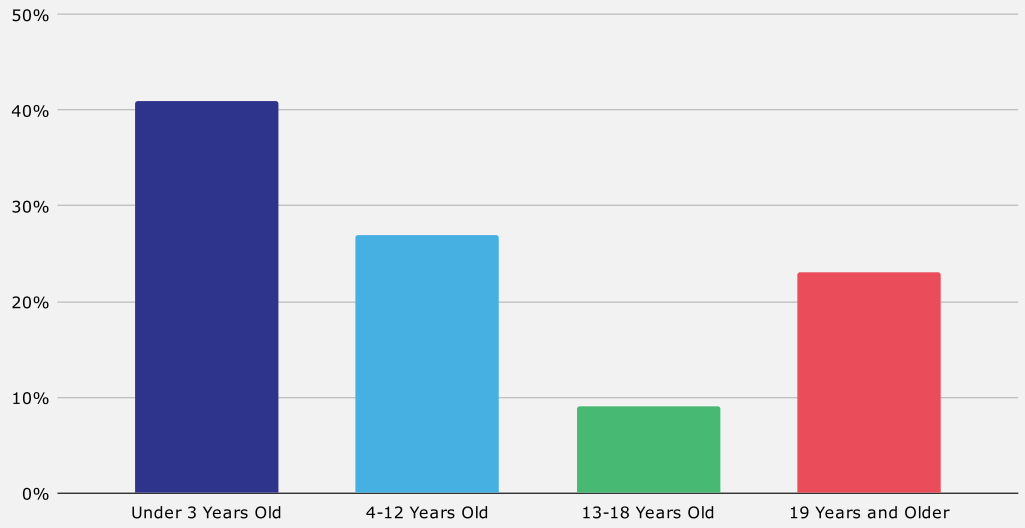


Input From a Broad Range of People Whose Lives Have Been Impacted by Dravet

- Parents
- Grandparents
- Aunts/Uncles
- Family Friends
- Siblings
- Cousins
- Healthcare Professionals

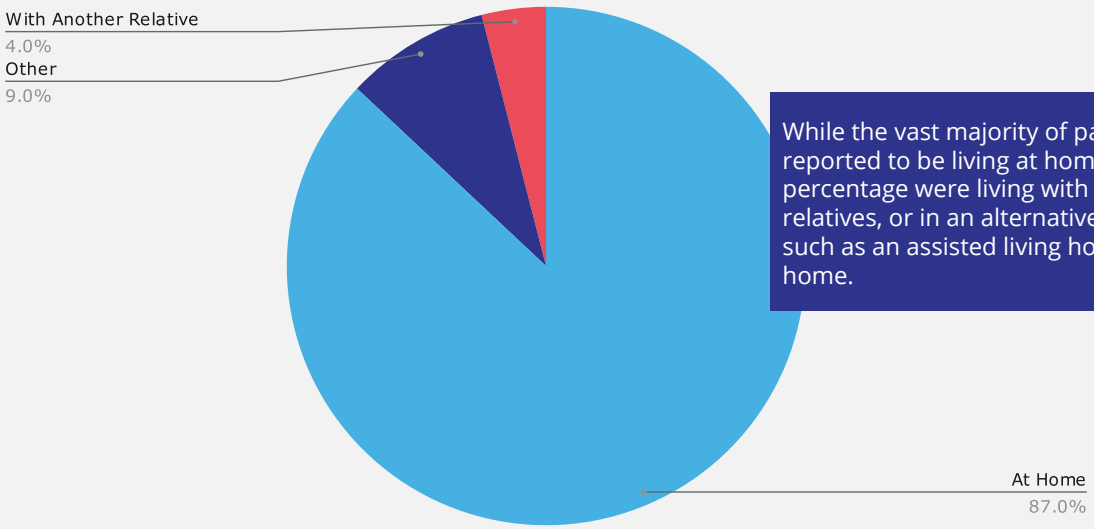
Patient Age at the Time of This Study

The patients represented in this survey are distributed across all age groups from under 3 to over 18 years old. Currently, most (41%) are children that are under 3 years of age.



Living Situation

Most Dravet patients referred to in this study were living at home with their parents at the time the survey was taken.



While the vast majority of patients were reported to be living at home, a small percentage were living with other relatives, or in an alternative facility such as an assisted living home or group home.

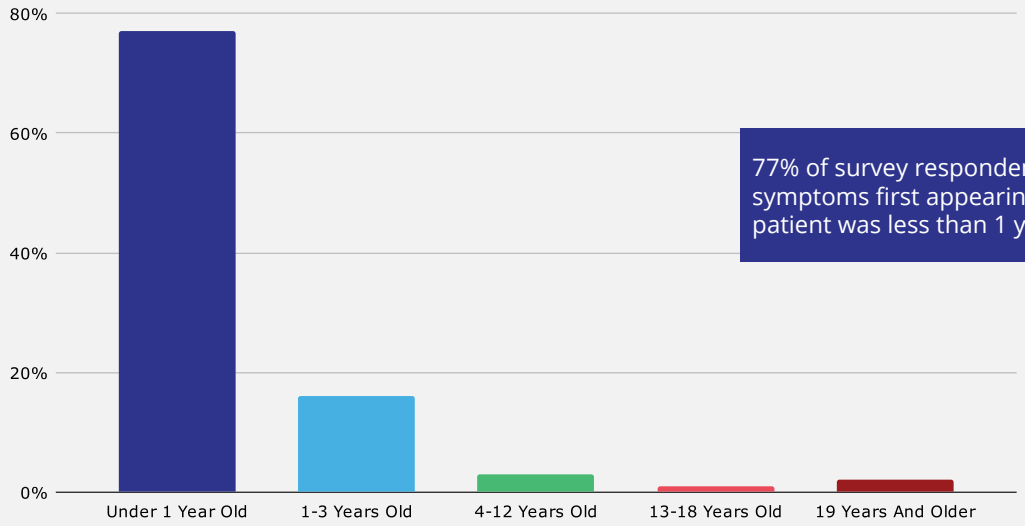
Early Symptoms

Most survey respondents recalled noticing the following initial symptoms of Dravet. More information about the group's experiences with diagnosis can be found on page 8.

Seizures	Heat, Fever, etc.	Development /Cognitive	Behavioral Issues	Chronic Infections
86%	42%	33%	22%	16%

Percentage of survey respondents who recalled these symptoms occurring.

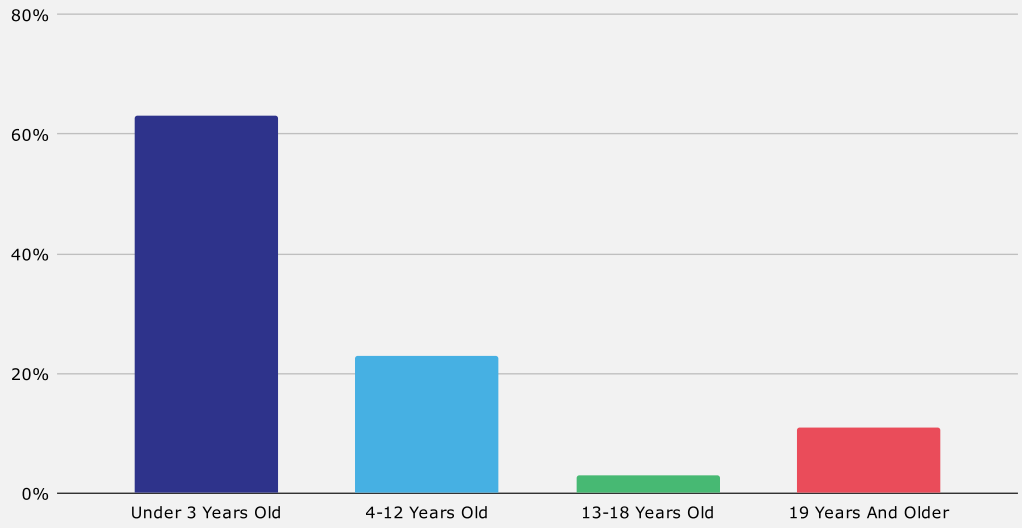
Patient Age When Symptoms First Appeared



77% of survey respondents recalled the symptoms first appearing when the patient was less than 1 year old.

Patient Age at Diagnosis

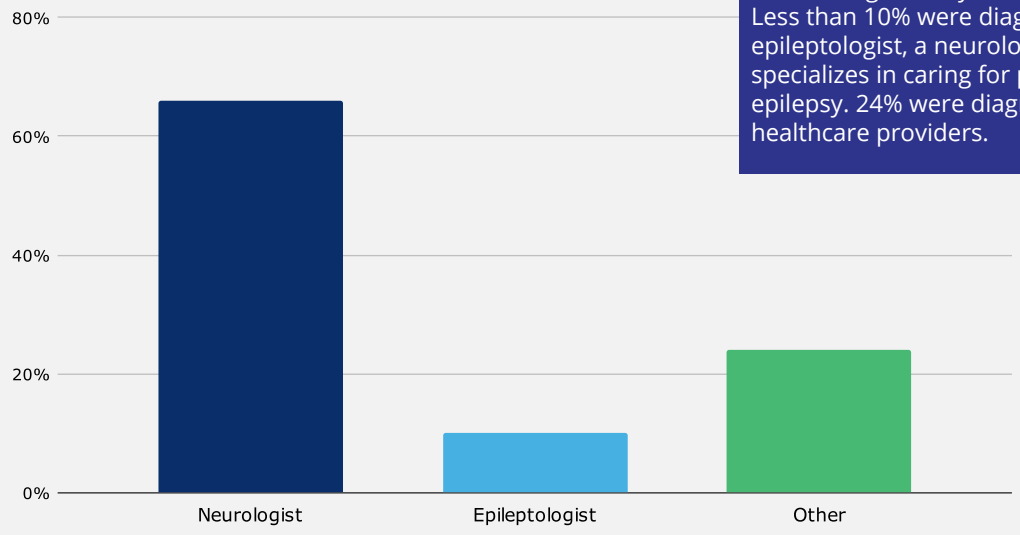
Virtually all of the patients referred to in this survey were diagnosed after birth, with most diagnosed before they were 3 years of age.



Diagnosing Physician

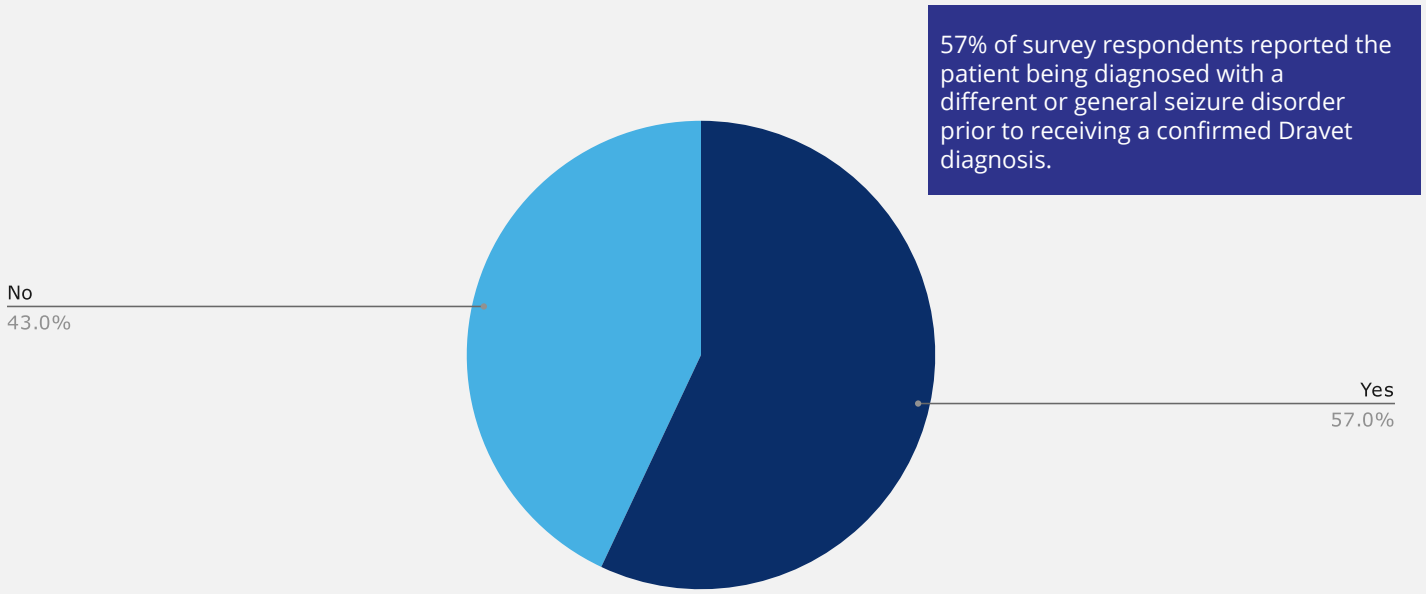
66% of patients received a confirmed Dravet diagnosis by their neurologist.

Most patients (66%) received a confirmed Dravet diagnosis by their neurologist. Less than 10% were diagnosed by an epileptologist, a neurologist who specializes in caring for people with epilepsy. 24% were diagnosed by other healthcare providers.



Other Diagnoses

Almost 60% of the patients referred to in this study were diagnosed with a different or general seizure disorder prior to being diagnosed with Dravet.

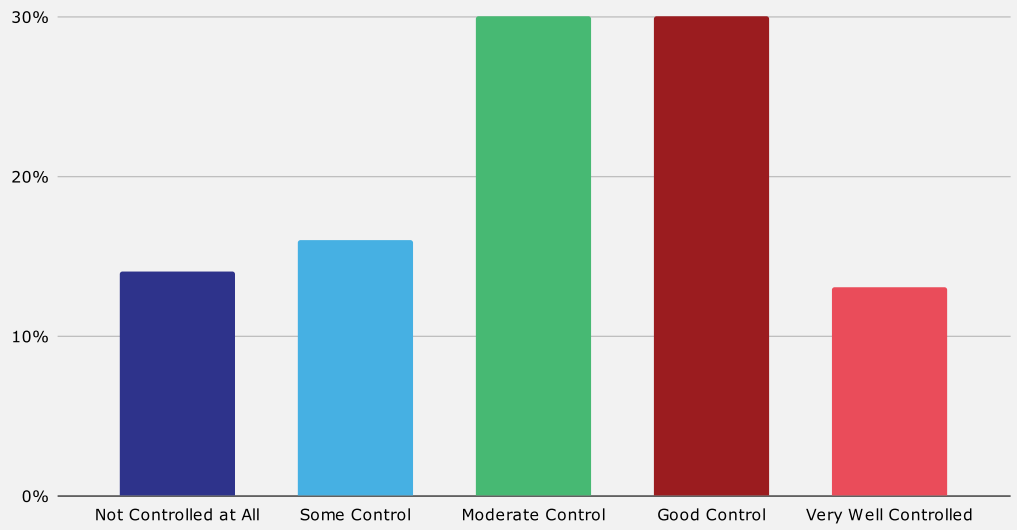


Prior Diagnoses Mentioned in This Study

- Epilepsy
- Infantile spasms
- Inoperable tumors on the brain
- Benign myocolonic epilepsy of infancy
- Febrile seizures
- Mucopolysacridosis
- Schizencephaly
- Focal epilepsy
- Lennox-Gastout syndrome, (LGS)
- Severe epilepsy of childhood

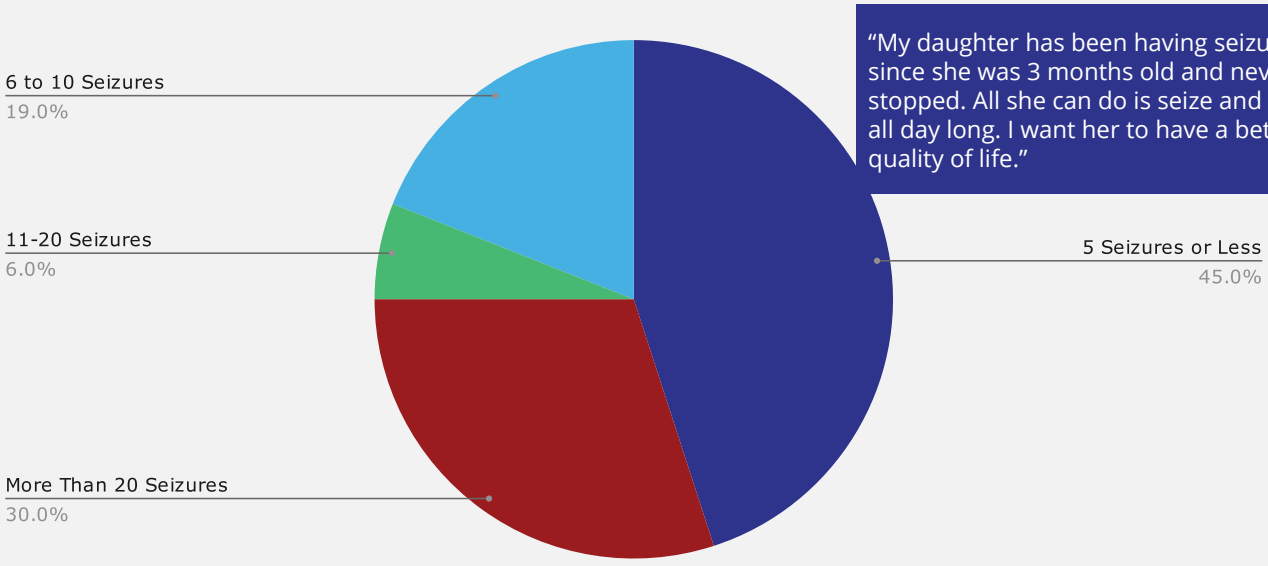
Current Level of Seizure Control

Almost 60% of survey respndents reported low to moderate degrees of seizure control, with 10% saying the seizures were “not controlled at all.”



Number of Seizures Experienced in an Average Month

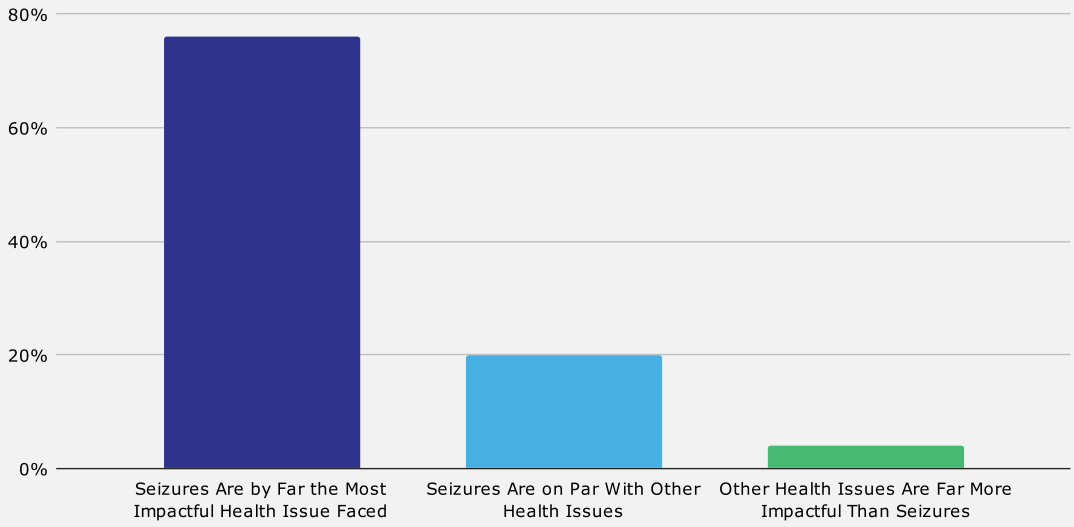
30% of survey respondents reported more than 20 seizures per month.



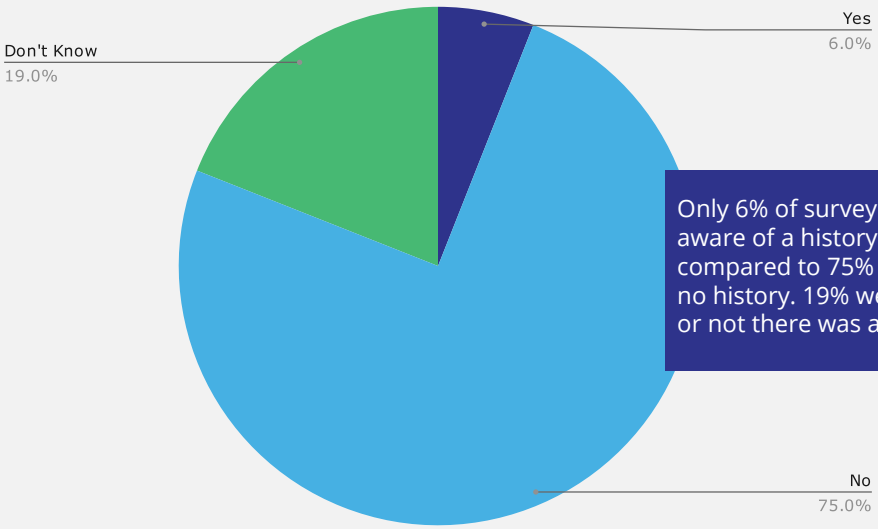
“My daughter has been having seizures since she was 3 months old and never stopped. All she can do is seize and sleep all day long. I want her to have a better quality of life.”

How Seizures Relate to Other Health Concerns

Only 20% of survey respondents felt that the seizures associated with Dravet were equally impactful versus other health issues resulting from the disease. 76% found the seizures to be by far the most impactful health issue they faced.



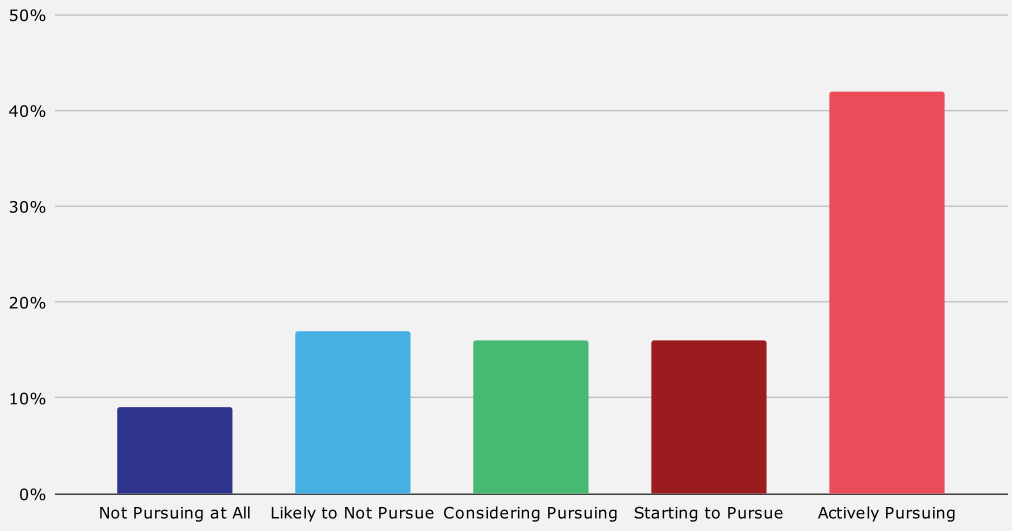
Family History of Dravet



Only 6% of survey respondents were aware of a history of Dravet in the family, compared to 75% who believed there was no history. 19% were unsure of whether or not there was a family history.

Looking for New/Different Seizure Treatments

Survey responses about pursuing new seizure treatments were mixed but leaned heavily toward pursuing new options. 9% said they were not pursuing new options at this time, while 58% said they were starting to pursue or actively pursuing new options.



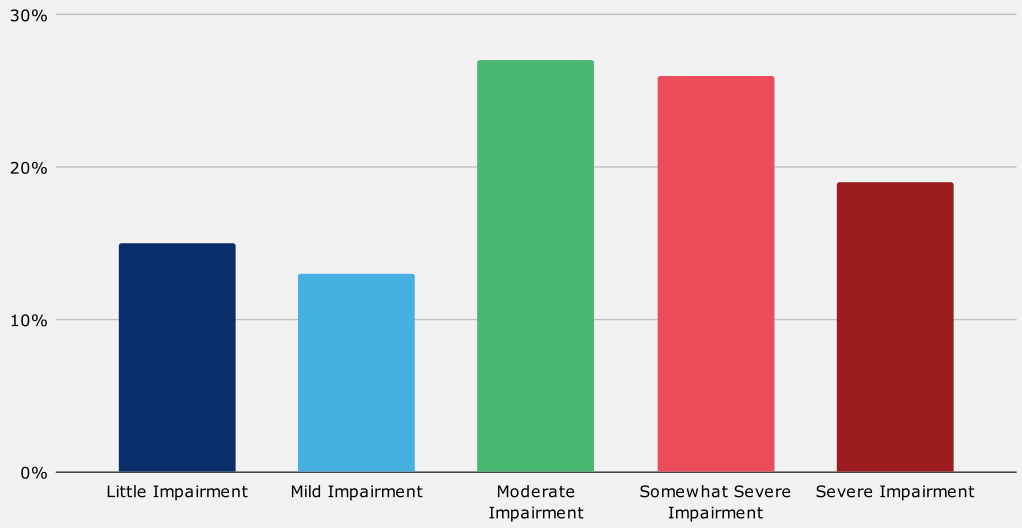
Treatments Used to Manage Seizures

Prescription Medications	Diet	Non-FDA-Approved CBD Available Online, in Stores, or in Dispensaries	Surgery (Including VNS or Implants)	Other
96%	33%	29%	25%	3%

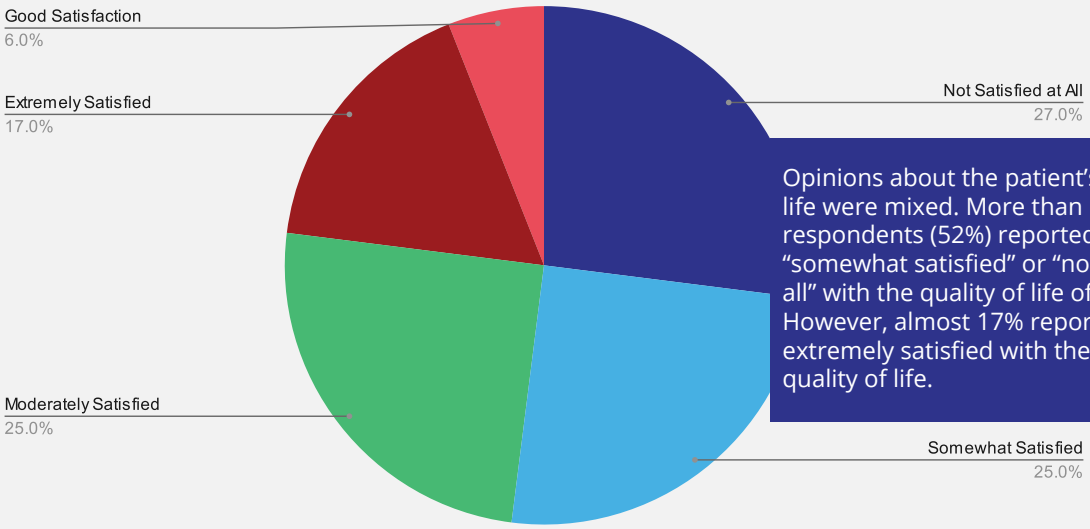
Percentage of survey respondents who mentioned these treatments.

Impairment of the Dravet Patient

Survey respondents provided their view of the impact Dravet has had. About 45% of the survey respondents felt the disease results in somewhat severe to severe impairment on the patient's life. Almost 40% felt the disease resulted in mild to moderate impairment. 15% felt the disease had little impairment on the patient's life.



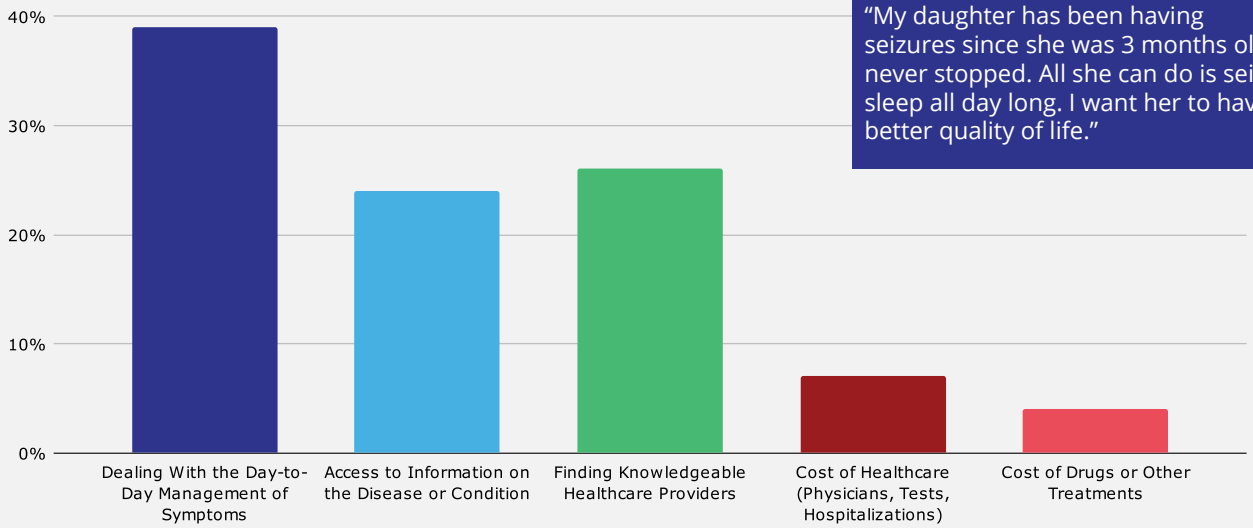
Quality of Life for the Dravet Patient



Opinions about the patient's quality of life were mixed. More than half of survey respondents (52%) reported being only "somewhat satisfied" or "not satisfied at all" with the quality of life of the patient. However, almost 17% reported being extremely satisfied with their current quality of life.

Challenges Caring for Someone With Dravet

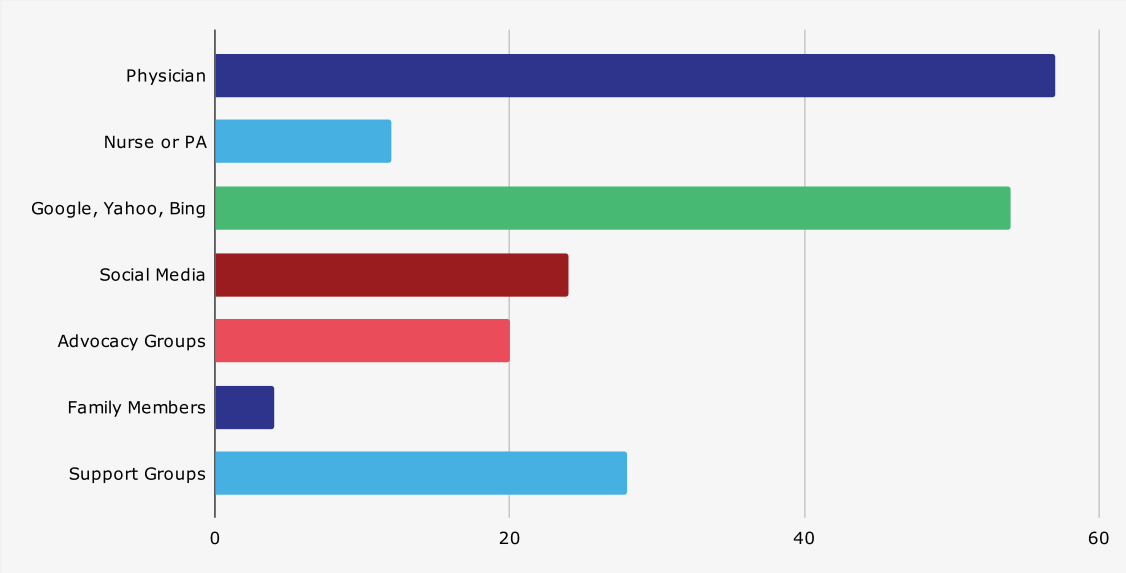
Dealing with the day-today management of symptoms is considered to be the biggest challenge in living with Dravet.



"My daughter has been having seizures since she was 3 months old and never stopped. All she can do is seize and sleep all day long. I want her to have a better quality of life."

Valued Sources of Information

Not surprisingly, physicians and online search engines were mentioned most frequently as valued sources of information. Support groups, social media, and social advocacy groups were mentioned next most frequently. Nurses/PAs and family members were mentioned least frequently.



QUOTES

We'd like to thank many of our survey respondents for sharing their feelings and emotions with us. It is our hope that through these quotes, others will find comfort in knowing their experiences with Dravet are shared by others, and that they are not alone.

"Very hard for the family and **no quality of life**. We are always looking for **other ways** to better help her."

"**It's hard to find someone who knows about it** where I live and then they refuse saying they know nothing about it."

"There is currently no cure, just a **guessing game** of what medication combinations work. I am always hopeful for **new treatments**."

"She hasn't had a GTC since December 2010. She hasn't had complex partial seizures in 2 years."

"We are looking for **clinical trials** specifically gene therapy."

"My daughter has been **having seizures** since she was 3 months old and never stopped. All she can do is seize and sleep **all day long**. I want her to have a better quality of life."

"She's my baby girl. **Hoping new seizure treatments** will become available."

"Satisfied I can handle it. I just **wish we could do more**."

"I feel as if **we are stuck** where we are and **I'm scared** to make medication changes because he seizes so easily."

"**Researching online** for alternatives, actively trying new supplements/ workflows/ for her."

"Would like to have controlled seizures. I want a much **better treatment plan**."

"Looking for new treatments. We feel we have **exhausted most options available.**"

SUMMARY

Dravet syndrome, like many rare diseases, is clearly a challenging and difficult disorder to live with. Each individual's experience is different, and life after diagnosis has many challenges for the patients and their families and friends. This study, which includes experiences from 490 parents and other caregivers, captures the group's experiences across the diagnostic and disease management process.

Dravet has clearly impacted our survey respondents early in life. Approximately 63% of the patients in this study were diagnosed before they were 3 years old, with more than 77% first experiencing symptoms before they were 1 year old. Seizures, developmental delays, behavioral issues, and sensitivity to heat were the most common symptoms which led to seeking medical care and ultimately to a confirmed Dravet diagnosis. Further, many patients were diagnosed with a different seizure disorder prior to being diagnosed with Dravet.

Clearly, a significant challenge faced by the survey respondents is managing the seizures associated with Dravet. 75% felt that seizures are by far the most impactful health issue faced. 42% of survey respondents reported actively looking for new and different treatments.

Finally, survey respondents voiced a high degree of dissatisfaction with the quality of life of the Dravet patient. Approximately 32% of survey respondents reported not being satisfied at all, or only somewhat satisfied with the quality of life of the Dravet patient. These findings underscore the need for new and different approaches to managing this disease.

ABOUT PATIENT JOURNEYS

At Patient Journeys, our mission is to support rare disease patients and their caregivers by helping them access the best care possible. We believe, that by sharing information and experiences with each other, we can accelerate the diagnostic process and help patients receive the right treatment sooner.

Patient Journeys was started by the parents of children living with rare disorders who have experienced first-hand the challenges that exist with getting accurate and relevant information, and the critical role patients and caregivers can play to help support others in need.

If you have any questions or comments, please email us at info@patientjourneys.org.