

DRAVET SYNDROME FOUNDATION

5 YEAR STRATEGIC PLAN 2023 - 2028



**Dravet
Syndrome
Foundation**



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WELCOME

It all started in 2009, when four parents came together looking for connection with one another and a desire to improve outcomes for their own children who had been diagnosed with Dravet syndrome (DS). Now, 14 years later, there have been many exciting advancements in the field of DS, many thanks in part to DSF. We have positioned ourselves as a convener to bring together patient families, clinicians, researchers, and industry members to help move the field forward and we are proud to be the largest nongovernmental funder of Dravet syndrome research, worldwide.

We are closer than ever before to therapies that are truly disease-modifying; therapies that will address the cause of Dravet syndrome and have the potential to improve the spectrum of symptoms that patients experience. There are many animal and cell models that allow researchers to study Dravet syndrome, and DSF is working with researchers to close final gaps in the development of and access to these research tools. Our understanding of the SCN1A gene and the mutations that cause Dravet syndrome has expanded, leading to more precise diagnostics as well as uncovering how this impacts brain development and spurring novel avenues to genetic-based therapies. We continue to build on the knowledge that exists of the progression of symptoms in Dravet syndrome, and DSF is investing in efforts to more closely characterize the clinical picture with the intention that this will improve clinical recognition, outcomes, and treatments.

With these exciting advancements, I am proud to share with you our new 5-year strategic plan. It includes strategic focus areas that were identified by our community stakeholders, with objectives developed to address the four priorities outlined in our 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.

Community. Research. Progress.

Mary Anne Meskis

Executive Director

WELCOME



The purpose of our 2023-2028 strategic plan is to articulate the long-range direction and priorities for DSF, and clarify our mission and goals.

The content for this strategic plan was derived by examining the current Dravet syndrome landscape. We listened to a broad range of input from key stakeholders within our community regarding current community priorities, emerging needs, and organizational strengths and vulnerabilities.

The objectives and strategies described in this plan have been reviewed and approved by our Board of Directors, Scientific Advisory Board, and Medical Advisory Board. These strategies will guide DSF's efforts and investments over the next five years and will balance our current and future needs.

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OUR MISSION

The mission of Dravet Syndrome Foundation (DSF) is 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.

WHAT IS DRAVET SYNDROME?

Dravet syndrome is a rare, catastrophic, lifelong form of epilepsy that begins in the first year of life, with frequent and/or prolonged seizures. It affects 1:15,700 individuals, over 80% of whom have a mutation in their SCN1A gene. While seizures persist, other comorbidities such as developmental delays and abnormal EEGs are often not evident until the 2nd or 3rd year of life. Common issues associated with DS include:

- Prolonged and/or frequent seizures
- Behavioral & developmental delays
- Movement & balance issues
- Orthopedic conditions
- Delayed language & speech issues
- Growth & nutrition issues
- Sleeping difficulties
- Chronic infections



Patients with Dravet syndrome face a 15-20% mortality rate due to SUDEP (Sudden Unexpected Death in Epilepsy), prolonged seizures, seizure-related accidents (such as drowning), and infections.

Current treatment options are limited, and the constant care required for someone suffering from Dravet syndrome can severely impact the quality of life for both the patient and their family. Receiving a diagnosis of Dravet syndrome can be overwhelming and leaves families with many unanswered questions while they adjust to the many needs of their loved one. Because Dravet syndrome is a rare disease, many medical professionals are not as familiar with the disease, leaving families to learn all they can to ensure their child is receiving the best care.

Life with Dravet syndrome is difficult. Research for a cure offers patients and families hope for a better quality of life for their loved ones.

ACHIEVEMENTS & RESEARCH OVERVIEW



Since our inception, Dravet Syndrome Foundation has made significant strides in uniting the Dravet community and funding research. From 2010-2023, DSF granted awards totaling more than \$9.1M for Dravet-specific research grants and postdoctoral fellowship awards. By uniting our patient community and supporting early-stage research, we have brought Dravet syndrome to the forefront - increasing interest among researchers, expanding funding mechanisms, and broadening the research community.

| RESEARCH | AWARENESS & SUPPORT |
|---|--|
| Over \$9.1M in Research Funding | 5 Biennial Family & Professional Conferences |
| 14 Research Roundtables | \$250K+ in Patient Assistance Grants |
| International Treatment Consensus | DSF Family Network |
| Increased Research Funding Categories and Amounts | Day of Dravet Regional Workshops |

RESEARCH OVERVIEW

In the US, most biomedical research is funded through the National Institutes of Health (NIH), which awards large grants of \$100K-\$400K per project per year to highly qualified researchers. Epilepsy has traditionally been underfunded, receiving just one-quarter of the research dollars awarded to breast cancer, despite a similar incidence. As a rare form of epilepsy, DS projects received just over \$9M total from the NIH between 2003 and 2009 before DSF was founded, and few studies were being published each year.

DSF began awarding small research grants in 2010 of up to \$150K in the hopes of drawing researchers into DS and establishing dedicated laboratories throughout the US. Interest in DS has since grown exponentially. The NIH steadily increased their spending each year from 2010 to 2017, totaling over \$53M on DS research. From 2018-2023, the NIH further increased spending, directing over \$10M yearly to DS-related research. DSF-funded researchers have subsequently secured over \$30M in NIH funding for projects focused on DS or related epilepsies.

RESEARCH OVERVIEW - CONTINUED



DSF’s strategic \$9.185M investment has broadened the research community, increased the importance of DS under other funding mechanisms, and made a lasting impact that sustains research growth.

As of 2023, DSF has hosted 14 annual Research Roundtables, which bring together researchers, clinicians, geneticists and other professionals with a strong interest in DS and epilepsy for an evening of presentations, group discussion, and brainstorming about where research needs to go to advance knowledge toward a cure. What started as a meeting of approximately 30 researchers has grown to almost 200 attendees in 2023.

These Roundtables, led by DSF’s highly esteemed Scientific Advisory Board, have been extremely successful in drawing top scientists and clinicians into the fold, prioritizing DS to be at the forefront of conversations about epilepsy research throughout the year. They have also allowed DSF the opportunity to establish a Research Roadmap to guide us in funding research projects that address the critical challenges of the syndrome and which will offer the most promising breakthroughs at the fastest pace.

| RESEARCH AREA | \$ USD |
|--|-------------|
| SUDEP (Sudden Unexplained Death in Epilepsy) | \$1,180,000 |
| Gene Therapy | \$1,630,000 |
| Drug Discovery, Screening or Treatment | \$1,210,000 |
| Genetics | \$1,650,000 |
| Epidemiology | \$1,120,000 |
| Neuronal Network | \$1,440,000 |
| Other | \$ 955,000 |
| Total | \$9,185,000 |





As a rare disease, awareness of Dravet syndrome is severely lacking among health care providers, as well as the general public. Access to specialists can be challenging often with long distances between patients and specialists. More knowledge is needed about Dravet syndrome so that patients are diagnosed early, receive appropriate treatment, and are allowed to have the best quality of life possible.

When our community bands together to raise awareness, we have the opportunity to advocate so that more money can be raised for research, change legislation, and encourage pharmaceutical companies to develop drugs that will better treat Dravet syndrome.

DSF leads several initiatives to offer awareness and education for families, professionals, and the general public, including:

- DSF Biennial Conference
- Day of Dravet Workshops
- Dravet Awareness Month (June)
- Social Media Campaigns
- DSF Representation at Professional Meetings
- Educational Resources and Webinars
- Voice of the Patient Report



SUPPORT & ADVOCACY



DSF offers assistance to families of patients throughout the entire medical journey, covering aspects such as diagnosis, symptom management, educational support, accessing suitable clinical care, and facilitating connections with other families who share similar experiences. Our range of programs and educational resources are crafted by patient families, for patient families, and include:

- DSF Family Network
- Family Ambassador Program
- Newly Diagnosed Patient Kits
- Private Caregiver Support Groups
- Patient Assistance Grants
- Caregiver Connect Modules
- Sibling Resources
- Transition of Care Resources
- Virtual Support Sessions
- Educational Webinars
- Bereavement Support
- Legislative Advocacy Program (new in 2024)



Patient families who connect with DSF and participate in our programs are empowered and confident, allowing them to advocate for the best possible care for their loved one as well as on behalf of others living with Dravet syndrome.

Having the opportunity to connect with other patient families also eases isolation and allows the opportunity for families to learn from one another, share their experiences, and forge life-long community connections.



DSF Family Network & Patient Support Groups: Register so your family is counted and connected. Members receive updates on new clinical trials, educational events, and fundraising events near them. www.dsffamilynetwork.org

Biennial Conference: Attend our 3-day event for the unique opportunity to connect with and learn from other families, medical professionals, researchers, and industry partners. www.dsconference.org

Research Roundtable: DSF's Annual Research Roundtable is a unique event that brings together leading experts in Dravet syndrome from across academic research, clinical care, and industry to present cutting-edge research advancements. www.dsresearchroundtable.org

Research Grants: These seed grants offer funding for research directly related to Dravet syndrome and fund initial research hypotheses that have not been fully explored. www.dsresearchgrants.org

Day of Dravet Workshops: Learn about the latest in research and treatments, as well as connect with local families at these biennial one-day regional events. www.dayofdravet.org

DSF Patient Assistance Grants: Learn about grants for necessary medical equipment, therapy devices, and educational aides not covered through private insurance or other assistance programs. www.dsfpatientassistance.org

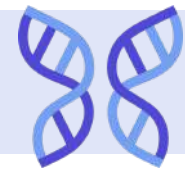
Birthday Buddies: Register your loved one with Dravet syndrome and for their birthday they will receive a birthday card and a small gift from DSF's mascot, Aurora. www.dsfbirthdaybuddies.org

Sibling Resources: Explore resources created based on research and inspiration from the community, with guidance from health care providers specializing in Dravet syndrome and mental health experts. www.dsfsupersibs.org

Newly Diagnosed Kits: DSF offers a kit for newly diagnosed families that includes a guidebook for families after diagnosis, as well as a medication bag and other materials to ensure that families have the knowledge, tools, and resources they need for their child's care.

Bereavement Support: Losing someone you love or care about, particularly a child, is very painful. DSF offers information and resources for coping with grief and loss including a Bereavement Support Group and Remembrance Wall.

STRATEGIC OBJECTIVES



Dravet Syndrome Foundation has developed four strategic drivers with associated objectives. Our strategic plan includes resource plans, timelines, and measurable outcomes. Our strategic drivers are the guiding force behind our five-year plan and each category is critical in shaping the future and direction of DSF and advancing our mission.



IMPROVE ACCESS & QUALITY OF CARE



SUPPORT & EMPOWER CONSTITUENTS



ACCELERATE RESEARCH



BUILD & STRENGTHEN ORGANIZATION



IMPROVE ACCESS & QUALITY OF CARE

Through collaboration and communication with our research community, we can improve the timeline for better treatments and a cure. By supporting opportunities that allow for greater education of Dravet syndrome throughout the healthcare system, we can increase diagnosis and assure the best quality of care.

| Objective | Initiatives |
|---|--|
| Increase partnerships with industry | <ul style="list-style-type: none"> • Biannual Industry Stakeholders Meeting |
| Increase payer engagement | <ul style="list-style-type: none"> • Explore and understand current landscape • Actively engage with other patient advocacy organizations and trade associations |
| Identify and engage with pediatric and adult neurology providers, particularly those in rural areas | <ul style="list-style-type: none"> • Collaborate with pharmaceutical reps to raise awareness of DSF & its programs |
| Increase engagement with healthcare providers, including around DSF programs and services | <ul style="list-style-type: none"> • Annual Comprehensive Care Center webinar and/or additional outreach • Collaboration with industry reps to share materials at office visits • AdWords and/or direct mail campaign |

STRATEGIC OBJECTIVES



SUPPORT & EMPOWER CONSTITUENTS

We will continue to support patients and families with disease education and advocacy needs through maintenance and expansion of patient resources, as well as family-to-family support.

| Objective | Initiatives |
|--|---|
| Provide continued opportunities for community engagement and connection | <ul style="list-style-type: none">• Increase number of volunteers in our Family Ambassador program, with a minimum of 3 per region for a total of 15• Increase number of awarded Caregiver Connect Grants for patient family gatherings• Increase attendance at in-person educational events (Conference & Day of Dravet) |
| Create and provide educational programs and resources for patient families | <ul style="list-style-type: none">• Host Biennial Conference, with Day of Dravet workshops on alternate years• Host monthly virtual meet-ups• Host quarterly webinars on DSF programs and caregiver needs |
| Expand outreach to better serve underrepresented and underserved communities to ensure diversity, equity, and inclusion are at the forefront of our organization's culture | <ul style="list-style-type: none">• Annual DEI Training for Board & Staff• Increase use of Spanish-speaking resources |
| Stay abreast of and advocate for legislative issues affecting individuals with DS and their families | <ul style="list-style-type: none">• Development of Legislative & Advocacy Training Program for patient families, including 1-2 yearly educational initiatives• Development of Legislative & Advocacy Ambassador program |
| Continue to identify and address the unmet needs of caregivers of adult patients | <ul style="list-style-type: none">• Annual Family Network survey• Increase engagement with adult providers |

STRATEGIC OBJECTIVES



ACCELERATE RESEARCH

Our steadfast commitment to advancing Dravet syndrome research has helped expand our understanding of Dravet syndrome and enabled three new treatments, with several other clinical trials currently underway.

| Objective | Initiatives |
|---|--|
| Invest \$5+ million in research grant awards over the next five years | <ul style="list-style-type: none"> • Annual grant application period • Requests For Applications for special research focus areas |
| Increase funding for clinical research that directly studies patients with DS, including a focus on the full spectrum of symptoms, treatments, long-term outcomes, and quality of life | <ul style="list-style-type: none"> • Establish working groups that bring together thought leaders to begin discussing unmet needs and potential approaches. |
| Host annual Research Roundtable to bring together researchers, geneticists, neurologists, industry partners, and other professionals with a strong interest in Dravet syndrome for updates and collaborations | <ul style="list-style-type: none"> • Plan and execute annual meeting |
| Ensure patient community is educated about the state of and importance of research, as well as engaged in opportunities to participate in research studies | <ul style="list-style-type: none"> • Ongoing educational events and awareness around topic |
| Fund research that leads toward disease-modifying therapies including studies focused on mechanisms of SCN1A regulation, novel approaches to genetic therapy delivery, and discovery of the effects of therapeutically increasing SCN1A across cellular subsets and across varied developmental windows | <ul style="list-style-type: none"> • Annual grant application period • Requests For Applications for special research focus areas |
| Investigate methods to provide more evidence-based recommendations on optimal treatment choices for patients with DS | <ul style="list-style-type: none"> • Continued engagement with our Medical and Scientific Advisory Boards and research community |

STRATEGIC OBJECTIVES



ACCELERATE RESEARCH - CONTINUED

| Objective | Initiatives |
|---|---|
| Support research that investigates novel small molecules that have the potential to effectively treat seizures or other comorbidities of DS through investigation in model systems or pilot human studies | <ul style="list-style-type: none"> Continued engagement with our Scientific Advisory Board and research community |
| Expand funding to further support predoctoral and postdoctoral trainees | <ul style="list-style-type: none"> Increase postdoctoral funding to 2 years Add funding for predoctoral trainees Increase professional meeting sponsorships that assist with early investigator travel |
| Support researchers as they explore new animal models or further develop iPSCs and organoids to study DS | <ul style="list-style-type: none"> Continued engagement with our Scientific Advisory Board and research community |
| Support the development of research resources that can be shared broadly for future lines of inquiry, including facilitating the deposit of datasets, model organisms, and research tools to appropriate repositories | <ul style="list-style-type: none"> Commercial biobank of patient-derived induced pluripotent stem cell (iPSC) lines Ontology database Support building of databases of clinical information |
| Support research that explores mechanisms related to SUDEP, including respiratory and cardiac function | <ul style="list-style-type: none"> Continued engagement with our Scientific Advisory Board and research community Continued engagement with and support of PAME (Partners Against Mortality in Epilepsy) |
| Support research that explores the mechanisms of DS and identification of potential pathways for treatment | <ul style="list-style-type: none"> Continued engagement with our Scientific Advisory Board and research community |

STRATEGIC OBJECTIVES



BUILD & STRENGTHEN ORGANIZATION

Capacity building is an investment in the effectiveness and future sustainability of DSF, allowing us to deliver on our mission and enhancing our ability to have a positive impact on the Dravet syndrome community.

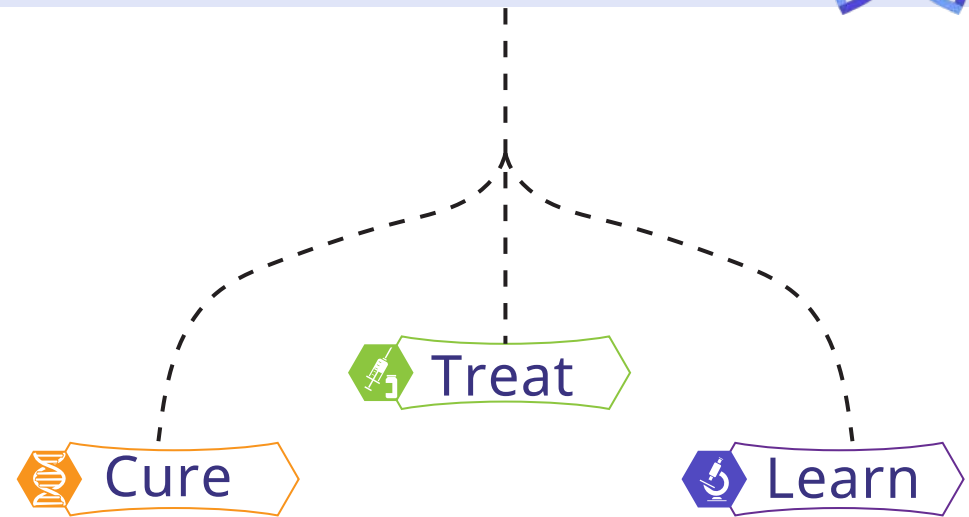
| Objective | Initiatives |
|--|---|
| <p>Maintain a 4-star Charity Navigator rating and a Candid Platinum rating, confirming our fiscal responsibility and efficiency</p> | <ul style="list-style-type: none"> • Quarterly budget comparisons to gauge progress • Annual update of Candid account listing |
| <p>Executive staff will participate/present at 20 or more diverse professional events annually, to increase our outreach/connections</p> | <ul style="list-style-type: none"> • Executive staff will continue to explore new and existing opportunities for engagement |
| <p>Recruit 1-2 new Board members annually with specific skills such as financial management and scientific/medical expertise, who reflect the diversity of our constituency and have the ability to raise substantial donations for targeted initiatives</p> | <ul style="list-style-type: none"> • Annual application period and Board Recruitment Committee interviews |
| <p>Implement in depth annual review to ensure integrity of constituent data with less than 2% duplicates by 2025 and maintain thereafter</p> | <ul style="list-style-type: none"> • Biannual clean up of CRM • Increase identification of patient and patient families in CRM |
| <p>Grow the Endowment Fund to \$10 million by 2028 and realize the maximum allowable annual contribution</p> | <ul style="list-style-type: none"> • Approach current and potential donors with a narrative of growth, strength, and greatness of mission • Increase awareness campaign and ease of participation for donors on legacy giving and qualified charitable distributions (QCDs) |
| <p>Increase staff size to assist with continued organizational growth</p> | <ul style="list-style-type: none"> • Continue to examine organizational growth and associated increased staff needs biannually |

RESEARCH PLAN



Dravet Syndrome Foundation (DSF), with the input of our community stakeholders, has developed a strategic research plan for Dravet syndrome to accelerate the research and development of new therapies.

Our 5-year strategic plan addresses the three highest priorities for research in our community. 1) Cure 2) Treat and 3) Learn. By distributing resources among these arms, DSF will continue to balance the need for progress toward a cure with the immediate need for better treatments and new pathways toward that cure.



PROGRAM 1: CURE

As a disease with a distinct genetic cause, Dravet syndrome is well-positioned for a cure based upon gene therapy or related treatments that modulate gene expression. A cure will target the underlying problem rather than simply treat seizures, hopefully providing true disease modification. The field of gene therapy is progressing quickly, but this will be a long-term investment for Dravet syndrome. Research has shown that treating the genetic mutation - which occurs in every cell of the patient's body but causes the most dysfunction in the inhibitory interneurons nestled in the brain, away from the bloodstream - is more difficult in Dravet syndrome than in other genetic disorders. DSF is committed to overcoming these barriers from the ground up, beginning

with investigating whether increased *SCN1A* reverses the course of Dravet syndrome or alleviates symptoms.

Potential cures or curative treatments for Dravet syndrome may involve:

- One-time gene therapy treatments, in which the mutated gene is corrected in the proper neurons
- Upregulation of the healthy copy of *SCN1A* or its transcripts to overcome haploinsufficiency caused by the mutated copy
- Altered regulation of other genes that compensate for the defective gene, such as other sodium channel isoforms



PROGRAM 2: TREAT

While a cure is the ultimate goal, patients need better treatments for their condition and multiple approaches may be necessary. Traditional anti-seizure medications have proven unsuccessful in controlling all seizures and reducing comorbidities in Dravet syndrome. DSF is committed to investing in drug discovery, supporting clinical research, and encouraging investigation of treatments for comorbidities associated with Dravet syndrome.

DSF will continue to help advance research to develop new treatments and improve the health and overall well-being of those with Dravet syndrome. With support from our community, we have played an instrumental role in advancing the field of Dravet syndrome by directly supporting research while encouraging and facilitating collaboration, collaborating with industry partners, and developing a clinical-trial ready community.



PROGRAM 3: LEARN

Our current understanding of Dravet syndrome is light years ahead of where it was when *SCN1A* was first associated with Dravet syndrome in 2001. With every expansion of knowledge comes the potential for new pathways for treatment and a cure, and it is this potential that strengthens DSF's commitment to supporting basic research into Dravet syndrome. Examples of the role basic science plays include the discovery in zebrafish of the effects of 5-HT receptors on Dravet syndrome (which have led to two Orphan Drugs, one of which has been designated as a Breakthrough Treatment), as well as the connection between cardiorespiratory failure and SUDEP (Sudden Unexplained Death in Epilepsy). Basic science has the power to arm researchers with preliminary data and preclinical evidence needed for pursuing larger grants from other agencies. This learning can be accomplished through several avenues including the use of:

- Traditional mouse models
- Alternative animal models such as zebrafish, drosophila (fruit fly), or other large mammal models
- Induced pluripotent stem cell (iPSC) models
- In vitro organoids developed from patient-derived cells



Community. Research. Progress.

DSF is a convener of patient families, medical professionals, researchers, and industry partners who want to improve life for those living with Dravet syndrome. Our motto explains our strengths and accomplishments. Having an educated and engaged community, along with strong organizational practices, procedures, and policies is what has brought our success and new advancements in the field of Dravet syndrome.

Thanks to our collaborative work over the last 14 years, DSF has led the way for significant advancements in the field of Dravet syndrome. Patients are now being diagnosed earlier; there is an international treatment consensus to help assure appropriate care; three new medications have received FDA approval, offering more treatment options; and there are currently 9 clinical trials underway, including treatments that offer the potential to not only control seizures, but may also offer disease reversal.

While these advancements are exciting, we still have more work to do, and we hope you will join us. As we move forward with our most recent strategic plan, we encourage you to join us. Whether that is through financial support or through donating your time and talents, our success is because of our community support. We are stronger together. Visit us at <https://dravetfoundation.org> to make a donation, host a fundraiser, learn about events near you, or volunteer.

If you have any questions, would like to offer comments on this strategic plan, or want to get more involved with DSF, please contact us at info@dravetfoundation.org.



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