

DRAVET SYNDROME FOUNDATION
Raising hope & changing lives through research

AUGUST 2020

STRATEGIC PLAN PROGRESS REPORT

In 2018, Dravet Syndrome Foundation (DSF) developed a 5-year strategic plan to articulate the long-range direction and priorities for our organization. The content for our strategic plan was derived by examining the 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.

We are pleased to offer our 2nd annual update on our progress from July 2019 - July 2020.

SPECIAL NOTE RE: COVID-19

While we could never have anticipated that 2020 would bring a health crisis of this magnitude and an upheaval of life as we know it, DSF has strategically positioned itself throughout our development to assure financial stability in times of uncertainty.

Our team has adapted quickly. While we have been forced to change some of our original plans for 2020, we have been working hard to continue to deliver the much-needed services that our community relies on, while continuing to nurture our relationships with our stakeholders, communicating transparently, and taking advantage of technology to continue to connect our community. All of our programs, including our Research Grant Awards and Patient Assistance Grants, remain intact. While our road to recovery will be long and challenging, we believe we will emerge from this crisis stronger, knowing how agile and adaptive that we were able to be without interrupting progress towards our mission.

GOAL 1: RESEARCH AND PROFESSIONAL EDUCATION

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 on Dravet syndrome throughout the healthcare system, we can increase diagnosis and assure the best quality of life.

Accomplishments from July 2019 - July 2020

- Hosted our 10th annual Research Roundtable and began planning the 11th annual event that will take place as a virtual event in December 2020.
- Hired a Research Coordinator to pursue short- and long-term research goals
- Added a 4th grant mechanism to our Research Grant Program to encourage academic pursuits of meaningful studies utilizing patient-derived data that will result in improved patient outcomes.
- Represented the patient voice in a series of educational videos on Dravet syndrome that were created by PlatformQ and NeuroSeriesLive



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GOAL 2: INCREASE REVENUE TO SUPPORT OUR PROGRAMS

Funding growth is challenging particularly for a rare disease nonprofit. By increasing and diversifying our revenue streams we can ensure we have a sustainable operating model that meets the demands of our continuously growing community, and guarantee our success and longevity.

Accomplishments from July 2019 - July 2020

Due to the unexpected impact from the pandemic, we needed to restructure and reimagine our in-person fundraising efforts for 2020.

- Hosted a new annual Casino Night fundraising event in Texas in February
- Hosted a virtual fundraiser, *Party in Your PJs for Dravet,* in place of our annual signature gala
- Turned our *Steps Toward a Cure* series of walks and 5Ks into a single, one-week long virtual event
- Developed an *Industry Partner Meeting* that will take place in September to explore new projects and other ways we can work together that will benefit the Dravet patient community

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GOAL 3: EXPAND FAMILY EDUCATION & ADVOCACY

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.

Accomplishments from July 2019 - July 2020

- Postponed our 2020 DSF Biennial Conference due to the pandemic
- Received approval for an ICD-10 Code for Dravet syndrome
- Added a new page to our website on Patient-Centered Outcomes Research
- Continued to identify physicians with experience in Dravet syndrome, for our *Find a Doctor* page
- Distributed over $15,000 through our *Patient Assistance Grant Program*
- Increased membership in our private parent support group by approximately 11%
- Developed VIP Sib Kits and associated parent resources to help navigate the complex emotions that sibs face when they have a brother or sister with Dravet syndrome
- Organized a virtual *Day of Dravet* workshop for October 17th, which will replace our annual in-person events
- Developed an *Educational Webinar Series* on topics important to the Dravet community
- Hosted twice monthly parent meet-ups to allow live face-to-face connections via Zoom



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GOAL 4: ENHANCE COMMUNITY DEVELOPMENT & COMMUNICATIONS

By increasing the leadership roles of our staff, board, volunteers, and donors we can build on current strengths through internal capabilities and resources, while strengthening our community connections. We recognize the importance of an ongoing assessment of our role within the community and the most effective use of our resources, as well as the need to continually communicate our priorities, goals, strategies, and accomplishments to inform and engage our stakeholders.

Accomplishments from July 2019 - July 2020

- Established 2020 initiatives based on the goals and objectives set by the Board of Directors
- Staff training on the development of our social media education and outreach plan
- Addition of two new board members, Ms. Ashley Kerns and Dr. Joseph Sullivan

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RESEARCH STRATEGY

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 and the immediate need for better treatments and new pathways toward that cure.

Since drafting our 5-year strategic plan in 2018, DSF has awarded an additional $790k in research grants, $390k of which has been invested into research on genetic approaches to treating Dravet syndrome. Three projects comprise the largest single focus of DSF's research dollars since the strategic plan's development.

1. **Cure:** DSF has continued to focus research on ways to cure Dravet at the genetic level. We awarded grants totaling $790k to basic science researchers attempting to correct the sodium channel insufficiency through microRNA-mediated expression, CRISPR/dCas-9 modulated expression, and alternative splicing techniques.
    - DSF continues to support Stoke Therapeutics, a biotechnology company developing an antisense oligonucleotide treatment aimed at increasing healthy SCN1A expression, to be delivered several times each year.
    - DSF also supports Encoded Therapeutics, a biotechnology company developing a one-time gene therapy that would permanently enhance expression of the remaining healthy copy of SCN1A.
    - DSF added a Clinical Research grant category ($150K/ 2 years) to encourage academic pursuits of meaningful studies utilizing patient-derived data that will result in improved patient outcomes.



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RESEARCH STRATEGY (continued)

2. **Treat:** DSF has continued to support the development of clinical treatments aimed at reducing seizures in Dravet syndrome by working with pharmaceutical and biotechnology companies to bring their treatments to the market. In 2020, Zogenix received FDA approval of fenfluramine (Fintepla) and since the Strategic Plan's inception, clinical trials have begun for the following treatments:

    - Tak-935 (soticlestat, inhibits 24-hydroxycholesterol synthesis) *Ovid presented two abstracts at American Academy of Neurology in Spring 2020 reporting promising results of decreased seizure frequency in short-term and extended clinical study arms.*

    - EPX-100 (clemizole, repurposed antihistamine, 5-HT modulation) *Announced successful completion of Phase 1 trials in January 2020 and listed Phase 2 clinical trials in July 2020 for patients with Dravet syndrome in US and Australia.*

    - EPX-200 (lorcaserin, repurposed weight-loss drug, 5-HT modulation)

    - STK-001 (TANGO, antisense oligonucleotide) *Began enrolling for Phase 1/2a clinical trials for Dravet syndrome in July 2020 and published first basic-research paper detailing their approach (Lim et al 2020 Nature Communications)*

3. **Learn:** While tremendous progress toward better treatments and curing the underlying issue have been made since 2018, there is undoubtedly much to learn about Dravet syndrome and its manifestations in humans. DSF has awarded grants aimed at understanding how SCN1A mutations affect the heart, metabolic pathways, synaptic pathways, and brain development in animal models and humans.

    - DSF funded a research project investigating novel microRNA-mediated modulation of the SCN1A gene

    - DSF added a Research Coordinator position to facilitate the pursuit of short- and long-term goals



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SHORT-TERM GOALS (1-2 years)		
Cure	**Treat**	**Learn**
Discover the effects of therapeutically increasing SCN1A expression on symptoms and disease course	Establish a Clinical Research Award	Support organoid and other models of DS
Determine whether targeting specific interneurons is necessary	Work towards establishing an ICD-10 code specific to DS	Explore mechanisms related to SUDEP, including breathing
If necessary, investigate mechanisms for specifically targeting interneurons for increasing SCN1A expression	Prepare the patient community for involvement in research	Identify and describe the natural history of DS
Investigate viruses with interneuron-specific promoter	Identify and describe the natural history of DS against which new treatments can be measured	Continue research on the mechanisms of DS and identification of potential pathways for treatment
LONG-TERM GOALS (3-5 years)		
Cure	**Treat**	**Learn**
Target SCN1A upregulation to interneurons	Identify the best interventions for seizures and comorbidities	Support researchers as they explore new models
Explore other mechanisms of increasing expression	Support clinical studies through research grants and patient engagement	Examine new imaging techniques to assess network dysfunction at the cellular level
Investigate gene therapy techniques that may prove useful in future DS studies		

Not Started
Not Done/ In Progress
Done/ Significant Progress

Accomplishments

- DSF added a Clinical Research grant category ($150K/2 years) to encourage academic pursuits of meaningful studies utilizing patient-derived data that will result in improved patient outcomes
- Supported movement to clinical trial phase for Stoke
- Participated in pre-clinical discussions with Encoded
- Awarded grant for new micro-RNA mediated expression approach
- Hired a Research Coordinator position to pursue short and long-term goals