ANNUAL REPORT

DECEMBER 2020



Dianely Cabrera listening to TCSF's Rising Tides Book Club book while caring for her son, Max.

Photo credit: Dianely Cabrera

SCNA EPILEPSY

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Katie Banks and her son Wyatt at the 2017 SCN8A Clinician, Researcher, and Family Gathering.

Photo credit: Ryan Collerd

A WORD FROM FOUNDER & DIRECTOR HILLARY SAVOIE

On The Cute Syndrome Foundation in 2020

2020 was an unbelievably difficult year, on many levels. However, it was a year that has taught many of us important lessons about priorities, community, and connection. At The Cute Syndrome Foundation we, too, have spent this year responding to these lessons.

Our SCN8A community already knows a lot about how to take a challenge, and turn it into an opportunity, and 2020's improvement surrounding our virtual engagement and fluency has absolutely been an opportunity for us, a community that was already isolated and vulnerable to viruses. Many of us were already living in quarantines of our own, well before anyone said the words COVID-19. However, 2020 provided our community with the tools and the incentive to connect, not just between ourselves as families, but also beyond.

TCSF cannot take credit for these first virtual meetings, although they were organized by one of our volunteers, Kenz Wardrope. In early 2020 Kenz gathered us together for a few informal virtual meetings on Zoom/Facebook where we met each others' children, shared our pandemic experiences around the world, and just took some time to be together. These meetings became the nugget of our later Social and Emotional Programming, funded initially by a Rising Tide Grant from Child Neurology Foundation, which provided zoom yoga classes, food and drink discussion group, and book club—as well as access to mindfulness apps and meal kits.

As our community came together to connect socially, it became clear that this venue also provided learning opportunities. So, we created a five-week Clinical Trial Readiness Educational series, two-part Pre-Gathering Educational series, and our virtual 6th Annual SCN8A Clinician, Researcher, and Family Gathering. All of which, in a world exhausted by webinars and virtual conferences, were not only well-attended, but provided deep and meaningful engagement opportunities.

Read more about these activities, and the people who made them possible, in this, our seventh Annual Report.

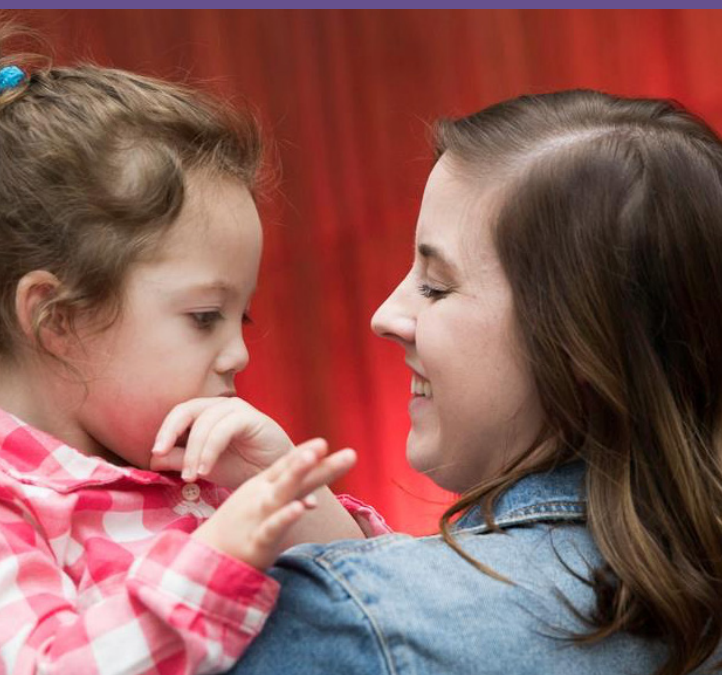


HILLARY SAVOIE

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(Below) Megan Varner holding Addy Wardrope at the 2017 SCN8A Clinician, Researcher, and Family Gathering.

Photo credit: Ryan Collerd





(Above) Kacie and Stella Craig.

Photo credit: Kacie Craig

SCN8A COMMUNITY

Megan Varner's Virtual TCSF

Megan Varner, who began volunteering with us in 2016, when she was 22, has been responsible for the graphics and tech behind almost everything TCSF has put out in the last several years. Megan has had a big lift every year. However, this year, she was undeniably our hardest working volunteer.

Megan was essentially solely responsible for all of our new virtual interfacing, from our Clinical Trial Readiness Series, Pre-Gathering Family Education Series, and our virtual Annual Clinician, Researcher, and Family Gathering. She has been the powerhouse of a virtual TCSF. She has troubleshot speaker issues on camera in the middle of events, she has engineered seamless transitions, and she has added extra events on little to no warning. She has done this all while working full time and without appearing to break a sweat...something she credits to her years of growing up around her sister Lily's medical emergencies.

Kacie Craig's Book Club

Kacie Craig is a more recent volunteer for TCSF—but she has been full-force at the job. This summer, we were awarded a Childhood Neurology Foundation Rising Tides Grant to support social and emotional programming for our SCN8A families. We offered things like meal kits and zoom yoga—however, one of our most popular offerings was a book club.

The idea to include a book club in the program was really, originally, Kacie's. She mentioned the idea at the New Orleans conference—and it took COVID and the grant application to bring that idea to fruition. When asked if she was willing to lead the book club this summer, Kacie quickly agreed. Later, when we decided to do our Clinical Trial Readiness Series, to help our families navigate a number of topics around patient data, trial design, and setbacks in drug development, the obvious choice for event host was Kacie. Kacie showed up and managed whatever such an event could throw at her—whether audio challenges, delays in getting guests over to the platform, or pesky time zone confusion—she was warm, professional, funny, and thoughtful.

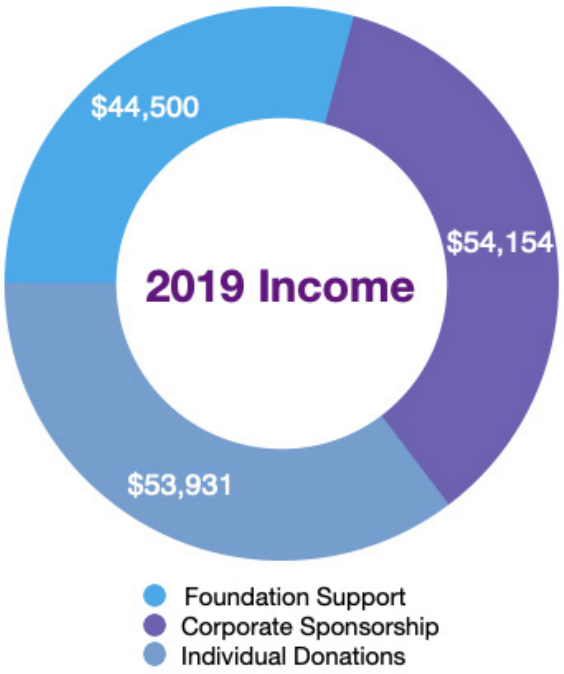
By the time she hosted our Annual Gathering in December, Kacie had the whole thing perfectly sorted!

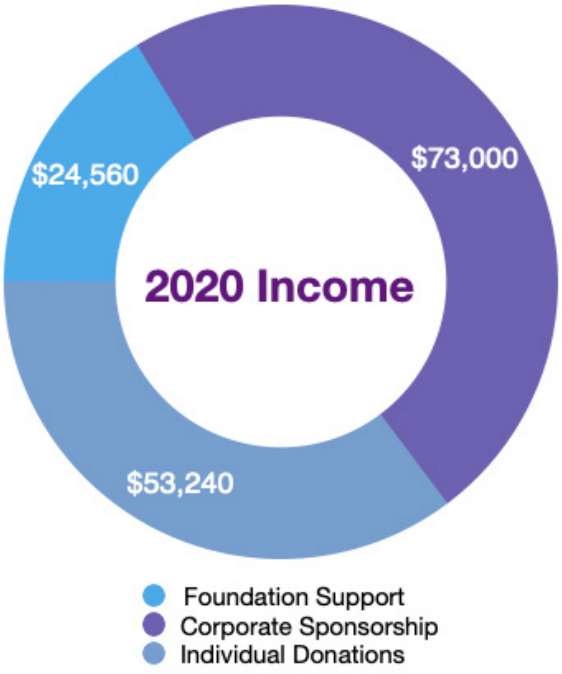
SCN8A FUNDING

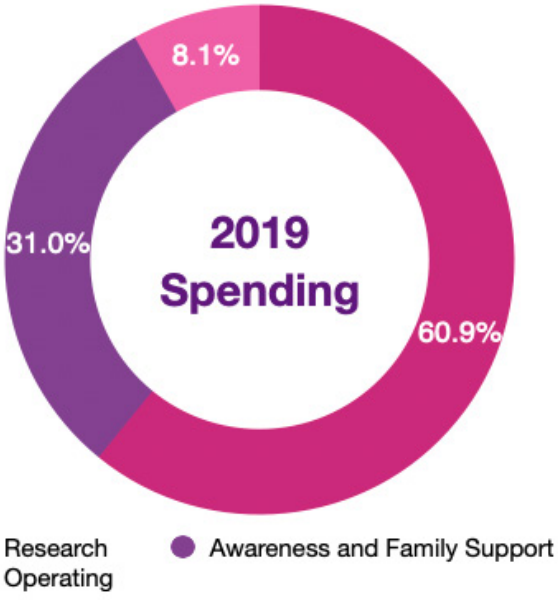
A Look at 2019 and 2020 Finances

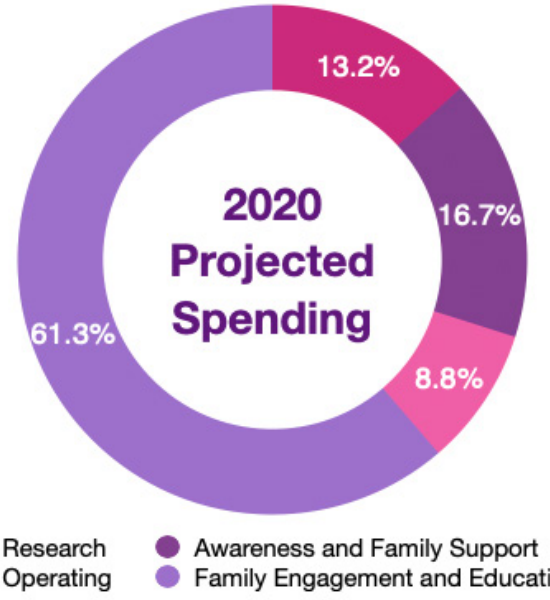
Our final 2019 numbers saw us raising a record-breaking $152,585—more than doubling our income from 2018. Our funding sources in 2019 were approximately evenly split between Foundation, Corporate, and Individual donations. Despite the pandemic, in 2020 we raised another $150,800. While we received two COVID-19 related grants in 2020 as well as funding from joint efforts with the Epilepsy Foundation of Metropolitan New York, our fundraising from foundation sources was down in 2020. However, impressively, we saw individual support for the foundation as well as a significant increase in corporate support for our programs.

While the majority of our spending in 2019 was for research—including support for research based meetings—2020 presented a different set of challenges and opportunities. In 2020 we focused our funding on programming to engage our families, prepare for upcoming clinical trials, and setting up platforms to learn more about the needs of the SCN8A community. We also managed to keep our operating expenses extremely low at 8.8% of our total budget.









SCN8A CONNECT

Social and Emotional Support

In June, The Cute Syndrome Foundation received a Rising Tides Grant from Child Neurology Foundation to lift a social and emotional support program off the ground. This program has included Zoom yoga and family martial arts classes, a food and drink discussion group, and a book club, as well as access to meal kits, mindfulness app subscriptions, and gift cards for yoga mats, books, etc. We knew this programming would be meaningful. However, as we dove into it, we began to realize just how much our community needed this—to feel supported, engaged, and connected. Also, simply, to have a bit of fun in the midst of everything.

We are so grateful to Child Neurology Foundation for not only making this program possible, but doing so in a way that allowed us to quickly and flexibly respond to our community's needs in real time.

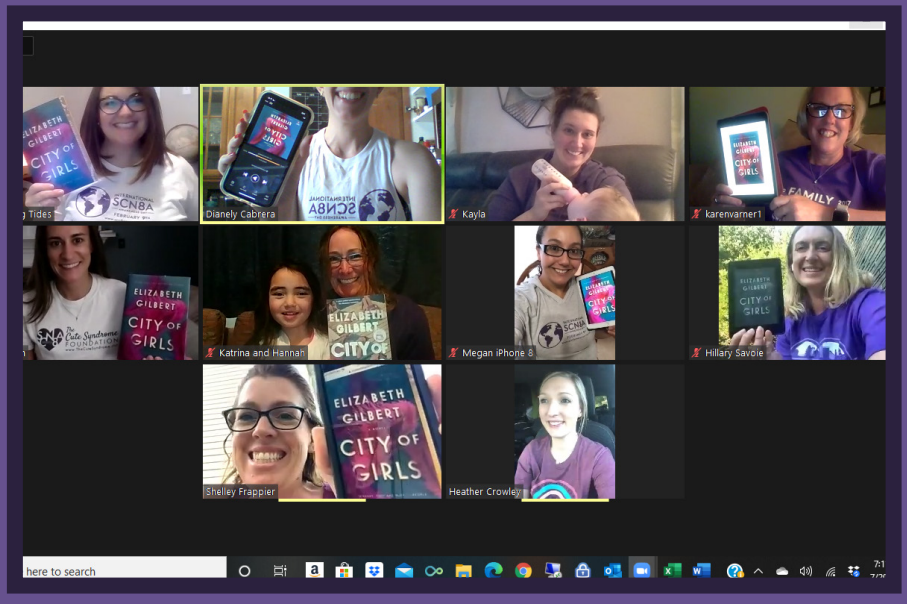
Clinical Trial Readiness Educational Series

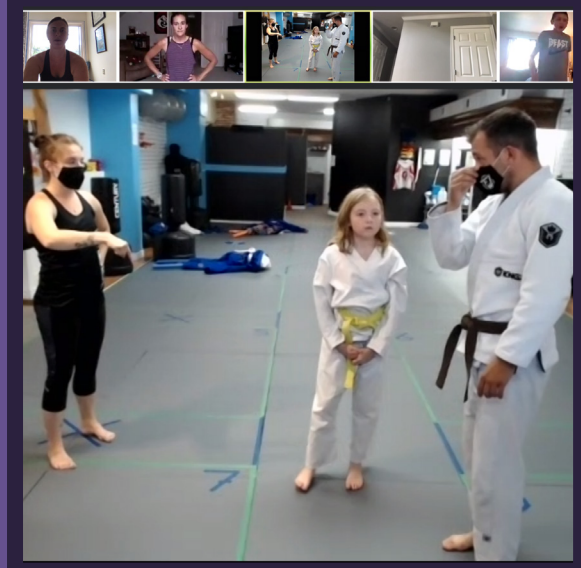
We believe that helping educate our families about drug discovery and clinical trials is essential to having an informed community that can consent to participate in studies. In September of 2020, we launched an educational series to engage the SCN8A families in important discussions surrounding drug discovery and clinical trials.

Using the Hopin virtual platform, we set up sessions with functionality for presentations, moderated conversations, and chat and video Q&A.

Topics included:

Humanizing Language in Pediatric Healthcare
A Conversation About Patient Data
Clinical Trials 101
Setbacks and Failures in Drug Development
Understanding Gene Therapy and How it Pertains to SCN8A





SCN8A ENGAGED

6th Annual Clinician, Researcher, and Family Gathering

TCSF's Annual SCN8A Clinician, Researcher, and Family Gathering has been held in conjunction with the American Epilepsy Society meeting since 2015. Last year we hosted well over 200 clinicians, researchers, family members, patients, and industry professionals during our first virtual event, which focused on the newest advances in drug development, SCN8A research, expert clinical care, and stories of life with SCN8A. We also held family-only virtual meetings, covering topics such as SCN8A 101, SCN8A sibling support, and a Q&A session on drug development. One of the most exciting things about this event was how the families were able to engage in visible conversations with medical professionals during the presentations in the chat function. We watched as they all shared important information back and forth...often among individuals who never would have been able to travel for this event.

41
COUNTRIES REPRESENTED

$20K
COVID-RELATED GRANTS

205
VIRTUAL GATHERING ATTENDEES

Engaging Our Growing Community

TCSF's ability to engage with SCN8A caregivers around the world is growing, as more and more families connect with us through our Facebook Support Group and our newsletter. While the world population of those diagnosed with SCN8A is currently unknown, it is believed to be somewhere on the magnitude of 450-500 individuals. Through our connections with families, we currently are in touch with 332 families in 41 different countries!

In 2020 we were awarded two competitive grants, totaling nearly $20,000 to help us engage our community better—The Rising Tides Grant from the Child Neurology Foundation allowed us to provide social and emotional support programming. It also allowed us to build up a virtual community that comes together to talk, exercise, eat, play...and engage. The NORD COVID-19 Rapid Response Grant supported us in establishing a robust virtual platform for continuing and deepening that engagement.

As a result, when it came time for our 6th Annual Gathering to go virtual, our community was ready to participate. We had 205 attendees—with 88% of registrants attending. 137 of those were SCN8A family members and 86 were professional attendees. The attendees represented 20 countries world-wide. What was most compelling was the level of participation—the chat discussions between families and researchers sharing data. A single hour-long session, held with families and a panel of key opinion leaders, yielded 327 comments. Even better, all of this interaction was captured, enabling us to ensure that all questions were answered and that any pertinent data could be referenced later.

Engagement is wonderful—but what is important to remember is that the purpose of engagement is to move the needle for our children with SCN8A and in 2020, with two clinical trials on the horizon, we did exactly that. In a very meaningful exchange with the FDA, our foundation was able to, overnight, request and then share more than 40 comments, concerns, and feedback from SCN8A families urging FDA to understand the unmet need within our community.

SCN8A RESEARCH

2020 Grant to The Danish Epilepsy Center

The Cute Syndrome Foundation and SCN8A Italia are thrilled to announce that in late 2020 we co-awarded a €9,000 Euro grant to the Danish Epilepsy Center and Primary Investigator Dr. Elena Gardella to support their 430 patient registry and natural disease evolution.

The grant funds the cost of a research fellow, Francesca Furia, to update information on approximately 400 of the 430 patients included in their SCN8A Registry. We are so thrilled to support the important work being undertaken by Dr. Gardella and the team at the Danish Epilepsy Center!

We are also thrilled that the collaborative partnership that we have established between TCSF and our European SCN8A partners is yielding such positive and measurable results already.

TCSF would like to congratulate Cinzia and SCN8A Italia on co-funding their first grant. While TCSF has co-funded nearly $300,000 in research since our inception, every single grant opportunity feels like we are lifting this community a bit higher, together.

We all look forward to many more such collaborations with our partners in the future!

SCN8A ITALIA

AND

SCN8A EPILEPSY



(Left) Cinzia Scarcelli at the 2019 SCN8A Clinician, Researcher, and Family Gathering.

Photo credit: Ryan Collerd



(Above) Hillary Savoie with Katrine Johannesen, Rikke Steensbjerre Møller, and Elena Gardella of The Danish Epilepsy Center during our virtual Pre-Gathering Poster Discussion Series.

SCN8A GRATITUDE

We would like to extend our thanks to:

Volunteers

Angie Day
Denise Juroske Short
Dianely Cabrera
Emma Johnston
Erin Ledrich
Heather Crowley
Jacob Gard
Jaden Bitton
Jen Costello
Jennifer Stout
Katie Banks
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Megan Gard
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Nora Lyons
Ryan Phillips
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Gail Fraizer
Gail M. Meister
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Georgia Varner
Georgina Claydon
Geri & Harry Hopper
Gina Momsen Change
Giorgio Duro
Glenn and Barbara Gronseth
Grace Caufield
Guang Yue
Hank Kratt
Helen Lees
Holly Weix
Hugo Niceplace
Inna Mitelman
Inna Zaydman
Iris Sung
Iris Varner
Jacqueline, Ryan, and Riley Barnett
Jake and Cassady Varner
James Purcell
Jan Kornder
Janice Miller
Jaydip Das Gupta
Jean Cooper-Smith
Jeanna Dunleavy
Jen Mullen
Jennifer Krastel
Jennifer Redekopp
Jennifer Tam
Jessi Winner
Jessie Labbe
Jill Dunmire
John & Jane Clark
John & Leslie Sands
John and Charlene Lareau

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SCN8A GRATITUDE

We would like to extend our thanks to:

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Joyce Browner
Joyce Smith
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Julie Killian
Julie Murphy
Julien Beaulieu
Karen Solms
Karen Varner
Karina Hopiavuori
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Nicole Kornder
Nicole Sweeny
Nina Zanella
Owen & Donald
Padden Family
Patrice Beaulieu
Patricia Hayward

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SCN8A Families at the 2019 SCN8A Clinician, Researcher, and Family Gathering.

Photo credit: Ryan Collerd

SCN8A GRATITUDE

We would like to extend our thanks to:

Patrick Junk
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