

2022 ANNUAL REPORT



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Navigating New Horizons, Achieving Greater Heights Together

In this year's Annual Report, I am both honored and humbled to address you as The Cute Syndrome Foundation's new Executive Director, following in the significant footsteps of our Founder and now President, Hillary Savoie. As we reflect on the achievements of the past year, I am reminded of the remarkable foundation that has been laid, thanks to the leadership and dedication of my predecessor.

Taking on this role is a responsibility I do not take lightly. Hillary has been an instrumental force in steering our organization towards its current success and I am committed to building upon the strong framework that she established and carrying forward the vision that has inspired so many.

The challenges of filling such significant shoes are matched only by the opportunities that lie ahead. As I step into this role, I am reminded that true leadership is not about merely replicating the past, but also infusing fresh perspectives and innovative ideas into our ongoing efforts. I am excited about the collaborations, growth, and positive impact that we have achieved together.

In this Annual Report, you will see the continued dedication of our Executive Leadership Team, volunteers, and partners who have embraced change and remained steadfast in their commitment to TCSF's mission. The achievements highlighted here are a testament to the collective passion and hard work that drive our organization forward.

I am grateful for the support and encouragement I have received during this transitional phase. As we look to the future, I am excited about the possibilities that await us and I am confident that we will navigate new horizons and achieve greater heights together.

Thank you for your trust, and I look forward to our shared journey ahead.

Kacie Craig

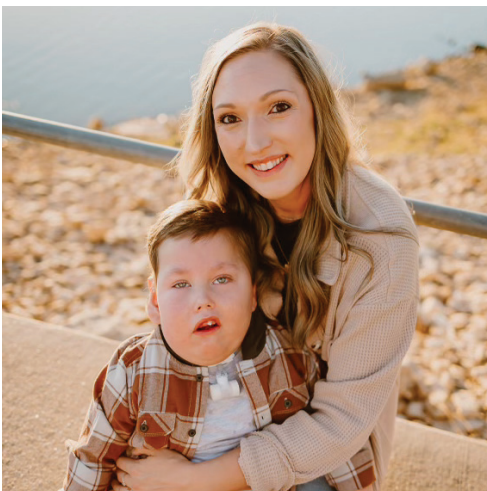
Kacie Craig
Executive Director



Volunteer Highlights

TCSF believes that it's important to shine a spotlight on individuals who are making a tangible difference within our community. In this volunteer highlight, we showcase two dedicated individuals whose passion and commitment to volunteering are creating positive ripples of change. Through their unwavering efforts and meaningful contributions, they embody the true spirit of giving back and serve as an inspiration to us all. Join us in celebrating Heather Crowley and Danielle Hayward and the impact they're making in the lives of others.

Heather Crowley is the mother of SCN8A Warrior, Tate, and officially began her TCSF volunteer journey in 2020. She is heavily involved in our social media team and in the summer of 2022, in conjunction with the organization Missouri Family to Family, she hosted a three-part workshop titled *Cheers To a Good Life: Charting the Lifecourse Framework*. This virtual workshop introduced tools to help our families plan and create their "good life" while enjoying a cocktail or mocktail with their fellow SCN8A caregivers.



“Volunteering for TCSF has been so healing for me. It really gave me the opportunity to get to know our SCN8A peers on a deeper level. I know what it feels like to drown in a diagnosis. If my time can raise money and awareness for these kids or help a family feel less alone or isolated, then it's worth it!”

Heather Crowley

Tate's Mom

Danielle Hayward joined TCSF's volunteer team in 2021 and swiftly made a lasting impression. In 2022, Danielle played pivotal roles across multiple initiatives, serving on the social media team, contributing to the creation of our fundraising calendar, and spearheading our inaugural virtual silent auction.

Her dedication and innovative spirit significantly enhanced our fundraising efforts, paving the way for greater success. Recognizing her exceptional leadership and commitment, she was appointed as the Fundraising Director on TCSF's Executive Committee by the end of 2022.

Behind her remarkable achievements lies a deeply personal motivation: Danielle is the devoted mother of SCN8A warrior Lucy, whose journey fuels her tireless advocacy for our cause.



“Volunteering for TCSF is one of the most fulfilling opportunities I’ve ever had. I love that I can use my education and professional experience to benefit a cause that is so meaningful and personal for me. Being a volunteer has allowed me to build new connections and friendships with other SCN8A caregivers while supporting our amazing SCN8A community. I’m so grateful to be a volunteer!”

Danielle Hayward
Lucy’s Mom



Virtual Silent Auction Fundraiser

SCN8A sibling, Trinity Young, reached out to Executive Director, Kacie Craig, searching for a way she could spread awareness and help fundraise for TCSF. She's watched her sister, Natasha, face challenges and overcome obstacles due to her SCN8A diagnosis and Trinity was ready to jump into action to help TCSF's cause!

Due to geographical limitations, a virtual silent auction made the most sense. Trinity did an amazing job planning the event, getting her local community involved, and worked closely with TCFS's Fundraising Director, Danielle Hayward, to collect donations and set up the virtual auction platform, GiveButter. Many of the items donated were handmade, including the wheelchair-adapted blanket Trinity made herself!

A huge thank you to our shipping sponsor, Swissray, for covering all of the costs to ship the items to the highest bidder and to all our item donors! It was a great success and a wonderful way for a big sister to get involved within the SCN8A community.



Item Donors: Adaptabilities, Flying Squirrel, Hanna B Designs, Jamey Reed Photography, Kendra Scott, Lillie's Artisan Shop, Martin Electric, Morgan's Wonderland, River Dog Bakery, Rusty Wallace Foundation, Zoo New England, Regina Duff, Sari Hope Gertson, Brian and Pam Hart, Kendra Hart, Pat Hayward, Tim Mossberger, Trinity Young

“

So often students like me are obligated to do volunteer work that doesn't mean anything to us, it was really special for me to be able to choose to do this for my sister.”

Trinity Young

Natasha's sister



“

I loved planning our first Virtual Silent Auction alongside SCN8A sibling Trinity Young. We had a tremendous amount of support from within our SCN8A community as well as from outside organizations. We are proud to share that we **raised nearly \$5,000 through our first Virtual Silent Auction** and hope to repeat this success next year!”

Danielle Hayward

Fundraising Director



Pitino Family Comedy Fundraiser

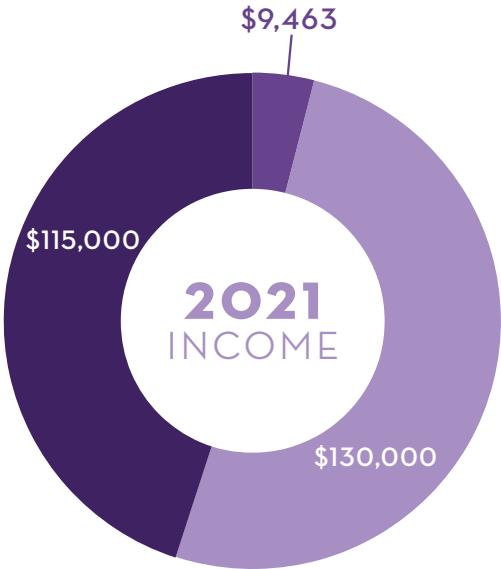
In September 2022, the Pitino Family held a remarkable fundraiser for the Cute Syndrome Foundation. It was a night of comedy held by Laugh.Events at the Cafe Wha? in NYC. The lineup included Jo Firestone, Katherine Branford, and Jared Fried, in addition to the talented Cafe Wha? band!

Between the silent auction, ticket sales, and donations, the event **raised over \$62,000** for TCSF to support our efforts in SCN8A research, clinical excellence, and family support! More specifically, \$10,000 of the money raised was earmarked for TCSF's Dr. Mandy Harris Family Travel Grant which allowed TCSF to fully fund 19 families to attend the 8th Annual Clinical, Researcher, and Family gathering in Nashville, Tennessee.

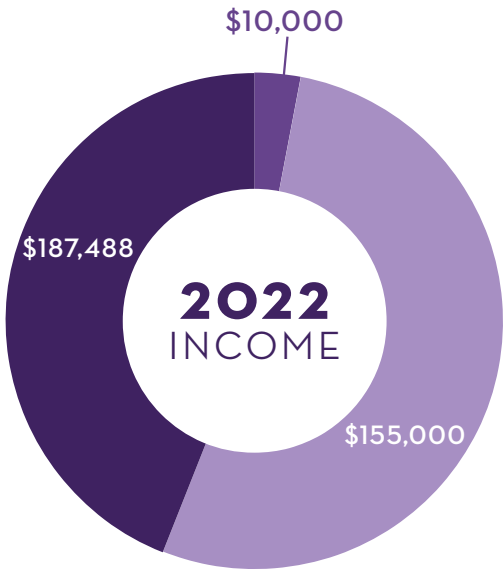
We are so grateful for Laugh.Events and Cafe Wha? for making the event A Comedy Show for Charity possible and to all of those who donated and attended the event, but most of all, please help me in thanking our TCSF Board Treasurer, Ryan Pitino, and his wife, Maura Giordano Pitino, for their generosity in planning this event and filling it with their very generous friends and family!



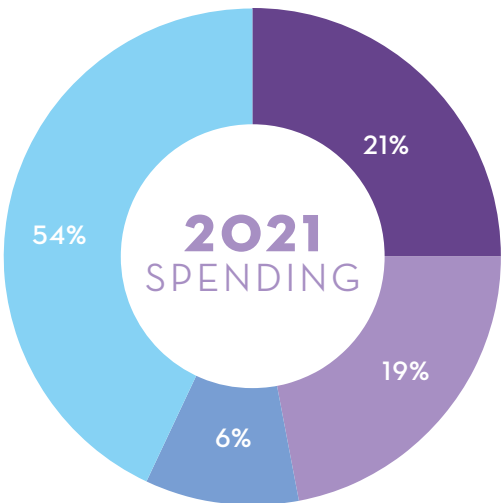
A Look at 2021 and 2022 Finances



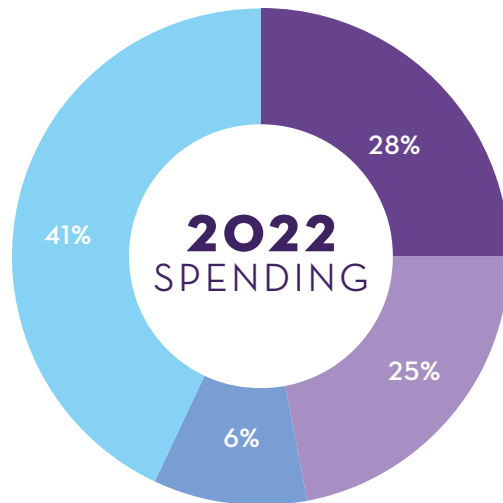
- FOUNDATION SUPPORT
- CORPORATE SPONSORSHIP
- INDIVIDUAL DONATIONS



- FOUNDATION SUPPORT
- CORPORATE SPONSORSHIP
- INDIVIDUAL DONATIONS



- RESEARCH
- AWARENESS AND FAMILY SUPPORT
- OPERATING
- FAMILY ENGAGEMENT AND EDUCATION



- RESEARCH
- AWARENESS AND FAMILY SUPPORT
- OPERATING
- FAMILY ENGAGEMENT AND EDUCATION

2nd annual

Meaningful Change Series

This series looks at the ways in which the knowledge found within the SCN8A community can be leveraged to better support patients and caregivers. This year’s theme was “Looking Forward: Long-term Planning for Your SCN8A Child” as we focused on future planning in the form of vehicle and home modifications, financial planning with Michael Chiodini, and a Life-Course Workshop with planning tools with Mary Turner. We also heard from Praxis Precision Medicines with an update on the Ciitizen campaign and from Neurocrine Biosciences with an update to their clinical trial program.

Nora Lyons shared the following about her experience participating in the series:

“I have a long list of to-dos for my son’s financial future. The tips, ideas, and strategies presented were fantastic. There were things I thought of and some that did not cross my mind. The financial planning session helped me reconfigure my financial to-do list to a more efficient and effective list.”

142

total series views

32

views of “Clinical Trial Endpoints and Updates”

31

views of “Long-term Financial Planning”

29

views of “Ciitizen Update”

20

views of “LifeCourse Workshop”

30

views of “Vehicle and Home Modifications”



Long-term Financial Planning

Michael Chiodini, Chiodini Financial
Kacie Craig, The Cute Syndrome Foundation

The Cute Syndrome Foundation's
Meaningful Change Virtual Series



LifeCourse Workshop

Mary Turner, Missouri Family to Family
Heather Crowley, Missouri Family to Family and SCN8A Mom
Kacie Craig, The Cute Syndrome Foundation

The Cute Syndrome Foundation's
Meaningful Change Virtual Series



Clinical Trial Endpoints and Updates with Neurocrine Biosciences

Carolyn McMicken, Neurocrine Biosciences
Heather Bentley, Neurocrine Biosciences
Tyler Reagan, Neurocrine Biosciences
Kacie Craig, The Cute Syndrome Foundation
Hillary Savoie, The Cute Syndrome Foundation

The Cute Syndrome Foundation's
Meaningful Change Virtual Series



Ciitizen Update with Praxis Precision Medicines

Kelley Dalby, Praxis Precision Medicines
Christian Rubio, Praxis Precision Medicines
Kacie Craig, The Cute Syndrome Foundation
Hillary Savoie, The Cute Syndrome Foundation

The Cute Syndrome Foundation's
Meaningful Change Virtual Series



Vehicle and Home Modifications

with SCN8A parents Dianely Cabrera, Kacie Craig, Karen Varner,
Krista Smith, Nora Lyons, Sandy Bush, and Shelley Frappier

The Cute Syndrome Foundation's
Meaningful Change Virtual Series

Cheers to a Good Life

This summer, TCSF volunteer Heather Crowley partnered with Mary Turner of Missouri Family to Family to present a three-part series consisting of one-hour workshops that introduced the Charting the LifeCourse framework and tools to help our families plan and create their very own “Good Life.”

Heather and Mary taught us how to create a vision for our family member’s “good life” by using the Integrated Support Star, Trajectory tool, and the Mapping Relationships tool. Each tool provided us space to explore the things going well in our lives or the things that are stopping us from reaching our good life and helped us build a circle of support for our child beyond only primary caregivers.

Our motto during this workshop was, “Stop merely surviving, begin your Good Life today.”

Charting the LifeCourse Integrated Supports Star

The diagram illustrates the Integrated Support Star, a five-pointed star with a central circle. The star is divided into five colored segments: red (top-left), teal (top-right), purple (right), green (bottom-right), and blue (bottom-left). The central circle is light blue. The star is set against a white background with a light blue border.

PERSONAL STRENGTHS & ASSETS
Skills, personal abilities, knowledge or life experiences;
Strengths, things a person is good at or others like and admire;
Assets, personal belongings and resources

TECHNOLOGY
Personal technology anyone uses;
Assistive or adaptive technology with day to day tasks;
Environmental technology designed to help with or adapt surroundings

RELATIONSHIPS
Family and others that love and care about each other;
Friends that spend time together or have things in common;
Acquaintances that come into frequent contact but don't know well

COMMUNITY BASED
Places such as businesses, parks, schools, faith-based communities, health care facilities;
Groups or membership organizations;
Local services or public resources everyone uses

ELIGIBILITY SPECIFIC
Needs based services based on age, geography, income level, or employment status;
Government paid services based on disability or diagnosis, such as special education or Medicaid

COMMUNITY
FAMILY
PERSON

Charting the LifeCourse www.lifecoursetools.com

3rd annual

Clinical Trial Readiness Series

Our 3rd annual Clinical Trial Readiness Series expanded on topics from the previous year. We began with a live community conversation with Praxis Precision Medicines to discuss the molecule PRAX-562 and its potential for SCN8A. We then welcomed back Neurocrine Biosciences to discuss

NBI-921352 and Kayak Study updates, with a look back at the last 6 months and what's to come in the next 6 months of their trial. We concluded with updates from TCSF executive committee members about the Global Genes Summit conference, Neurocrine Biosciences lab tour, and foundation aspirations for the future.

242

total series views

128

views of "From Data to Development: A Deeper Look at PRAX-562 for SCN8A-DEE"

92

views of "Research Updates and Moving Beyond Clinical Trials 101"

22

views of "The Cute Syndrome Foundation Updates and Drivers"



From Data to Development: A Deeper Look at PRAX-562 and Your Role in its Progress

Kelley Dalby, Praxis
Raji Mahalingam, Praxis
Christian Rubio, Praxis
Karen Utley, Praxis
Kacie Craig, The Cute Syndrome Foundation



Research Updates and Moving Beyond Clinical Trials 101

Heather Bentley, Neurocrine
Carloyn McMicken, Neurocrine
Tyler Reagan, Neurocrine
Svetlana Shore, Neurocrine
Kacie Craig, The Cute Syndrome Foundation



The Cute Syndrome Foundation: Updates and Drivers

Kacie Craig, The Cute Syndrome Foundation
Shelley Frappier, The Cute Syndrome Foundation
Karen Varner, The Cute Syndrome Foundation

CUTE Connections

Although providing year-round virtual activities is imperative to our families because we are far too often isolated, even in the best of times, TCSF recognized an urgent need for families to engage in face-to-face activities. The pandemic has left our families suffering from extreme social isolation and loneliness and requesting in-person fellowship activities, in addition to virtual events.

The goal of Cute Connections family activities is to first and foremost give our community a worry-free opportunity to give and receive actual hugs, spend time catching up, and offer each other moral support. We want to provide a safe and accessible environment for our kids to do what they do best, with minimal stressors for parents.

In July of 2022, several TCSF families braved the summer heat and met in San Antonio, Texas to attend the handicap-accessible theme park, Morgan's Wonderland, and waterpark, Morgan's Inspiration Island.

Everyone had a blast enjoying each other's company and seeing ALL of our cuties participating throughout the theme park and waterpark! TCSF's goal is to organize at least one face-to-face family activity each year and cover as many of the costs of that activity as possible for families.

3,904
Total Miles
Traveled



The Cute Syndrome Foundation's Family is Growing

TCSF's Family Support Group, established with the mission *to provide assistance, guidance, and community for families navigating the challenges of SCN8A-related conditions*, witnessed remarkable growth by the end of 2022. With over 400 members spanning across more than 40 countries and reaching families in 40 states

throughout the United States, the group has become a global hub for support and solidarity. Through various online platforms, meet-ups, and resource-sharing initiatives, the group continues to foster a sense of belonging and empowerment for individuals and families affected by SCN8A-related conditions worldwide.

MIA | USA
Mia is globally delayed, goes to a lot of doctor appointments and therapies, has multiple diagnoses, and has tried multiple medicines to get her to where she is today. She has a long way to go, but she is strong and sassy! SCN8A is not always so cute, but Mia sure is.

THOMAS | ENGLAND
At five years old they found a genetic cause for my epilepsy and developmental delays...SCN8A, but it didn't hold me back. There are bad days, but lots of good days.

AMELIA | USA
#OurCuteStory is about a girl who lived—defying the odds with every hospital admission. Amelia has been fighting her whole life. From status seizures, to being intubated several times, to being put on the oscillator as a last option, to “we just don't know if she'll make it”, to smiling and laughing...it's a full-circle moment each time.

BILLY | USA
We didn't receive our SCN8A diagnosis until 13 years later—when our insurance finally approved the testing—which led us to The Cute Syndrome. We have an amazing neuro now, Billy is med free, and is happy.

SCN8A THE CUTE SYNDROME FOUNDATION
www.TheCuteSyndrome.com

SCN8A EPILEPSY
INTERNATIONAL AWARENESS DAY
FEBRUARY 9TH
www.scn8aawarenessday.net

OUR CUTE STORY
2022

OVER
400
MEMBERS

REPRESENTING
40
COUNTRIES

WITH MEMBERS IN
40
US STATES

Advocacy and Awareness

Throughout the year 2022, TCSF actively engaged in advocacy and awareness efforts through participation in conferences, speaking engagements, and awareness events. These initiatives provided valuable opportunities for networking and collaboration with other advocacy groups, professionals, and families affected by SCN8A.

"I really enjoy interacting with the families and sharing knowledge about SCN8A, as well as networking with leaders from other advocacy groups."

Karen Varner
Family Support Director





8th Annual

SCN8A Clinician, Researcher, and Family Gathering

In 2019, we all left Baltimore, Maryland thinking we'd hug each other once again in 2020, but we all know what happened next...COVID-19 destroyed all plans for an in-person event in both 2020 and 2021. Although both events, virtual and hybrid, were a huge success, nothing can replace the magic of being in the same room with other families and the clinicians, researchers, and industry partners fighting for our community.

So, after three long years, TCSF's community traveled distances near and far to attend the 8th Annual SCN8A Clinician, Researcher, and Family Gathering as an in-person event in Nashville, Tennessee! There was a very large presence of families this year and TCSF was thrilled to cover all travel and lodging costs for all 19 families that applied for the Dr. Mandy Harris Family Travel Grant program!

Recognizing the challenges SCN8A families face, especially when traveling, TCSF offered a live stream and recording of the Friday festivities. Almost 35 individuals participated in the live stream, and the recording has garnered over 285 views.

Over 100 SCN8A families and patients, researchers, clinicians, and industry partners gathered in-person to hear clinical trial updates from our industry partners, Praxis Precision Medicines and Neurocrine Biosciences. Once again, Dr. John Schreiber hosted the Clinician Roundtable and Dr. Manoj Patel hosted the Researcher Roundtable. Each roundtable included four renowned presenters in their respective areas of expertise.

**SCAN TO VIEW MORE PHOTOS
FROM THE EVENT!**





This nearly five-hour event was a true integration of our community as families asked researchers and clinicians questions after their presentations and researchers witnessed seizures and heard medical device alarms going off.

During the second day of our Annual Gathering, we had the opportunity to watch the “Unseen” documentary and have a group discussion. This documentary gives a glimpse into the lives of caregivers and the challenges they face. The goal of the documentary is to inform caregivers about available resources and educate organizations, providers, etc about how they can help support these caregivers.



Dance Party!

This year we held our first ever SCN8A Dance Party! While our annual Gathering offers a lot of great information and learning opportunities in one place, one of the greatest benefits is the face-to-face interactions and connections with other SCN8A family members. We held a dance party this year (complete with a line-dancing instructor!) as a way for our families to let loose and connect with each other in a less structured setting.



Rare Epilepsy Partnership Award



Jillian McKee, MD, PhD

Children's Hospital of Philadelphia

TCSF and CURE Epilepsy were thrilled to announce the granting of a Rare Epilepsy Partnership Award to Jillian McKee, MD, PhD at the Children's Hospital of Philadelphia for her project titled "Reconstructing the longitudinal disease history in SCN8A-related disorders".

Dr. McKee's project aims to understand the natural history and genetics of SCN8A-related disorders. Her team will use data from electronic medical records (EMR) to understand the full range of clinical features over time. Through analysis of this data, the team proposes to identify previously unknown clinical subgroups, disease courses, and medication responses to improve clinical care, medication choice, and aid in the design of clinical trials and targeted therapies.

The one-year grant totaling \$100,000 is co-funded in equal part by TCSF and CURE Epilepsy, whose portion is sponsored by the Robert Withrow Wier Fund.

2022 CUTIES Award Presented to Hillary Savoie

After the first SCN8A Clinician, Researcher, and Family Gathering, held in 2015, TCSF saw a handful of individuals rising to the opportunity to work collaboratively to improve the health and safety of those living with SCN8A. In recognition of that work, we created the CUTIES, our annual awards for Champions for Understanding, Treating, Investigating, and Empowering those with SCN8A.

Typically, we present three separate awards going to a researcher, clinician, and family member that have gone above and beyond for our community. But this year, we chose only one person, Hillary Savoie.

Hillary has worked tirelessly for this community since the very beginning. She is not a clinician or researcher, but she'd be

worthy of an honorary degree in both. She is a mother who, in the midst of her darkest days, rose above to fight for her child and then took on fighting for all of our kids.

She made herself and the SCN8A community be seen within the epilepsy space. Even when our community was fewer than 100 people, she stood tall and made people listen. She saw that our children deserve better and she was committed to finding people to help her fight. And she did it. She found clinicians, researchers, and multiple industry partners who now fight along our side.

Congratulations, Hillary Savoie! You are more than deserving of this award and TCSF is lucky to have you as our Founder and President!



A Heartfelt Thank You to Our Generous 2022 Donors

| | | | |
|-----------------------------|----------------------------|-----------------------------------|------------------------|
| Aaron Williams | Denise Dance | Kris Kahlig | Richard Pitino |
| Alex Youngquist | Devon and Tom Hosford | Leigh Harrington | Rick Avare |
| Allison and Troy Donohue | Edward Detmer | Lisa Gidaro | Rick Pitino |
| Alonzo Guess | Elizabeth Monaghan | Lisa Jones | Rickie Le |
| Ashish Sgrolarik | Erin Jones | Loraine Hughes | Rob Minardi |
| Ashley Flynn | Erin Mara | Lori Hayward | Ryan Pitino |
| Barbara Hallberg | Fazlin Ahlgren | Lori Wilson | Samantha Oberkramer |
| Bonnie Johnson | Francine Dion | Lydia Toso | Sayde Dunlap |
| Brad Gianiny | Frederick Adair | Maite Schenck | Scott Maddox |
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| Brian Bachman | Hannah Wingo | Marie Bundhoo | Stacey Gagnier |
| Brian Lasky | Heather Grant | Marie Duncan | Stefan Hrdina |
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| David Bauer | Kevin Hubschmann | Pricilla Merryman | Valintina Munoz |
| DeAnna Daniels- Fischer | Kimberly Overton | Richard and Barbara Leckerling | Victoria Hight |
| | | Richard Bosman | William Minardi |



Thank you, **Volunteers**

The Cute Syndrome Foundation's work depends on all of our volunteers. Thanks to each of you, we are able to advance our mission of raising awareness of SCN8A mutations, funding the dedicated and talented scientists researching SCN8A, and supporting the families around the world who are affected by this disorder.

Executive Committee

KACIE CRAIG

Executive Director

KAREN VARNER

Family Support Director

LIZ RAMIREZ

Bereavement Support
and Grants Advisor

MEGAN VARNER

Marketing Director

SHELLEY FRAPPIER

Director of Patient Engagement
and Data Management

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