Dear Friends,

October was a month of devastating loss for our community. Five young warriors taken too soon. How do we move on to 'Thankful November' with such heavy hearts? First, by adding their names to our growing list of warriors to remember. And then by channeling that loss into a fierce resolve to fight harder and faster for life-saving cures. FamilieSCN2A is incredibly grateful for unwavering support from our warrior families and friends, scientists and researchers, clinicians and caregivers. Every day we are closer to having cures, but until then, every day finds another SCN2A Warrior in crisis. Throughout the month of November, we'll be sharing gratitude in many ways and invite you to do the same on our social media sites. Look for an exciting announcement on ***Giving Tuesday, 11/28,*** *to ring out 2023 with a bang! - Leah Myers, Founder & Executive Director*

Record-breaking SCN2A Warrior Challenge!

We are so THANKFUL for the 38 teams who raised $70,000!!

WARRIOR CHALLENGE VIDEO

WHAT'S HAPPENING?

Current SCN2A-related Clinical Trials

Updates on Praxis Precision Medicine's Clinical Trials

FamilieSCN2A Medical & Scientific Advisory Board member, Dr. Heather Olson, answered questions about the clinical trial for PRAX 562. The Town Hall was recorded and will be posted on the DEE-P webinar website soon. To learn more about the Praxis 562 Embold Study, click here.

Praxis recently reported positive early findings from the Prax 222 Embrave Study. Click here to learn more - starting at Slide #11.

FamilieSCN2A's CSO, Shawn Egan, hosted a Town Hall with Praxis CEO, Marcio Souza on Oct.15th. Watch it here!

LONGBOARD PHARMACEUTICALS

Longboard Pharmaceuticals' Pacific Study (for DEEs, including SCN2A) is finished! Phase 2/3 results expected in Q1 2024. **Read Longboard's recent update here.**

SCN2A FAMILY TOWN HALL DATES

LED BY SHAWN EGAN, PHD

Register once in advance for all meetings:

Nov 15, 2023 06:00 PM

Dec 15, 2023 09:00 PM

Times are Eastern Standard. After registering, you will receive a confirmation email with the link to join. These sessions are for **families only** and are not recorded.

Opportunities to raise awareness are everywhere! Lead the way by changing your social media profile frame for Thankful November or Epilepsy Awareness Month

November is Epilepsy Awareness Month! Put on some purple to raise awareness and tag #CureSCN2A in a post. The FamilieSCN2A Foundation works closely with many epilepsy organizations. We are members of the American Epilepsy Society's Epilepsy Leadership Council and one of our board members serves on the ELC Steering Committee. **Tell your neurologist to stop by our Booth # N641 at the AES Annual Meeting in December. If you live near Orlando and want to meet up, let us know!**

Ben's Story = over 1 MILLION views across social media!

People are moved to action by impactful stories they see and hear. Every family reading this has a story worth telling about the real-life impact of SCN2A-related disorders. We invite you to share *your* story during Thankful November and move the world to act faster to cure SRDs! Let us know how we can help you tell your story, your way, to raise awareness and move us towards a world where every SRD story has a happy ending! To get started, click here. Stories already shared are on our website and social media. Check out this helpful storytelling toolkit from Global Genes.

The **2024 SCN2A Warrior Calendar** is now available! A year-long celebration of over 250 SCN2A warriors. Order your calendar today plus a few extra for gift-giving! Now is the time to stock up on SCN2A gear for the holidays. Doesn't everyone need a new hat or mug? Browse the online store today.

SCN2A CTRS
CLINICAL TRIAL READINESS STUDY

One of The FamilieSCN2A Foundation's core values is collaboration. We believe that the best path forward for curing SRDs is to broadly share information so that researchers and industry may gain access to it. We are excited to announce an agreement to share CTRS data with The Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®), an FDA-funded initiative that provides a centralized and standardized infrastructure to support and accelerate rare disease characterization, with the goal of accelerating therapy development across rare diseases.

Giving Tuesday is a global movement unleashing the power of radical generosity and encouraging people to do good things. On the Tuesday after Thanksgiving, Black Friday, and Small Business Saturday in the US, Giving Tuesday celebrates every act of generosity. **What will you do on November 28?** Check in on an SCN2A friend? Volunteer your time and talents? Share your SCN2A story? Make a donation? Then share it on social media #CureSCN2A!

IN RECENT NEWS

FAMILIESCN2A
SCN2A-RELATED AUTISM & EPILEPSY

Hodgkin-Huxley Research Grant

DID YOU SEE THE PRESS RELEASE?!

Congratulations to **Rikke Steensbjerre Møller** of *Danish Epilepsy Centre, Filadelfia/University of Southern Denmark* recipient of the first international FamilieSCN2A Foundation's Hodgkin-Huxley Research award in the amount of $38,243 for her project, "Bridging the gap between ongoing knowledge and clinical practice – establishment of an international SCN2A database."

The aim of this collaborative project by the research teams of **Rikke Møller** and **Dennis Lal** (University of Texas-Houston) is to establish and maintain an international SCN2A database that can be used to study genotype-phenotype relationships in SRDs. The goals are to 1) Establish and maintain a database including clinical, genetic and epidemiological data of all published individuals with SCN2A-related disorders; 2) Assess all reported variants and classify according to the AC MGG guidelines; and 3) integrate all data in the existing SCN portal (scn-portal.broadinstitute.org)–an interactive website designed to provide updated and comprehensive information on SCN2A-related disorders.

SCN2A Publications:

Electroencephalographic insights into variant function and clinical outcomes in SCN2A encephalopathy | medRxiv. A collaborative project to study EEGs as potential biomarkers for clinical care and trials.

Five reflections from the FamilieSCN2A Annual Family and Professional Conference | Beyond the Ion Channel by Stacey Cohen, MS, LCGC, Children's Hospital of Philadelphia

MONTHLY RECAP

We are so grateful to the Egan family for hosting their 3rd Annual Golf FORE SCN2A Tournament in Buffalo in August. A fun event for the whole family that raised $44,000!

Thinking of hosting a golf fundraiser yourself? We can help!

Chan Zuckerberg Initiative "Advancing Patient-Driven Research in Rare Diseases: Science in Society"

In September, Jenny Burke, Leah Myers, Shawn Egan, PhD, and Brad Bryan, PhD, were invited to the 4-day CZI convening which brought together multiple experts and innovators who have a shared goal to expedite treatments and cures for rare diseases like SCN2A-related disorders.

Do you know the difference between a buffalo and a cow in a rainstorm? (Michael Hund, 'Kansas guy thing to say')

Cattle do what they've always done, they slowly move in the other direction to avoid the storm. Buffalo run straight into the storm so they can get to the other side faster despite the challenges and obstacles. This particular convening hosted 400 buffalos! It was a fantastic learning and networking event.

As part of the CZI Rare As One Network (RAO), patient-led organizations are developing and launching collaborative research networks in partnership with clinicians and scientists. Patients and caregivers, being experts in their specific disease areas, possess invaluable knowledge that has the potential to significantly hasten the pace of research. The RAO project provides funding, tools, capacity-building support and training to make that happen.

The FamilieSCN2A Foundation is entering it's 3rd and final year as an RAO grantee.

In mid October, Shawn Egan, PhD and Leah Myers participated in the The NORD Rare Diseases and Orphan Products Breakthrough Summit where they joined thought leaders in discussing solutions to the most pressing issues facing the rare disease community, such as: The Sustainability of Rare Disease Drug Development, Gene-Editing, Artificial Intelligence (AI) and Digital Health, and Moving DEI from Good Intentions into Effective Action. The Summit offered ample networking opportunities for our team to foster meaningful connections with influential stakeholders across the rare disease community.

We are thankful for Board Trustee, Mery Oman, who joined Anita & Jayline to represent FamilieSCN2A at the 11th Annual Epilepsy Awareness and Education Expo, the largest gathering of

Epilepsy Physicians and Patients on earth! This late October event leads up to Epilepsy Awareness Month and Epilepsy Awareness Day at Disneyland.

In case you missed it, the 2023 SCN2A Family and Professional Conference videos from July's meeting in Boston are on the website!

FamilieSCN2A Crisis and Loss Support *has a heartfelt mission to carve out a special space within our already wonderfully inclusive community – a space dedicated to remembrance. We*

understand that grief takes on many forms, and it doesn't adhere to a one-size-fits-all approach. With this understanding, we've worked diligently to ensure that every community member, regardless of their preferred mode of communication or level of engagement, feels supported and deeply connected. We are working to expand our outreach methods, creating spaces that accommodate various needs. Whether it's through dedicated online platforms, virtual support groups, or in-person events, we've made it our mission to ensure that no one within our community ever feels isolated during their grief. We want every family to know, unequivocally, that they have a place to turn for support, understanding, and remembrance. Together, we are building a more compassionate and inclusive community, where every voice is heard, and every warrior, whether they are with us or watching over us, is cherished and remembered.

Experienced with loss, Tracy Umezu and Angie Weaver lead the Crisis and Loss Support Committee. If you have a heart for families experiencing crisis or loss, but have not experienced the loss of a child, please consider serving alongside them. Contact info@scn2a.org.

WHY WAIT? DONATE TODAY!