SCN2A RELATED AUTISM & EPILEPSY

FAMILIESCN2A FOUNDATION

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Dear Friend,

We are writing to tell you about the amazing progress the FamilieSCN2A Foundation has made towards our mission to improve the lives of those living with SCN2A related disorders.

This year we are celebrating our 6th anniversary of becoming a nonprofit! SIX years of helping families and funding life-saving research.

Changes in the SCN2A gene are recognized as the 3rd most common cause of Autism and 5th most common cause of early onset Epilepsy. Understanding the cause of these devastating disorders has inspired researchers to find new treatments.

Over the last 6 years, we have funded more than $750,000 in research to find a cure for SCN2A disorders.

We firmly believe that through our continued dedicated research funding we will not only find a cure for SCN2A disorders, but also contribute to the larger Autism and Epilepsy communities.

This year we are at a turning point. Our research initiatives have led to the launch of the first ever SCN2A Clinical Trial Readiness Study, which will provide meaningful endpoints for our industry partners to target when designing clinical trials.

Clinical trials are coming soon!

We are proud to give an important update on the work we are supporting at Nationwide Children's Hospital in Ohio. The Foundation is sponsoring a safety study in juvenile, non-rodent, animals for a small molecule drug which is currently in clinical trials for other neurodegenerative disorders and has years of positive safety data in adults. Efficacy has been shown in SCN2A patient cell lines and mice and if the safety data is positive, we hope to rapidly advance towards clinical trials in SCN2A, maybe even this year!

In addition, there is another molecule in Phase I trials (healthy humans) and while advancements in gene therapy are happening at lightning speed in many diseases, they are still a few years away for SCN2A. Our children are very sick and the urgency is even more evident when we lose one of our warriors. This is why the Foundation supports research for treatments such as drug repurposing, new compounds and small molecules as well as gene altering therapeutics.

To help pave the way for new treatments to be approved, we have arranged an SCN2A Listening Session with the US FDA where families will share their experiences living with SCN2A disorders. The **patient voice** has become an integral part of the approval process!

In 2020, we initiated our Patient Assistance Grant program, which sets aside funds each year to help SCN2A patients with necessary equipment and therapy associated with the condition that are not covered through other means. In addition, we helped meet the needs of many families devastated by loss of income due to the pandemic by offering Covid-19 Emergency Grants. We are proud to say we were able to fund nearly every grant application and, with your help, plan to continue these critical programs.

"The FamilieSCN2A Foundation has changed my life in such a profound way. They have been helpful in answering questions, setting up conferences with relevant information and providers from all over, sharing experiences, providing support and mostly becoming part of our family. In a world where I felt alone with my son's diagnosis, they helped change that and made me feel like there is always someone on my side. Never any judgement passed or made to feel any question was not valid to ask. I am so grateful for this group and what they have to offer." —Jane D., SCN2A warrior parent

While there has been great progress to date, it is critical that we continue to grow our dedicated research pipeline and offer support to patients and their families who desperately need these lifesaving opportunities.

In honor of our 6th anniversary, please consider making a thoughtful donation in support of our foundation as we continue to help children and families with SCN2A disorders fight through this disease.

Any size donation truly does make an impact. IF you are able to donate $5, $50, $500 or $5,000 today, you are moving our mission forward through research and directly helping families who struggle daily through this journey. Please click on the link to give https://www.scn2a.org/donate.html.

Gratefully,

Carla Forbes
President, Co-Founder
FamilieSCN2A Foundation

Leah Schust Myers
Executive Director, Co-Founder & Research Chair
FamilieSCN2A Foundation

P.S. This year we are honoring the VOICES of those affected by SCN2A, most of whom cannot speak for themselves. Please, voice *your* support with a donation.