

2021 VISION MISSION VOICE



May 2021 Newsletter: See what we've been up to!

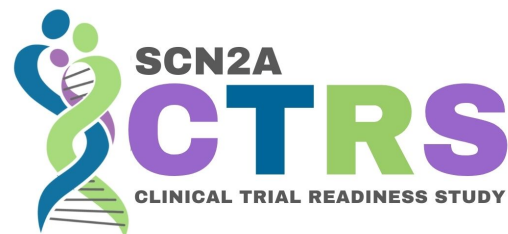
FAMILIES: Where are you in the CTRS process?

**The SCN2A CLINICAL TRIAL
READINESS STUDY
(CTRS) has launched!**

Haven't registered yet? It's not too late!

Visit www.scn2a.study

Once you've done your interview, log
back in to SCN2ACTRS.study to
complete your **BASELINE**
measurements and earn your first \$50
gift card!



Make your voice heard in advancing research!

[More Info](#) [Questions?](#)

Why participate? The best way to determine if a new treatment will work is by having appropriate measurements to demonstrate effectiveness. This study will identify those measurements, enabling the best possible outcomes for clinical trials. We need YOUR input, your VOICE. As the parents of those with SCN2A related disorders you are an integral part of the research process.

What if I'm not eligible? There will be more studies and future trials. If you aren't eligible for the CTRS there is a one-time Global Survey you can do now that will connect you to future projects. The good news is that the results of the CTRS will benefit all SCN2A patients.

SCN2A RELATED AUTISM & EPILEPSY
FAMILIES SCN2A FOUNDATION
WWW.SCN2A.ORG

ANNUAL CAMPAIGN
VOICES
FOR
SCN2A
#CURESCN2A

SCN2A ANNUAL CAMPAIGN
WWW.SCN2A.ORG/DONATE

MAY 1, 2021 - JULY 31, 2021
Research ● Impact ● Cure ●

CAMPAIGN GOAL: \$60,000

This year we started the [Annual Campaign](#) in May. In the next 10 weeks our goal is to raise \$60,000 toward life-changing research and support for families affected by SCN2A disorders. Funding for rare diseases is hard fought and we raise much of ours in small increments through grassroots donors. We can provide resources ([we've already written a letter to share!](#)) and assistance ([contact us](#)) as you reach out to your network. Our friends and family often want to help but aren't sure how. This is one easy way! Check out our new [Social Media Toolkit](#) that simplifies fundraising by helping you share your story through social media and email (or even snail mail).



10 REASONS TO GIVE

#1 Meet Brooks

Brooks is almost 2 and is still waiting on a cure! Last year, his family gave back in a big way, hosting the top fundraiser during the Annual Campaign by sharing their SCN2A journey. [Visit his page](#) on our website and check out the [toolkit](#) for information on how to share your story.



Coming this summer...


Rx: Hope

**Virtual
Table Top
Talks**



The SCN2A Family & Professional Conference is going to look a little different this year, however, it will be just as impactful! On a bi-weekly basis, throughout the summer, we will be hosting 1.5 hour Zoom sessions where you, the SCN2A Experts, can meet with SCN2A professionals to learn about their work, ask your questions directly and answer theirs!

Topics will include:

SCN2A LoF: Why some present with seizures and others do not

SCN2A gene and RNA therapy

Progress in drug discovery

What we are learning from animal models

Updates on functional analysis of the SCN2A variants

...and SO much more!

Another opportunity to make your VOICE heard!



On April 29, the FamilieSCN2A Foundation made our voices heard in a 90-minute, virtual **Patient Listening Session** with the US Food and Drug Administration. Six families and three of our long-time research partners presented the full picture of what SCN2A is, what we are doing to accelerate research and what it's really like to live every day with a broad range of SCN2A related disorders. **COMING SOON:** A formal report from the meeting will be available on our website and at FDA.gov.

PROFESSIONALS: Please share this opportunity!

ACTION POTENTIAL RESEARCH GRANT

REQUEST FOR APPLICATIONS
OPEN MAY 1, 2021



TAKE ACTION. CREATE POTENTIAL.

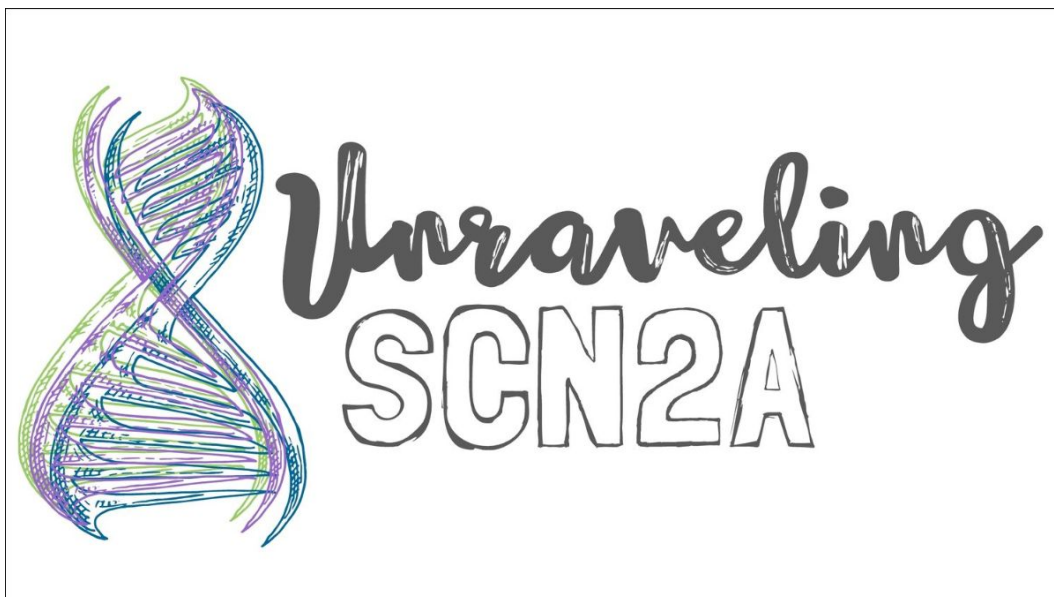
DEADLINE FOR LOI IS JUNE 1. FULL APPLICATION DUE JULY 1. [DETAILS](#)

RESEARCH



We are proud to give an important update on the work we support with Dr. Kathrin Meyer and Dr. Nicolas Wein at Nationwide Children's Hospital in Columbus, Ohio. The Foundation is sponsoring a safety study in juvenile, non-rodent, animals for the molecule CuATSM. CuATSM is currently in clinical trials for other neurodegenerative disorders with years of positive safety data in adults. This molecule has shown efficacy in in-vitro experiments using SCN2A patient cell lines as well as a severe gain-of-function SCN2A mouse model. Should the data from the juvenile safety study confirm this, we hope to rapidly advance the program towards clinical trials in SCN2A, maybe even this year!

[Read our latest blog post for research updates from Praxis Precision Medicines.](#)



give the gift of hope today



[View this email in your browser](#)



Copyright © 2021 The FamilieSCN2A Foundation, All rights reserved.

Want to change how you receive these emails?
You can update your preferences or unsubscribe from this list.