SCN2A
CTRS
CLINICAL TRIAL READINESS STUDY

Dear SCN2A Parent,

Over a year ago, we set a goal for our community of performing the SCN2A Clinical Trial Readiness Study (CTRS) to make sure we had the right outcome measures to use in the up-and-coming clinical trials of novel therapies for our children. Thanks to you and so many other parents, that goal is being met. Click here to better understand how critically important this work is to our community.

We still need your help though. If you haven't had time to finish your baseline forms, please take a moment to do so now. You can login to your portal and complete them all at once or over a few days. But please complete them before the end of this month.

We are thrilled to see so many of you continuing with the 2-week, 3-month, and now even 6-month interviews. This is fantastic and provides the critical evidence needed for the FDA to approve Clinical Trial designs, particularly the outcome measures. Again, we need your help. Anything we at the FamilieSCN2A Foundation can do to support you and anything that the research team at Lurie Children's can do to facilitate you in this mission is yours for the asking. Always feel free to contact Leah Schust Myers or the study team if you need anything. We are here to help you!

Early results are already being shared with our community. You can view and download the initial summary data. Two webinars by the lead investigator that explain why we are doing the study and what we are learning can also be viewed here and here.

We all know how complicated the life of an SCN2A family can be, and we know it can be hard to find the time. But we can guarantee that this is worth it and will help our community move forward toward finding successful treatments for our loved ones with SCN2A-associated neurodevelopmental disorders.

Can we count on your continued support to push this project across the finish line?

Thank you for all you do.

Carla Forbes

President, FamilieSCN2A Foundation

To learn more about the study and to begin your registration and consent process, please go to the following link: https://scn2a.study/

If you have questions about the study, please email WhatIsEpilepsy@luriechildrens.org

12 Month Study Eligibility (Full CTRS)

- verified pathogenic or likely pathogenic SCN2A variant
- between the ages of 1-25
- willingness to participate over the next 12 months
- any **country** with an English proficient speaking parent