

FamilieSCN2A Foundation Partners with NORD® to Initiate Registry for SCN2A-related disorders

Global research study plans to accelerate treatments for SCN2A-related disorders, a rare disease associated with epilepsy, intellectual disability, and autism.

GETTYSBURG, PA, UNITED STATES, March 7, 2024 /EINPresswire.com/ -- The FamilieSCN2A Foundation has partnered with the National Organization for Rare Disorders (NORD®) to launch the DRAGONFLY Study to collect natural history data from families affected by [SCN2A](#)-related disorders.

The DRAGONFLY Study is a patient-centered registry designed by researchers, medical experts, and caregivers. The data, which is entered by patients and/or their caregivers, will help pave the way for future research in SCN2A-related disorders.

SCN2A-related disorders are a group of rare genetic conditions characterized by changes in the SCN2A gene. These changes give rise to an array of neurological and developmental symptoms, including [epilepsy](#), intellectual disabilities, and [autism](#) spectrum disorders. This study has the potential to help frame the clinical needs of SCN2A patients and to reinforce benchmarks used in clinical trials to support the development of new treatments.

“We are embarking on an extraordinary journey to launch an international registry for SCN2A-related disorders called the DRAGONFLY study, which will enhance our understanding and management of these complex conditions,” said Leah Myers, Founder and Executive Director of the FamilieSCN2A Foundation. “Our vision extends beyond today; it’s a vessel to drive progress,



The DRAGONFLY Study is launching soon.

accelerate therapies, and ultimately bring the light of discovery to the SCN2A community."

The registry will be hosted on NORD's IAMRARE® online platform.

FamilieSCN2A is a member of NORD, and these two organizations will work together to understand the challenges and to identify opportunities to advance research for this rare disease patient population. The IAMRARE® platform is designed to allow us to track the course of SCN2A-related disorders over time in a set of electronic surveys completed by patients, or their caregivers, from anywhere in the world. The data is confidential and stored securely on the platform. One key goal of the FamilieSCN2A Foundation is to share the collected data with individuals or institutions conducting research or clinical trials in SCN2A-related disorders.

The FamilieSCN2A Foundation - learn the difference 'families' make.



“The DRAGONFLY Study was set up for success by having members of the SCN2A community actively participate in its design. Combined with the input of experts, this ensures that our surveys collect the most relevant information necessary to advance research and tell the important stories our community needs to share,” said Myers.

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*Leah Myers, Executive Director
for the FamilieSCN2A
Foundation*

Jenny Burke, the FamilieSCN2A Foundation Board Chair adds, "The DRAGONFLY Study is the next step in our efforts to combine ALL available patient data on SCN2A-related disorders, a significant value for researchers." The Foundation recently shared its SCN2A Clinical Trial Readiness Study data with the Critical Path Institute's (C-

Path) Rare Disease Cures Accelerator - Data and Analytics Platform (RDCA-DAP®), an FDA-funded initiative to support and accelerate rare disease therapy development. C-Path has also partnered with NORD to leverage its IAMRARE® registry platform.

There is no cure for SCN2A-related disorders. Current treatments primarily focus on managing

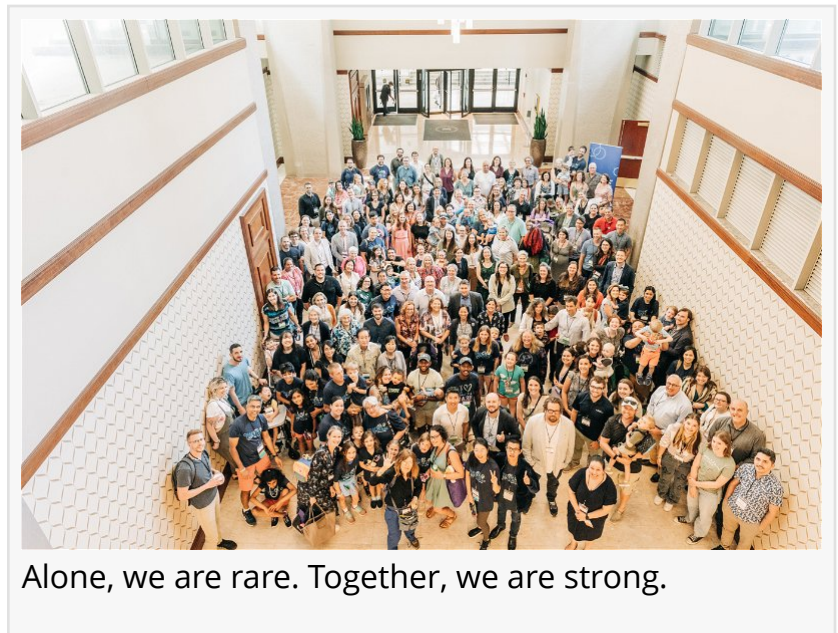
the symptoms and providing supportive care.

About FamilieSCN2A Foundation:

FamilieSCN2A Foundation is a nonprofit organization created by parents of children diagnosed with rare forms of epilepsy and autism because of a change in the SCN2A gene.

Founded in 2015, the FamilieSCN2A Foundation works to build a world with effective treatments and cures for all SCN2A-related disorders. Its mission is to accelerate research, build community, and advocate to improve

the lives of those affected by SCN2A-related disorders around the world. With unwavering dedication, the Foundation provides a nurturing and informative community where clinicians, scientists, parents, caregivers, and individuals with SCN2A-related conditions come together to share experiences, knowledge, and hope. Visit scn2a.org.



Alone, we are rare. Together, we are strong.

About National Organization for Rare Disorders, Inc. (NORD®):

The National Organization for Rare Disorders (NORD) is the leading independent advocacy organization representing all patients and families affected by rare diseases in the United States. NORD began as a small group of patient advocates that formed a coalition to unify and mobilize support to pass the Orphan Drug Act of 1983. Since then, the organization has led the way in voicing the needs of the rare disease community, driving supportive policies, furthering education, advancing medical research, and providing patient and family services for those who need them most. Together with over three hundred disease-specific member organizations, more than 17,000 Rare Action Network advocates across all 50 states, and national and global partners, NORD delivers on its mission to improve the lives of those impacted by rare diseases. Visit rarediseases.org.

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