FamilieSCN2A Foundation Awards $75,000 Action Potential Grant for Breakthrough RNA Gene-Splicing Research

Netherlands geneticist, neurobiologist Dr. Nicky Scheefhals will investigate a novel therapeutic strategy designed to reduce neuronal hyperexcitability in SRDs



Take Action, Create Potential

SCN2A-RELATED AUTISM & EPILEPSY
FAMILIESCN2A®
FOUNDATION
'Families' is in our name for a reason.

GETTYSBURG, PA, UNITED STATES, September 11, 2026 / EINPresswire.com/ -- The FamilieSCN2A Foundation is pleased to announce the recipient of its 2026 Action Potential Grant, awarding the grant to Dr. Nicky Scheefhals. Scheefhals serves as a geneticist and neuroscientist within the Department of Human Genetics at Radboud University Medical Center, as well as the Donders Institute for Brain, Cognition and Behaviour located in Nijmegen, Netherlands.

SCN2A-related disorders are severe genetic conditions caused by alterations in the SCN2A gene. The gene contains two mutually exclusive exons: exon 5N, expressed predominantly during neonatal development, and exon 5A, expressed as the brain matures into adulthood. Because the neonatal channel variant (exon 5N) requires stronger electrical depolarization to activate, it acts as a natural intrinsic brake on brain cell excitability, similar to a built-in speed limiter that caps a vehicle's maximum speed to keep it driving safely.

“Our top priority is getting real treatments to our families as quickly as possible,” said Leah Schust Myers, Executive Director of the FamilieSCN2A Foundation. “SCN2A is a global disorder, and we are proud to support families and advance science around the world. This project represents exactly the kind of innovative thinking we want to encourage—approaches with the

> Our top priority is getting treatments to families as quickly as possible. Instead of trying to fix hundreds of rare mutations one at a time, this research targets the root cause of the condition."
>
> *Leah Myers, co-founder and Executive Director*

potential to help groups of patients rather than addressing mutations one at a time. Supporting early-stage science like this can help promising ideas move toward therapeutic development and, ultimately, bring us closer to effective, life-changing treatments for families everywhere."

Dr. Scheefhals' research project, titled "Therapeutic Modulation of SCN2A Exon Switching to Reduce Neuronal Hyperexcitability in SCN2A-related Disorders", targets this precise developmental mechanism. Rather than trying to design custom therapies for hundreds of individual, patient-specific mutations, her strategy uses RNA-based antisense oligonucleotide molecules to artificially shift splicing back from the adult exon 5A to the protective neonatal exon 5N. By restoring neonatal exon expression, the therapy aims to decrease the activity of hyperactive sodium channels and thereby directly lower neuronal hyperexcitability, a mechanism with broad potential application for patients driving excessive cellular firing. In other words, instead of trying to patch hundreds of unique genetic typos one by one, this research forces brain cells to switch back to their naturally calmer, infant-stage settings to reduce overfiring, and in turn, seizures. This is a novel and unique approach with broad potential for therapeutic applications across the SCN2A-related disorder population.

"I'm incredibly grateful to the FamilieSCN2A Foundation for supporting this project. What excites me most is that we can potentially make use of a natural developmental switch that already exists in the brain to reduce neuronal hyperexcitability. I'm looking forward to testing whether this approach can open up a new therapeutic avenue for people with SCN2A-related disorders. It is especially meaningful to do this together with such an engaged community, bringing together patients and families, scientists and clinicians with the shared goal of finding new treatments," said Dr. Scheefhals.

The Foundation selected the project because it combines a well-defined biological mechanism with the potential to address a broader group of patients than a variant-specific therapeutic strategy. "Scientifically, what makes this approach particularly exciting is that it takes advantage of a naturally occurring mechanism for regulating Nav1.2 function rather than requiring us to engineer an entirely new one," said Jeffrey Cottrell, Ph.D., Interim Chief Scientific Officer of the FamilieSCN2A Foundation. "If shifting exon usage can meaningfully reduce the hyperexcitability caused by gain-of-function SCN2A variants, it could provide a therapeutic strategy applicable across multiple variants and potentially reach a much larger group of patients. The Action Potential Grant program is designed to give innovative ideas like this the opportunity to generate the proof-of-concept data needed to determine whether they can become real therapeutic programs."

About the Action Potential Grant Program
The Action Potential Grant is an investigator-initiated funding mechanism created by the FamilieSCN2A Foundation to accelerate early-stage, translational research. Specifically designed to fund novel proof-of-concept studies, the program targets high-priority projects that advance understanding of disease mechanisms or explore potential therapeutic interventions. By providing seed funding to scientists studying SCN2A-related disorders, the foundation helps researchers generate the preliminary data required to validate new treatment concepts and lay the foundation for future clinical development.

About FamilieSCN2A Foundation
The mission statement of the FamilieSCN2A Foundation is to accelerate research, build community, and advocate to improve the lives of those affected by SCN2A-related disorders around the world. The FamilieSCN2A Foundation has awarded Action Potential Grants since 2019 and invested more than $9.5 million in research funding since its founding in 2014.

Leah Myers
FamilieSCN2A Foundation
+1 301-252-8070
email us here
Visit us on social media:
LinkedIn
Bluesky
Instagram
Facebook
YouTube
TikTok
X
Other

This press release can be viewed online at: https://www.einpresswire.com/article/940630533

EIN Presswire's priority is source transparency. We do not allow opaque clients, and our editors try to be careful about weeding out false and misleading content. As a user, if you see something we have missed, please do bring it to our attention. Your help is welcome. EIN Presswire, Everyone's Internet News Presswire™, tries to define some of the boundaries that are reasonable in today's world. Please see our Editorial Guidelines for more information.
© 1995-2026 Newsmatics Inc. All Right Reserved.