FamilieSCN2A Foundation Announces 2025 Action Potential and Accelerator Award Recipients

GETTYSBURG, PA, UNITED STATES, August 19, 2025 /EINPresswire.com/ -- The FamilieSCN2A Foundation recently announced the recipients of its 2025 Action Potential and Accelerator Awards during their annual family and scientific conference in Denver, Colorado. These prestigious awards recognize groundbreaking scientific innovation and commitment to finding treatments for SCN2A-related disorders (SRDs). While rare, changes in the SCN2A gene are one of the most common monogenetic causes of profound autism, epilepsy, developmental delays, and cognitive impairment. Research that leads to treatments and cures for SRDs could impact the development of treatments for many of these conditions.

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

> ‘Mahzi’ means ‘together’ in Greek, and together we will relentlessly work toward developing a therapy for SCN2A.”
>
> *Yael Weiss, MD, PhD*

The Action Potential Award, which supports early-career investigators pursuing novel research on SCN2A disorders, was presented to Morgan Robinson, PhD, the Lillian Gilbreth Postdoctoral Fellow at Purdue University. Dr. Robinson’s project applies cutting-edge CRISPR-Cas9 prime editing technology, packaged in lipid nanoparticles developed in collaboration with experts at the University of British Columbia, to correct disease-causing changes in the SCN2A gene at the DNA level. By restoring proper SCN2A function in patient-derived neurons, this work aims to create a scalable therapeutic pipeline that can be adapted to a wide range of genetic variants.

Robinson said: "As a Cystic Fibrosis patient-scientist, I know firsthand how vital early research is for developing effective treatments for rare diseases. This Action Potential grant empowers our lab’s mission to develop a cure for patients with SCN2A disorders."

The Accelerator Award, designed to support industry-led therapeutic development, was presented to Mahzi Therapeutics, led by CEO Yael Weiss, MD, PhD. Mahzi will develop an allele-specific antisense oligonucleotide (ASO) program targeting the pathogenic SCN2A allele, with the

goal of reducing the dominant-negative effects driving disease. The initial phase will focus on ASO identification and validation in cell line models, laying the foundation for preclinical development.

"Team Mahzi is honored to be the first company to receive the Accelerator Award. It is also a great responsibility," said Dr. Weiss. "I'd like to thank FamilieSCN2A and all of those affected by SCN2A-related disorders for welcoming us, sharing your lives, and making this award possible. 'Mahzi' means 'together' in Greek, and together we will relentlessly work toward developing a therapy for SCN2A."

This year's award cycle reflected a growing global interest in SCN2A research, with eight Action Potential letters of intent, seven full Action Potential applications, three Accelerator letters of intent, and three full Accelerator applications. The process was overseen by a 29-member Grant Review Panel representing basic, translational, and clinical science, bioethics, industry, and patient perspectives.

"The caliber and creativity of proposals this year reflect an exciting new chapter in SCN2A research," said Karen Ho, PhD, of FamilieSCN2A. "Both awardees are pushing the boundaries of science in ways that bring us closer to targeted, effective therapies for our community."

Congratulations to Morgan Robinson, PhD

Congratulations to Mahzi Therapeutics

The FamilieSCN2A Foundation extends deep gratitude to the donors, volunteers, and families

whose dedication and fundraising efforts make these awards possible. The Foundation also wishes to heartily thank the Grant Review Committee, whose efforts support and guide the Foundation's wise stewardship of donated funds.

About the FamilieSCN2A Foundation
The FamilieSCN2A Foundation is a 501(c)(3) nonprofit organization dedicated to accelerating research, improving clinical care, and supporting families affected by SCN2A-related disorders. Through funding innovative research, building global collaborations, and empowering patient voices, the Foundation works toward the ultimate goal: effective treatments and cures for all SCN2A-related disorders.

About SCN2A-Related Disorders
SCN2A-related disorders (SRDs) are rare genetic conditions caused by changes in the SCN2A gene, which provides instructions for making a critical sodium channel (NaV1.2) in the brain. These disorders can present in a wide spectrum of ways, most often in early childhood, including treatment-resistant epilepsy, developmental delay, intellectual disability, autism spectrum disorder, and more. The severity and symptoms depend on the specific genetic variant, but SRDs are often life-altering, requiring intensive medical care and ongoing support. There are currently no approved disease-modifying treatments for SRDs, making research and therapeutic development an urgent priority for affected families.

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