

2022 THROUGH 2025



STRATEGIC PLAN



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

Our vision is a world with effective treatments and cures for all SCN2A-related disorders.

Our mission is to accelerate research, build community, and advocate to improve the lives of those affected by SCN2A-related disorders around the world.



OVERVIEW



Dear Friends,

It is with great enthusiasm that we present the FamilieSCN2A 2022 - 2025 Strategic Plan. Before you take a dive into this exciting document, we would like to share a new way of referring to SCN2A-related disorders. To simplify things, we will begin using 'SRD' to represent 'SCN2A-related disorder' in this strategic plan and moving forward.

The mission 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. To ensure that our strategic direction is aligned with our mission, the FamilieSCN2A Foundation Board of Directors developed this strategic plan to execute impactful programs for the SCN2A community. We began by gathering data from our patient and professional community through surveys and discussion groups. The data was then carefully analyzed and translated into visionary goals that will drive change.

With your support since 2015, we have steadily constructed a strong foundation that is anchored by our dedication to: **research**, **community**, and **advocacy**. In 2022, our focus has been on **building**; building towards a CURE! The CZI: Rare As One Grant Network has energized us to be bold as we take the leap forward to expand our infrastructure. This capacity building will ultimately allow us to fulfill our mission even more quickly. Over the next three years, we are poised to make the most of the \$600K CZI grant and access to the impressive network of CZI resources to strengthen our organizational capacity, grow our community, and advance international collaborative research toward shared priorities. Together we will build towards a cure!

While we are bold in our goals, we set them while holding a mirror to ourselves, as we are committed to ensuring the goals are within our organization's strengths and capacity. Integrity is one of our prioritized values: We say what we mean and we do what we say. This document gives an overview of the goals we are setting for the next three years. We share this information standing by our values of urgency, integrity, collaboration and inclusion, so that we are transparent with our full community and hold ourselves accountable for making an impact.

With hope and gratitude,
FamilieSCN2A Foundation Board of Directors and Staff

A special thank you to the Chan Zuckerberg Initiative for supporting our strategic plan development.



OUR STRATEGIC OBJECTIVES



Three strategic objectives represent key focus areas aimed at improving the lives of those affected by SRDs.

1

RESEARCH

Coordinate and collaborate with researchers in order to accelerate scientific investigation of SCN2A.

2

COMMUNITY

Provide educational, emotional, financial and clinical support for patients and families suffering from SRDs.

3

ADVOCACY

Increase outreach in order to advance important issues and to improve the lives of those impacted by SRDs.

CURRENT STATE OF RESEARCH



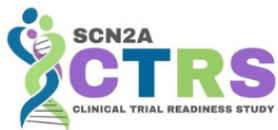
The FamilieSCN2A Foundation is committed to supporting research that advances understanding of the cellular, molecular, genetic, and systems-level mechanisms of SRDs.

We do this through:

take
ACTION,
create
POTENTIAL

The FamilieSCN2A Action Potential Grant

The Action Potential Grant is an investigator-initiated grant program intended to accelerate the development of therapeutic treatments and disease-modifying advancements for those living with changes in the SCN2A gene. This grant program is designed to facilitate preliminary investigations that will potentially lay the groundwork for subsequent grants from the government, industry, or other funding sources, including the FamilieSCN2A Foundation.



Clinical Trial Readiness Study (CTRS)

The SCN2A CTRS helps to determine the performance of specific functional, adaptive behavior, and related measures over time in children with SRDs. This study will provide information on the stability of these measures and the extent to which they change with age. For therapeutic trials in which these measures could be used as primary or secondary outcomes, the data from this study will provide the basis for designing an efficient and robust trial.



CuATSM Study

FamilieSCN2A Foundation sponsored 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. We are working to advance the program toward clinical trials in SCN2A.

Ongoing Research in SRDs

Other partners in SRD research are acknowledged below. For a full description of research activities in SCN2A, please visit www.scn2a.org/research

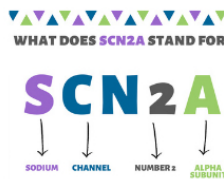


CURRENT COMMUNITY PROGRAMS



The FamilieSCN2A Foundation is dedicated to providing community programs to individuals living with SRDs and their families. We are proud to offer the following mechanisms to help those affected by SCN2A.

Community programs include:



Newly Diagnosed Toolkit



Birthday Club



Global Support Network



Family Conference & Scholarship Program



Warrior Wednesday



Patient Assistance Grant



Crisis & Bereavement

CURRENT ADVOCACY INITIATIVES



The FamilieSCN2A Foundation raises public awareness of SRDs through various platforms and media in addition to establishing an international presence of the disorder through awareness campaigns and initiatives.

Our advocacy initiatives include:



Held annually on February 24th, FamiliesSCN2A engages families across the globe for International SCN2A Awareness Day.



FamilieSCN2A issues a quarterly newsletter to constituents with regular research, awareness, and event updates.



FamilieSCN2A has produced a series of educational videos on SCN2A, delivered by experts in the field.



FamilieSCN2A is actively engaged on social media. Join us on Facebook, Twitter, Instagram, Pinterest and YouTube.

OBJECTIVES & GOALS



FamilieSCN2A identified three major goals to support each strategic objective:

1 RESEARCH

2 COMMUNITY

3 ADVOCACY

While broad goals were identified during the FamilieSCN2A Strategic Planning meeting in March 2022, they were further prioritized by the SCN2A community through surveys and discussion.



RESEARCH PRIORITIES



Coordinate and collaborate with researchers around the world to find effective treatments and cures for all SRDs



TOP PRIORITIES:

1

PATIENT-LEVEL VARIANT CHARACTERIZATION

Expanding the knowledge of how each unique variant within SCN2A affects the patient in order to guide treatment decisions and clinical trial selection.

2

DEVELOP A RESEARCH ROADMAP FOR SCN2A

Efficiently and effectively allocate resources to the projects that meet our vision and mission.

3

FOUNDATION-SUPPORTED, TARGETED THERAPEUTIC RESEARCH

Ensuring the patient voice is integrated into the drug development process from the start.

COMMUNITY PRIORITIES



Provide resources for educational, emotional, financial, and clinical support for patients and families suffering from SRDs



TOP PRIORITIES:

1 **Develop Diagnosis and Treatment Guidelines (Standards of Care Protocol) for SRDs.**

Expanding the knowledge of how each unique variant within SCN2A affects the patient in order to guide treatment decisions and clinical trial selection.

2 **Support/coordinate establishment of 'centers of excellence' for SCN2A to serve the complex medical needs of patients and provide resources for families to access the centers.**

Efficiently and effectively allocate resources to the projects that meet our vision and mission.

3 **Maintain comprehensive resource list on website that addresses financial, emotional, and educational support for patients and their families.**

Ensuring the patient voice is integrated into the drug development process from the start.

*Based on feedback collected from community surveys in mid-2022

ADVOCACY PRIORITIES



Increase outreach in order to advance important issues and to improve the lives of those impacted by SRDs



TOP PRIORITIES:

1 Create a global SCN2A awareness campaign

Although SCN2A is one of the leading single-gene causes of neurodevelopment disorders, it is significantly under-diagnosed. It is important to know the cause of disease as new gene-modifying treatments with the possibility of improving quality of life are in the pipeline.

2 Increase awareness and advocate for the need and utility of genetic testing through commercial partnerships, media campaigns, and by potentially funding testing in underserved populations

Genetic testing is the only way to identify SCN2A patients.

3 Lobby for state and federal funding for SRDs

Urgency is a core value of the Foundation's because our patients' futures are not promised. Funding for translational science is critical to the survival and quality of life of the patients.

*Based on feedback collected from community surveys in mid-2022

HOW WE WILL GET THERE



FamilieSCN2A understands that in order to meet our strategic goals now and in the future, it is essential to set ourselves up for long-term success. The following four categories broadly reflect our sustainability initiatives to help us achieve our goals and are based on feedback from our community members.



Increase development strategies, including engagement opportunities with families.

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Continue to partner with researchers, harness existing relationships, and build new ones.

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Strengthen communications and marketing.

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Focus on diversity, equity, and inclusion; ensure FamilieSCN2A Foundation is representative of all patients around the world.

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"Families" is part of our name for a reason. Rare and devastating, SCN2A-related disorders (SRDs) affect the entire family. Our team of leaders strive every day and in every way to improve the lives of not only the patient, but the entire family.



STAY IN THE FAMILY

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 @FAMILIESCN2A FOUNDATION

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