FamilieSCN2A and Cellectricon Partner to Accelerate Drug Development for a Top Autism and Epilepsy Gene, SCN2A

Powerful new partnership to lead to faster cures for SCN2A-related disorders

GETTYSBURG, PA, UNITED STATES, February 24, 2025 /EINPresswire.com/ -- The FamilieSCN2A Foundation, the leading nonprofit organization in the world dedicated to improving the lives of individuals affected by SCN2A -related disorders (SRDs), is proud to announce a strategic partnership with Cellectricon, a cutting-edge biotechnology company specializing in advanced neuronal assay technologies. This collaboration aims to accelerate the development of targeted therapies for SRDs.

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SCN2A encodes the sodium channel NaV1.2, which helps neurons send electrical signals. In other words, it plays a key role in brain function and communication between nerve cells. Variants in SCN2A can result in either gain-of-function (GoF), associated with early-onset epilepsy, or loss-of-function (LoF), linked to intellectual disability and Autism Spectrum Disorder (ASD). However, many variants exhibit mixed effects, complicating treatment strategies. SRD is a severe, and life-threatening condition with no approved treatments, underscoring the urgent need for targeted therapeutic development.

Cellectricon was selected as a partner for its innovative screening technology, which enables direct measurement of how SCN2A variants impact neuronal activity in human stem cell-derived neurons. Unlike typical methods that rely on non-neuronal cell lines and provide limited insights, Cellectricon's platform combines advanced high-throughput electrode and fluorescence imaging systems to measure neuronal excitation in real-time. This unique approach ensures precise, reproducible data by using controlled electrical stimulation rather than relying on inconsistent

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> *Shawn Egan, PhD, Chief Scientific Officer for the FamilieSCN2A Foundation*

spontaneous activity, as used by other assay platforms. By offering a clearer understanding of how SCN2A mutations directly affect neuron function, this breakthrough technology paves the way for developing targeted SRD therapies much faster.

"The Cellectricon team is honored to have been selected as a partner to the FamilieSCN2A Foundation," said Mathias Karlsson, Chief Executive Officer at Cellectricon. "We are very much looking forward to this collaboration where we see great opportunity in applying our deep know-how and unique approach to probing neuronal activity in human stem cell-derived models to improving the lives of individuals affected by SRDs."

This research initiative aims to bring both immediate and long-term benefits to patients and families affected by SRDs. In the short term, researchers will identify how specific SCN2A variants impact neuronal activity and screen potential compounds that could restore normal function. This could lead to precision therapies tailored to individual variants, with the possibility of benefiting a wider patient population. In the long run, the project aims are to develop and validate a standardized screening assay that can be used by researchers and pharmaceutical companies worldwide. This tool will help characterize SCN2A variants, test potential treatments, and accelerate drug development. Additionally, all cell lines created during the project will be made available to the broader scientific community, further advancing research and treatment options for SRDs.

FamilieSCN2A is excited to collaborate with Cellectricon to develop critical infrastructure for SCN2A drug development, believing that Cellectricon's expertise and these efforts will provide the fastest and most effective way to assess the therapeutic potential of treatments for SCN2A-related disorders (SRDs). Once this assay is validated and an SCN2A allelic series of NGN-2 iPSCs is established and housed at Cellectricon, it is expected that there will be significantly shorter drug development timelines, increased interest from drug developers willing to test at Cellectricon, and the ability for FamilieSCN2A to directly evaluate compounds which will collectively accelerate the Foundation's vision of a world with effective treatments and cures for all SCN2A-related disorders.

Furthermore, it is believed that this assay has best-in-class potential. Validation in SCN2A would likely suggest utility across other developmental and epileptic encephalopathies (DEEs) and other neurodevelopmental disorders which would create value for the entire space.

"FamilieSCN2A is excited to collaborate with Cellectricon to develop critical infrastructure for SCN2A drug development," said Shawn Egan, Chief Scientific Officer of FamilieSCN2A.

“Cellectricon’s expertise will help accelerate the evaluation of potential treatments for SCN2A-related disorders (SRDs), shortening drug development timelines and attracting more interest from pharmaceutical partners. Additionally, its potential extends beyond SCN2A, with possible applications across other developmental and epileptic encephalopathies (DEEs) and neurodevelopmental disorders.”

Forward-Looking Statement:
This collaboration presents an opportunity for investors and biotech companies to fast-forward drug development for SCN2A-related disorders and potentially other DEEs and neurodevelopmental disorders. If interested in partnership on this opportunity please contact FamilieSCN2A Foundation’s Chief Scientific Officer, Shawn M Egan, PhD at shawn.egan@scn2a.org.

About FamilieSCN2A Foundation
The FamilieSCN2A Foundation is a parent-led organization dedicated to improving the lives of those affected by SCN2A-related disorders, which cause rare forms of epilepsy and autism. Their mission is to accelerate research, build a supportive community, and advocate for effective treatments and cures worldwide. Founded by families who understand the challenges firsthand, they strive to bring hope and progress to individuals and their loved ones. With a vision of a world where SCN2A-related disorders are treatable, they work tirelessly to advance science and support those in need.

About Cellectricon
Cellectricon is a leading provider of neurobiology in vitro research services. The company’s mission is to create groundbreaking in vitro concepts that will advance the neuroscience research field and enable clients from the pharmaceutical industry to conduct successful drug discovery programs. The experienced team of neurobiologists combines drug discovery expertise and disease area knowledge with cutting-edge technologies to create complex disease models and assays for functional and morphological screening.

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