FamilieSCN2A Foundation Announces Inaugural $2.5M Hodgkin-Huxley Research Award

Yang Yang, PhD, of Purdue University is the recipient of a major 5-year grant for Personalized Treatment Development in SCN2A-Related Disorders

EAST LONGMEADOW, Mass., May 9, 2023 (Newswire.com) - The FamilieSCN2A Foundation, a nonprofit organization dedicated to improving the lives of individuals with SCN2A-related disorders (SRDs), has awarded a first-stage $2.5 million dollar research grant to a Purdue University researcher.

Yang Yang, associate professor of Medicinal Chemistry and Molecular Pharmacology in Purdue's College of Pharmacy received the grant to support the development of personalized treatments for this group of disorders after peer-review by scientific experts in the field.

The grant was funded by a restricted, single-family donation through the *FamilieSCN2A* Foundation's Hodgkin-Huxley (HH) Research Grant. The grant was named to honor Nobel Prize winners Dr. Alan Hodgkin and Dr. Andrew Huxley for their innovative discovery of action potentials in neurons.

SRDs are a group of rare genetic conditions caused by mutations in the SCN2A gene, which encodes an ion channel that is essential for the function of brain cells. These disorders can cause a wide range of neurological symptoms including difficult-to-control seizures, profound autism, developmental delays, movement disorders, and intellectual disabilities.

The five-year grant will support Yang's research aimed at developing personalized treatments for a splice-site *SCN2A* variant through n=1 drug development. The goal of n=1 projects is to create treatments tailored to the unique genetic and clinical profiles of individual patients, with the hope of improving outcomes and quality of life for all those affected by these disorders.

"We are incredibly grateful to the family who helped make this grant possible," said Leah Myers, founder of the FamilieSCN2A Foundation. "Through their generosity and commitment to research, this grant will help to accelerate the development of personalized treatments for SRDs, a critical need for families affected by these devastating conditions."

Shawn Egan, Chief Scientific Officer for the FamilieSCN2A Foundation agreed.

"We expect this project to take a large leap forward into the understanding and treatment of SRDs with splice site mutations, which is an area that has been understudied to date," he said.

The FamilieSCN2A Foundation and its scientific advisors reviewed the project proposal prior to awarding the grant and will manage the project to ensure that it meets the highest scientific standards while advancing the foundation's mission.

Yang said he is honored to have received the award and looks forward to the research advances it will enable.

"I am extremely honored and privileged to receive this grant from the *FamilieSCN2A* Foundation to embark on this uncharted journey to fight SRD together with the families," he said. "This grant is built on discoveries that were funded by prior *FamilieSCN2A* Awards and National Institutes of Health (NIH) research grants, which laid a solid foundation for us to take the next giant leap to tackle SRDs.

"With this award, we are excited to build new disease models and work on developing personalized treatments for SRDs, including cutting-edge pharmacological and genetic interventions. We greatly appreciate the trust from the donor family with this substantial award to initiate this high-risk/high-reward research to advance transformative n=1 therapeutic discovery and investigate innovative approaches to drug development for many. I believe this award will help us move closer to a future where individuals with SRDs can receive treatments tailored to their specific needs."

The award will contribute to the Purdue College of Pharmacy's robust pursuit of impactful discoveries that improve lives, said Eric Barker, Jeannie and Jim Chaney Dean of Pharmacy.

"The Purdue College of Pharmacy is grateful to the FamilieSCN2A Foundation for supporting Dr. Yang's impactful research," he said. "We know that in situations like this, the passion for discovery is fueled by deeply personal motivations for the families involved. We are committed to advancing basic and translational research that helps move discoveries from the laboratory to directly improving patients' lives through new therapeutic interventions."

According to Yang, most rare diseases are genetic disorders and do not have a cure; however, recent progress in precision medicine and gene therapy are offering hope for treating these types of disorders.

The Hodgkin Huxley Award will advance precision and personalized medicine to treat these disorders and improve quality of life for the people affected by them. It will also greatly contribute to ongoing translational research within the Purdue College of Pharmacy's Department of Medicinal Chemistry and Molecular Pharmacology, and the growing drug discovery pipeline at Purdue University, said

Department Head Zhong-Yin Zhang, who also directs Purdue's Institute for Drug Discovery.

"Dr. Yang's research has tremendous potential to improve the lives of countless people through personalized therapies," he said. "We are extremely grateful to the donor family and to the FamilieSCN2A Foundation for their generosity in advancing this incredibly important research."

Yang specializes in neurological diseases, including autism, epilepsy, pain, and dementia; pharmacogenomics; and gene therapy. He is a leading investigator for the Purdue Institute for Integrative Neuroscience (PIIN), said Chris Rochet, Professor of Medicinal Chemistry and Molecular Pharmacology and the John and Donna Krenicki Director of PIIN.

PIIN is committed to unraveling mysteries of the healthy brain and advancing treatment of central nervous system disorders.

"Since arriving at Purdue in 2017, Dr. Yang has made important contributions to the field of neuroscience by discovering changes in neuron firing that account for altered brain function in individuals with epilepsy or autism spectrum disorder," Rochet said. "We thank the FamileSCN2A Foundation for their generous investment in Dr. Yang's research program, and offer our full support to help maximize the long-term impact of this groundbreaking study."

The FamilieSCN2A Foundation is committed to accelerating research, building community, and advocating to improve the lives of those affected by SCN2A-related disorders around the world.

For more information about the FamilieSCN2A Foundation and its mission, or to donate to this cause visit www.scn2a.org.

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About FamilieSCN2A Foundation

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Company Address

FamilieSCN2A Foundation
East Longmeadow, MA
United States