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Rare Genomics Institute BeHEARD Challenge Awards 21 Rare Disease Grants

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Unique biotechnology contest allows companies to contribute their technology to make a difference for the rare disease community

The Rare Genomics Institute (RG) announced the winners of the 2019-2020 BeHEARD (Helping Empower and Accelerate Research Discoveries) Rare Disease Science Challenge, a global competition that offers rare disease researchers, who traditionally have difficulty attracting funding, grants of the latest life science innovations and technologies. This unique biotechnology contest allows companies to contribute their technology to make a difference for the rare disease community.

“This year the competition received submissions on 27 rare diseases, coming from universities and foundations located in thirteen different countries,” said Danielle Fumagalli, BeHEARD Director. “Twenty-one cutting-edge technology prizes were awarded to study a number of rare diseases globally.”

Rare diseases affect more than 300 million worldwide, yet less than five percent of the 7,000 known rare diseases have treatments available. The winning scientists from BeHEARD will use their awards to yield key medical research insights on rare diseases that can lead to new treatments for patients.

Two examples are a mouse model, sponsored by Taconic Biosciences, and a TurboKnockout® mouse model vector construction, sponsored by Cyagen Biosciences, that were awarded to Prof. Puneet Opal at Northwestern University. These technology grants will allow his lab to test therapy options for Giant Axonal Neuropathy, a severe neurological disorder with no effective treatments. Beginning in early childhood, Giant Axonal Neuropathy causes progressively worsening balance issues, muscle weakness, seizures, paralysis, and dementia. Most sufferers become bedridden by early adulthood and die in their twenties. Prof. Opal will use the awarded mouse model and vector construction to test whether administering a replacement version of the defective protein that causes Giant Axonal Neuropathy can stop or reverse the disease.

Other winners were awarded vector constructions from Cyagen Biosciences, PCR design and analysis software from DNA Software, reagents from Addgene, and antibodies, proteins, and peptides from Bio-Techne. These technologies will be put to good use to make progress on therapies for rare diseases, ranging from the FOXG1 Research Foundation's study of FOXG1 Syndrome, a severe brain disease that causes seizures and lack of body control, to the Jansen's Foundation's research on Jansen's metaphyseal chondrodysplasia (JMC), a skeletal disease that causes short stature, bowed legs, and joint deformities.

A full list of the BeHEARD winners is below:

Cyagen TurboKnockout® Vector Construction

Puneet Opal, Northwestern University, Giant Axonal Neuropathy

Nasha Fitter, FOXG1 Research Foundation, FOXG1 Syndrome

Heike Rebholz, Institute of Psychiatry and Neurosciences of Paris, Okur Chung Neurodevelopmental Syndrome

Salisu Balarabe, Usmanu Danfodiyo University Sokoto, Post-traumatic Epilepsy

Rebecca Sloan, Axenfeld-Rieger Foundation, Axenfeld-Rieger Syndrome

Cyagen Transgenic Vectors

Neena Nizar, The Jansen's Foundation, Jansen's Metaphyseal Chondrodysplasia

Cyagen Rosa26 CRISPR Knock-In Vector Construction/F1 Breeding

Sebastien Gauvrit, Max Planck Institute for Heart and Lung Research, Sturge-Weber Syndrome

Andres Muro, International Centre for Genetic Engineering and Biotechnology, Pompe Disease

Or Kahklon, Hadassah-Hebrew University Medical Center, Cori Disease

Lawrence Ostrowski, UNC-Chapel Hill, Primary Ciliary Dyskinesia

Neena Nizar, The Jansen's Foundation, Jansen's Metaphyseal Chondrodysplasia

Taconic Mouse Model

Puneet Opal, Northwestern University, Giant Axonal Neuropathy

DNA Software PCR Design and Analysis Software

Nasha Fitter, FOXG1 Research Foundation, FOXG1 Syndrome

Neena Nizar, The Jansen's Foundation, Jansen's Metaphyseal Chondrodysplasia

Salisu Balarabe, Usmanu Danfodiyo University Sokoto, Post-traumatic Epilepsy

Bio-Techne Antibodies, Proteins, and Peptides

Melanie Gillingham, Oregon Health and Science University, Long-chain 3-HydroxyacylCoA Dehydrogenase Deficiency

Bilon Khambu, Tulane University, Primary Sclerosing Cholangitis

Addgene Reagents

Terence Hebert, McGill University, GNB1 Disorder

Siddharth Prakesh, UT Health Science Center at Houston, Turner Syndrome

Mark Mellett, University Hospital Zurich, Generalized Pustular Psoriasis

Laura Trutoiu, Association for Creatine Deficiencies, Creatine Transporter Deficiency