NEWS

Gene therapy project receives major funding

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$1.14 million from CIHR will resolve treatment roadblocks for rare brain disorders

Gene therapy for rare neurological disorders will move one step forward thanks to a $1.14 million grant from the Canadian Institutes of Health Research (CIHR).

A collaboration between scientists at The Neuro, the Montreal General Hospital, the Montreal Children's Hospital and McGill University, this project will build capacity in gene therapy for rare neurological diseases treatment. Scientists will do this by comparing therapy delivery methods for safety and effectiveness.

The project will be led by Carl Ernst, PhD, from The Neuro, along with Dr. Guy Rouleau, Dr. Jason Karamchandani, Justine Clery, PhD, Sali Farhan, PhD, Dr. Massimo Pandolfo, Keith Murai, PhD, Dr. Maryam Oskoui, Dr. Ken Myers, and Benoit Gentil, PhD. The team will benefit from guidance from Sonia Gobeil, a mother of two children with a rare form of ataxia, who will provide the family perspective for research team.

“The institutions involved have a long history of treating neurological and neurodevelopmental disorders, including clinical trials involving gene therapy,” says Prof. Ernst. “Through our work in caring for patients, we and others have recognized several unique neurological syndromes which affect very few patients, and treatment options are typically limited or non-existent. With the ability to rapidly sequence whole genomes, many of the rare neurological and neurodevelopmental disorders that we see are now better characterized, and many of these pathologies represent monogenetic syndromes that could benefit from gene therapy. This grant will really help us move forward with new ways to more rapidly get treatments to patients with rare diseases.”

The research will benefit from the guidance of Sonia Gobeil, a mother of two children with a rare form of ataxia and co-founder of the Ataxia of Charlevoix-Saguenay Foundation. She will provide the family perspective for to the scientists.

“This is a significant and impactful project,” says Gobeil. “Gene therapy has the potential to significantly change the lives of those affected with a rare brain disorder like the one affecting my family.”

About the grant

The CIHR’s operating grant, entitled “Bringing Rare Disease Gene Therapies to Clinical Trial Readiness”, is part of the National Strategy for Drugs for Rare Diseases and is supported through Federal Budget 2019. The goal of the grant is to increase development of gene therapies for rare disease clinical trials in Canada, generate the evidence required for first-in-human clinical trials, in part by working with Canada’s biomanufacturing capacity and health technology regulator, and increase capacity within Canada to improve readiness of gene therapies for first-in-human clinical trials.

Related Links

Bringing Rare Disease Gene Therapies to Clinical Trial Readiness (https://cihr-irsc.gc.ca/e/53464.html)
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