FIGHTING BLINDNESS CANADA | VAINCRE LA CÉCITÉ CANADA

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THE FUTURE IS BRIGHT: HEALTH CANADA APPROVES FIRST SIGHT-RESTORING GENE THERAPY FOR INHERITED EYE DISEASES

TORONTO, October 15, 2020 – On October 14, 2020, Health Canada announced their approval of the first sight-restoring innovative treatment voretigene neparvovec (Brand name: Luxturna). This announcement is the first targeted gene therapy approved in Canada for any disease. This treatment is specifically for individuals with the inherited blinding eye diseases retinitis pigmentosa (RP) or Leber congenital amaurosis (LCA) resulting from mutations in the RPE65 gene that leads to vision loss and eventually blindness.

"Lives will be transformed in Canada because of approval of this research-delivered groundbreaking treatment. For the first time, it means we have a treatment option – bringing hope to families affected by genetic mutations causing blindness and to everyone in the vision loss community." says Doug Earle, President & CEO, Fighting Blindness Canada. "The cost of illness for inherited retinal diseases is $1.6 billion according to IRD Counts Canada released on October 8, 2020. Over 80% of these costs are borne by families. Today, research delivered hope to these families with this treatment."

For families like the Gandhi's, the approval of this treatment has been years in the waiting. Sophia Gandhi, mother of Zara, 10 years old born with blindness due to LCA from the RPE65 gene mutation says, "Zara was diagnosed with blindness at 6 months old. It was very difficult on our family, we didn't know anyone that could relate. We had great doctors, but treatment options were not available to help improve Zara's limited sight. When we learned about Luxturna many years ago, we knew one day it must and would come to Canada, it was only a matter of time. The time has now come – this can change Zara's life."

Dr. Elise Héon a clinician-scientist in the field of ocular genetics with The Hospital for Sick Children in Toronto, and Zara's eye doctor says, "This milestone approval of Luxturna is a significant step forward in the treatment of inherited blinding eye diseases. Until now, patients like Zara had no treatment options and the progression towards complete blindness was inevitable. These families now have hope with the promise of a one-time treatment option that can improve or restore vision, especially night vision." The Hospital for Sick Children will be one of the sites for this treatment in Canada.

While this treatment has been approved by Health Canada, families are eagerly awaiting recommendations from Canadian Agency for Drugs and Technologies In Health (CADTH) and

the Institut national d'excellence en santé et en services sociaux (INESSS) for coverage in provincial drug benefit programs later this fall.

Earle adds, "People with vision loss due to the RPE65 mutation for the first time in history have a chance for improved vision. And now, it's the provincial governments turn. Health Canada's approval of the treatment will not mean much if Canadian families can't afford to access it. We are calling on the provinces to fund this groundbreaking treatment for Canadian families in need."

"Fighting Blindness Canada urges all eye health practitioners to encourage their patients and families to continue supporting the science by getting their genetic testing done. Genetic testing will identify if they can be treated now and help advance research of all inherited blinding eye diseases." says Doug Earle. "Please visit FightingBlindness.ca to obtain a step by step guide of how to get genetic testing done in your province."

If you would like more information about the newly approved gene therapy treatment Luxturna, how to get genetic testing, or joining Fighting Blindness Canada's Patient Registry, please contact healthinfo@fightingblindness.ca or 1.888.626.2995.

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ABOUT VORETIGENE NEPARVOVEC AND HOW IT WORKS

Voretigene neparvovec (brand name: Luxturna) is the first targeted gene therapy now approved in Canada. It provides treatment for individuals with confirmed biallelic RPE65 mutation-associated retinal dystrophy that leads to vision loss and may cause complete blindness. The RPE65 gene provides instructions for making an enzyme (a protein that facilitates chemical reactions) that is essential for normal vision. Mutations in the RPE65 gene lead to reduced or absent levels of RPE65 activity, blocking the visual cycle and resulting in impaired vision. Individuals with biallelic RPE65 mutation-associated retinal dystrophy experience progressive deterioration of vision over time.

Voretigene neparvovec works by delivering a normal copy of the RPE65 gene using a naturally occurring adeno-associated virus that has been modified to deliver the human RPE65 gene directly to retinal cells. These retinal cells then produce the normal protein that converts light to an electrical signal in the retina to restore vision loss.

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Fighting Blindness Canada (FBC) is the largest charitable funder of vision research in Canada. With research at the heart of its focus, FBC has contributed over $40 million to the search for sight-saving cures and treatments for blinding eye diseases. With the support of its generous donors, FBC has funded over 200 research grants that explore the biology of vision, as well as essential pre-clinical and clinical vision research designed to connect patients to emerging treatments.

Media Contacts:

Jackie Rosen, Executive Assistant
416-360-4200 Ext. 246
jrosen@fightingblindness.ca

Fay Knights, Manager, Communications
1-800-461-3331 Ext. 255
fknights@fightingblindness.ca