Novartis and the pan-Canadian Pharmaceutical Alliance (pCPA) conclude negotiations for Luxturna®, a gene therapy for previously untreatable inherited vision loss[1]

Sep 21, 2022

- *Luxturna® (voretigene neparvovec) is a one-time gene therapy that provides healthy copies of the RPE65 gene for retinal cells that are lacking the normally functioning gene[1]*

Dorval, QC, September 20, 2022 — Novartis Pharmaceuticals Canada Inc. and the pan Canadian Pharmaceutical Alliance (pCPA) have successfully concluded negotiations for Luxturna® (voretigene neparvovec), a one-time gene therapy for the treatment of adult and pediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations.

Inherited retinal dystrophies (IRDs) are a major cause of early onset blindness[2]. RPE65-mediated IRDs are rare, serious, and progressive conditions that ultimately lead to severe visual impairment and blindness. Prior to the approval of Luxturna®, there were no available pharmacological treatment options for this form of inherited blindness[2].

“We are thrilled to have positively completed these negotiations for Luxturna®, one of two pioneering targeted gene therapies Novartis has introduced in Canada for patients and families devastated by rare, debilitating or life-threatening genetic diseases. For all Canadians who urgently need treatment with this innovative therapy, this is an important step to achieve access,” said Andrea Marazzi, Country President, Novartis Canada. “We will continue to work collaboratively with provincial and territorial jurisdictions so that patients whose vision is impaired as a result of a mutation in both copies of the RPE65 gene can have access to Luxturna® through public drug plans as quickly as possible.”

“This type of inherited eye disease affects children and young adults and creates a significant impact on the entire family,” said Doug Earle, President & CEO of Fighting Blindness Canada.

“For the majority, it can lead to complete blindness as cells in the retina, the light sensitive tissue of the eye, gradually stop working, work less effectively, or die[2]. Having access to a treatment that can help restore sight, can be life-altering for a child or young adult and their family. We encourage the provinces to recognize the hope Luxturna® represents and prioritize access as there are no approved alternative treatments available for these Canadians.”

About RPE65 mutation-associated inherited retinal dystrophy

Mutations in both copies of the RPE65 gene affect approximately 1 in 200,000 people and can lead to blindness[3,4]. Early in the disease patients can suffer from night blindness (nyctalopia), loss of light sensitivity, loss of peripheral vision, loss of sharpness or clarity of vision, impaired dark adaptation and repetitive uncontrolled movements of the eye (nystagmus)[4]. Patients with mutations in both copies of the RPE65 gene may be diagnosed, for instance, with subtypes of either retinitis pigmentosa or Leber congenital amaurosis[5].

About Novartis in Gene Therapy and Rare Disease

Novartis is at the forefront of cell and gene therapies designed to halt diseases in their tracks or reverse their progress rather than simply manage symptoms. The company is collaborating on the cell and gene therapy frontier to bring this major leap in personalized medicine to patients with a variety of diseases, including genetic disorders and certain deadly cancers. Cell and gene therapies are grounded in careful research that builds on decades of scientific progress. Following key approvals of cell and gene therapies by health authorities, new treatments are being tested in clinical trials around the world.

About Novartis in Canada

Novartis Pharmaceuticals Canada Inc., a leader in the healthcare field, is committed to the discovery, development and marketing of innovative products to improve the well-being of all Canadians. Over the last 5 years, our average annual research and development investment in Canada was $47 million. Located in Dorval, Quebec, Novartis Pharmaceuticals Canada Inc. employs approximately 1,000 people in Canada and is an affiliate of Novartis AG, which provides innovative healthcare solutions that address the evolving needs of patients and societies. The company prides itself on its commitment to diversity and to nurturing an inclusive and inspiring environment. Novartis is recognized as a Great Place to Work®, ranked among the Top 50 Best Workplaces™ in the country and is proudly named on the 2021 Best Workplaces™ for Women in Canada and Best Workplace™ for Mental Wellness lists. For further information, please consult https://www.novartis.com/ca-en/.

About Novartis

Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach more than 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 108,000 people of more than 140 nationalities work at Novartis around the world. Find out more at https://www.novartis.com/.

Novartis Media Relations
Daphne Weatherby
Novartis Pharmaceuticals Communications
+ 1 514 633 7873
E-mail: camlph.communications@novartis.com

References

1. Novartis Pharmaceuticals Canada Inc. Luxturna® (voretigene neparvovec) Product Monograph. April 20, 2022
2. Patel U BM, de Léséleuc L, et al. Voretigene Neparvovec: An Emerging Gene Therapy for the Treatment of Inherited Blindness. CADTH Issues in Emerging Health Technologies Ottawa (ON): Canadian Agency for Drugs and Technologies in Health; 2016. 2018 (169)
3. Novartis. Data on file. 2018.
4. Astuti GD et al. Comprehensive genotyping reveals RPE65 as the most frequently mutated gene in Leber congenital amaurosis in Denmark. European Journal of Human Genetics 2016; 24: 1071–79.
5. Morimura H et al. Mutations in the RPE65 gene in patients with autosomal recessive retinitis pigmentosa

or Leber congenital amaurosis. Proceedings of the National Academy of Sciences of the USA. 1998; 95: 3088–93.
