Backgrounder

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Meet the team behind Canada's first gene therapy for eyes



Alberta Health Services is the provincial health authority responsible for planning and delivering health supports and services for more than four million adults and children living in Alberta. Its mission is to provide a patient-focused, quality health system that is accessible and sustainable for all Albertans. www.albertahealthservices.ca

GOVERNMENT OF ALBERTA FACULTY OF MEDICINE & DENTISTRY The Faculty of Medicine & Dentistry at the University of Alberta is a leader in educating and training exceptional practitioners and researchers of the highest international standards. The faculty's mission is to advance health through excellence in teaching, research and patient care. It is home to one of the top-100 ranked medical schools in the world. www.med.ualberta.ca



Alberta Innovates – Health Solutions supports research and innovation activities to improve the health and well-being of Albertans and create health-related social and economic benefits for Albertans. AIHS provides leadership for Alberta's health research and innovation enterprise by directing, co-ordinating, reviewing, funding and supporting research and innovation. www.aihealthsolutions.ca.



Alberta's research and innovation system plays a vital role in Alberta's future. **Alberta Innovation and Advanced Education** provides leadership and strategic investments in research and science and technology initiatives. <u>http://eae.alberta.ca</u>



The Canadian Institutes of Health Research (CIHR) is the Government of Canada's health research investment agency. CIHR's mission is to create new scientific knowledge and to enable its translation into improved health, more effective health services and products, and a strengthened health care system for Canadians. Composed of 13 institutes, CIHR provides leadership and support to more than 13,700 health researchers and trainees across Canada. <u>www.cihr-irsc.gc.ca</u>

The Canada Foundation for Innovation gives researchers the tools they need to think big and innovate. By investing in state-of-the-art facilities and equipment in Canada's universities, colleges, research hospitals and nonprofit research institutions, the CFI is helping to attract and retain the world's top talent, to train the next generation of researchers, to support private-sector innovation and to create high-quality jobs that strengthen the economy and improve the quality of life for all Canadians. <u>www.innovation.ca</u>

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The goal of the **Choroideremia Research Foundation Canada** (CRFC) is to facilitate the development of and access to choroideremia treatments. It is prepared to make strategic investments worldwide to achieve this goal. CRFC is a member of a collaborative group of choroideremia organizations active in Canada, the U.S., France, Spain, Germany and the U.K. Our collective efforts have contributed to a wave of therapy development in the current decade and brought hope to those with choroideremia and related genetic eye diseases. www.choroideremia.ca

"We are very excited and pleased that the clinical phase of the University of Alberta trial has begun," says Robert Hillier, CRFC president. "Choroideremia patients worldwide are anxious to access this potentially sight-saving treatment. Progress towards making such treatment universally accessible can not happen fast enough."



The Foundation Fighting Blindness (FFB) is leading the fight against blindness by advancing retinal disease research, education and public awareness. The foundation offers hope to over one million Canadians living with vision loss by funding research to restore sight. The foundation is also known by its signature events: Comic Vision, Cycle for Sight, Ride for Sight, and Vision Quest. Visit <u>www.ffb.ca</u> or call 1.800.461.3331 to learn more.

The FFB began funding Dr. Ian MacDonald's choroideremia research in 1985. "This new step forward is extremely meaningful," says Sharon Colle, FFB President and CEO. "We believed in the translational potential of Dr. MacDonald's work since the very beginning and are extremely proud to be supporting the first clinical test of his research. This trial gives us hope and proof that laboratory science has the potential to transform into clinical research."



NightstaRx Ltd. — Nightstar is a private biopharmaceutical company focused on the development of therapies for retinal dystrophies. The company's lead program is a retinal gene therapy for choroideremia, a rare inherited cause of blindness that affects around one in 50,000 people. Gene therapy treatment has the potential to deliver a one-time cure for choroideremia and a range of other retinal dystrophies. The company's lead investor, Syncona LLP, an independent subsidiary of the Wellcome Trust, is an evergreen investment company, taking an active role in identifying, supporting and developing technologies with the potential to significantly impact the health care market of the future. <u>www.nightstarx.com</u>

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For media inquiries, contact:

Gregory Kennedy AHS Communications 780-342-8172