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Partnerships make first Fabry gene therapy trial a reality

CALGARY — Many clinicians and organizations helped make possible the first Fabry gene therapy trial in the world.

Dr. Jeffrey Medin is the overall principal investigator for the project. He began the background studies more than 20 years ago that eventually led to this trial. He worked with Dr. Roscoe Brady at the National Institutes of Health in Bethesda, Md. (Dr. Brady discovered the enzymatic defect in Fabry disease.) Groundwork for this present trial started in 2012 when Dr. Medin was a professor at the University of Toronto and affiliated with the University Health Network. In January 2016, Dr. Medin started a new position as an endowed Professor in the Departments of Pediatrics and Biochemistry at the Medical College of Wisconsin in Milwaukee.

Dr. Aneal Khan is the site lead for the Calgary arm of the project. He is an Alberta Health Services (AHS) medical geneticist and Associate Professor of Medical Genetics and Pediatrics at the Cumming School of Medicine, University of Calgary. He is also a member of the Alberta Children's Hospital Research Institute, which is a multi-disciplinary partnership of the University of Calgary, AHS and the Alberta Children's Hospital Foundation.

The trial is a true pan-Canadian effort. Key components of the trial were completed in Hamilton, Ont., under the leadership of Dr. Ronan Foley; in Toronto, under the leadership of Dr. Armand Keating; in London, Ont., under the leadership of Dr. Tony Rupar; in Sherbrooke, Que., under the leadership of Dr. Christiane Auray-Blais; and in Halifax, N.S., under the leadership of Dr. Michael West.

In Calgary, the project was supported at the Foothills Medical Centre by:

- Dr. Ahsan Chaudhry, oncology, Departments of Oncology and Medicine, Cumming School of Medicine, University of Calgary.
- Dr. Andrew Daly, hematology and internal medicine, Departments of Oncology and Medicine, Cumming School of Medicine, University of Calgary.
- Dr. Peter Duggan, hematology, Departments of Oncology and Medicine, Cumming School of Medicine, University of Calgary.
- Dr. John Klassen, internal medicine, Departments of Pathology & Laboratory Medicine, member of the Libin Cardiovascular Institute, Cumming School of Medicine, University of Calgary.
- Dr. Nicole Prokopishyn, Director, Cellular Therapy Lab, Calgary Laboratory Services, , Department of Pathology & Laboratory Medicine, Cumming School of Medicine, University of Calgary.

Collaborating organizations:

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.

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Calgary Laboratory Services (CLS) is a medical diagnostic laboratory that offers a full range of laboratory services to Calgary and parts of southern Alberta. Every year, more than 4,500 physicians access CLS services to help diagnose, treat and monitor their patients' health. Through community collection sites, mobile services and hospital laboratories, CLS serves more than 1.3 million people each year and performs approximately 29 million tests annually. CLS invests in the future of healthcare by providing training programs for tomorrow's laboratory professionals, and by taking an active role in medical research.

The University of Calgary's Cumming School of Medicine is a leader in health research, with an international reputation for excellence and innovation in health care research and education. On June 17, 2014, the University of Calgary Faculty Of Medicine was formally named the Cumming School of Medicine in recognition of Geoffrey Cumming's generous gift to the university.

The University of Calgary is making tremendous progress on its journey to become one of Canada's top five research universities, where research and innovative teaching go hand in hand, and where we fully engage the communities we both serve and lead. This strategy is called Eyes High, inspired by the university's Gaelic motto, which translates as 'I will lift up my eyes.'

The project is being funded by the Canadian Institutes of Health Research and the Kidney Foundation of Canada.

Other sites where the trial is currently recruiting include the Queen Elizabeth II Health Sciences Centre in Halifax and the University Health Network in Toronto.

- 30 -

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