Hereditary Amyloidosis Canada and Regroupement québécois des maladies orphelines Announce Akcea Canada Young Investigator Grant Program

-First Canadian Program to Stimulate Research into Transthyretin Amyloidosis -

Toronto, ON and Montreal, QC , December 19, 2019 -- Hereditary Amyloidosis Canada (HAC) and Regroupement québécois des maladies orphelines (RQMO) announced today that they have partnered to launch *Unfolding Transthyretin Amyloidosis: Akcea Canada Young Investigator Grants*, the first Canadian research grant program for transthyretin (TTR) amyloidosis. Funded by Akcea Therapeutics Canada, the three-year program is designed to stimulate early career researchers and clinician-scientists to study this complex, debilitating and life-limiting rare disease.

"Today's announcement is very good news for people living with TTR amyloidosis in Canada and worldwide, as increasing the level of support for research in this disease area is critical," said Anne Marie Carr, Founder and President of Hereditary Amyloidosis Canada. "As an organization, we are committed to serving the needs of our community in many different ways, including support for research which seeks to continue unfolding TTR amyloidosis."

Transthyretin amyloidosis occurs when there is a build-up of abnormal or "misfolded" proteins – known as amyloid fibrils – in the body's tissues and/or organs. The disease can manifest itself in many ways and with a broad range of symptoms, depending on where the amyloid protein is building up in the body. Most commonly it affects the peripheral nerves, heart, intestinal tract, eyes, kidneys, central nervous system, thyroid and bone marrow. The progressive accumulation of amyloid deposits in these tissues and organs leads to sensory, motor and autonomic dysfunction often having debilitating effects on multiple aspects of a patient's life.

"For the first time, new therapies in this rare disease are improving the outlook for patients and their families," said Gail Ouellette, a geneticist, registered genetic counsellor and Executive Director of Regroupement québécois des maladies orphelines (RQMO). "This new grant program will continue to build on this momentum by stimulating research into improving the patient experience and health outcomes in TTR amyloidosis."

A Scientific Advisory Committee with clinical experts from across the country have been recruited to provide advice and conduct the peer review process. The committee includes:

- Dr. Nowell Fine (Cardiologist, Calgary, AB)
- Dr. Vera Bril (Neurologist, Toronto, ON)
- Dr. Diego Delgado (Cardiologist, Toronto, ON)
- Dr. Angela Genge (Neurologist, Montreal, QC)
- Dr. Francois Tournoux (Cardiologist, Montreal, QC)

“As a company we are focused on addressing the unmet needs and complex medical challenges of patients with important, but less understood, serious and rare disorders such as TTR amyloidosis,” said Jared Rhines, Senior Vice President and Head, Americas, Akcea Therapeutics. “There is currently a dearth of knowledge in TTR amyloidosis and this significant investment in the Akcea Canada Young Investigator Grant Program will help to stimulate activity, build capacity and ignite interest within the research community, with the goal of improving patients’ lives.”

The program details, including eligibility and how to apply, will be announced in winter of February 2020. The first grants will be awarded in the summer of 2020.

About Hereditary Amyloidosis Canada (HAC)
Hereditary Amyloidosis Canada (HAC) is a patient advocacy organization founded by people who are personally impacted by this rare disease. The mission is to be a source of support and information, and to advocate for access to health services and treatments and other issues relevant to Canadian patients. For more information, visit www.madhattr.ca.

About Regroupement québécois des maladies orphelines (RQMO)
The RQMO (Regroupement québécois des maladies orphelines/Quebec Coalition of Orphan Diseases) is a coalition of nearly 30 rare disease patient organizations. It manages the iRare Centre, a free, bilingual information and support centre for individuals with a rare disease, their caregivers and health professionals. The RQMO transmits official, trustworthy and up-to-date information on any rare disease, helps people access financial and social services and genetic counselling, and provides information about clinical research projects. For more information, visit www.rqmo.org.

About Akcea Therapeutics Canada
Akcea Therapeutics Canada, based in Ottawa, Ontario, is the Canadian subsidiary of Akcea Therapeutics. Akcea, a majority-owned affiliate of Ionis Pharmaceuticals, is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious and rare diseases. Akcea Therapeutics Canada is a member of Innovative Medicines Canada, the industry association representing Canada’s research-based pharmaceutical companies.

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