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# Newsletter

myriad canada
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## At the Forefront of Rare Disease Research: Transforming Outcomes for Patients Worldwide

**"There are thousands of rare diseases. In the end, they touch 10 percent of the population."**

Gilles Atlan, Vice President, NF2 BioSolutions and father of an NF2 patient.

Thanks to the generosity of Canadian donors, Myriad Canada is supporting pioneering research into rare and complex diseases that often fail to attract the interest and resources they require. These diverse projects are united by a commitment to improving patient outcomes and quality of life, bringing new hope to those affected.

# Advancing Novel Treatments for Neurofibromatosis

Neurofibromatosis type 1 (NF1) and NF2-related Schwannomatosis (NF2-SWN) are rare genetic disorders that lead to non-cancerous tumor growth in the nervous system as well as increase the risk of cancer (for NF1). NF1 is often marked by neurofibromas (benign tumors growing on nerves or skin), bone deformities, and learning disabilities. NF2-SWN is rarer and causes tumors on nerves that result in deafness, blindness, facial palsy, pain, and mobility issues. Treatments for all forms of NF include delicate, high-risk surgeries and chemotherapy with cancer drugs, which often are not suitable for prolonged use.

@ Children's Tumor Foundation

**The Children's Tumor Foundation (CTF) Research Program is dedicated to discovering innovative treatments for NF1, and NF2- and non NF2-related schwannomatosis. Myriad Canada supports three critical research projects at CTF led by talented young researchers for a total of $215,000 CAD:**

**→ In Texas**

Tyron Porter is exploring a novel drug delivery using ultrasound-triggered fat bubbles. This approach may minimize long-term toxicity, making it safer for NF2-SWN as well as cancer patients.

**→ In Michigan**

Jamie Grit is developing a topical treatment to better manage cutaneous fibromas, a disfiguring and uncomfortable skin condition found in NF1 patients.

**→ In Sydney**

Geoff McCowage is working on a liquid biopsy test using a biomarker for early sarcoma diagnosis in NF1 patients, with the aim of reducing anxiety about developing cancer and improving outcomes.

CTF established the NF Data Portal (nfdataportal.org), a repository to connect researchers and accelerate breakthroughs. The foundation has created a collaborative ecosystem to rapidly deliver effective drugs to those in need. "Everything that blocks that process, are things we should address with funding and with talents," says CEO Annette Bakker. "For patients, it's worth the fight."

# Exploring Trailblazing Gene Therapy for NF2

Until now, NF2-SWN treatments have focused on prolonging function and life, but none have halted tumor growth entirely. The average life span of NF2 patients is just 36 years. In partnership with patient-led foundation NF2 BioSolutions, Myriad Canada is supporting promising gene therapy research aimed at a cure. "We brought a new vision of really being able to take care of the disease in the long term," says Vice President Gilles Atlan, whose 17-year-old daughter suffers from NF2.

Canadian funds supported a pre-clinical NF2 gene therapy study at the renowned Nationwide Children's Hospital in Ohio ($115,000 USD). Leading researchers have achieved promising initial results. At the same time, NF2 BioSolutions is connecting the research and patient communities to pursue several other novel approaches. "For patients and their families, the fact that we can do something positive makes us able to live with that stress," says Atlan.

Gilles Atlan and his family

# Collaborative Breakthroughs in Liposarcoma Treatment

Liposarcoma is a rare, life-threatening cancer that begins in fat cells and forms malignant tumors in areas like the abdomen, thigh, and behind the knee. No therapy has yet been shown to prevent tumor recurrence after initial removal.

The Rossy Foundation Fund at Myriad Canada is funding the David Liposarcoma Research Initiative to support innovative liposarcoma research at the Dana-Farber Cancer Institute in Boston for $14M USD. Based on a pioneering collaborative model, the initiative aims to advance scientific projects that improve outcomes for patients worldwide. "I've been amazed by the calibre of scientists and clinicians who have been involved in this effort," says Scientific Director Dr. Nicole Solimini.

The second phase of the project will build on recent discoveries, supporting 17 innovative research projects. One of these breakthroughs is a promising therapeutic strategy using targeted protein degraders, for which Myriad Canada is providing additional funding.

Thanks to a Canadian donor, Myriad Canada is also funding research at the Dana-Farber Cancer Institute on spindle cell rhabdomyosarcoma, an ultra rare subtype of rhabdomyosarcoma for $500,000 USD. By studying its genetic traits in adults, this project may offer new hope for treating this highly aggressive tumorous cancer.

Another crucial project at Dana-Farber addresses Lynch syndrome (LS), an often-undiagnosed hereditary condition that greatly increases cancer risk. With Myriad Canada's support, a new project will investigate cancer risk factors in LS patients, connecting these to clinical outcomes and enabling proactive care ($300,000 USD).

@ Bryce Vickmark

Dr. George D. Demetri, director of the Sarcoma Center at the Dana-Farber Cancer Institute

# Empowering MS Patients through Exercise Therapy

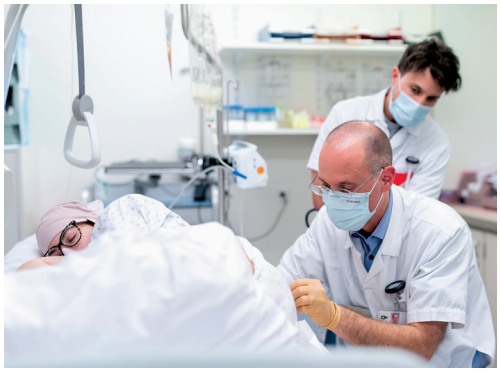
While it may not be a rare disease, multiple sclerosis (MS) is certainly very complex and debilitating. This neurological disease causes inflammation and neuron degeneration, leading to physical and cognitive disabilities. Traditional treatments offer limited success in addressing cognitive challenges, making alternative strategies like exercise training essential. "The most common cognitive problem in MS is slowed information processing speed, which is linked to depression, unemployment, and reduced ability to engage in daily life," explains Assistant Director of the Kessler Foundation's Center for Neuropsychology and Neuroscience Research, Dr. Brian M. Sandroff.

Thanks to a donation from a donor-advised fund at the TD Private Giving Foundation, Sandroff's team is studying how different types of walking exercise might improve cognition in MS patients, measuring brain changes via MRI ($70,000 USD). The goal is to develop exercise guidelines MS patients can use independently to improve cognitive function and daily living.

# Redefining What is Possible for Glioblastoma

Glioblastoma is the most aggressive and common type of brain cancer, with a poor prognosis despite rising cases. The Lundin Cancer Fund at Myriad Canada was established in memory of Lukas Lundin, who passed away from glioblastoma in 2022. To raise awareness and research funding for brain cancers, his four sons partnered with Myriad to expand the Fund across Canada, the U.S., and Europe.

Myriad Canada currently supports two vital projects at Lausanne University Hospital (CHUV) in Switzerland aimed at transforming glioblastoma care:



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Consultation with the Professor Hottinger

**One is working to develop and support 3 innovative clinical projects ($430,000 CAD):**

→ **Prof. Johanna Joyce's lab** is studying blood vessel formation in brain tumors and how it can be targeted for novel treatments, with promising findings now being tested in clinical trials.

→ **Prof. Denis Migliorini** is developing a rapid method to generate CAR T cells that enables tailoring the treatment directly based on the immune characteristics of each patient's glioblastoma, enhancing the immune response against cancer.

→ **Dr. Giulia Cossu's project** explores using hypnosis to reduce stress and improve comfort for patients undergoing awake surgery to remove tumors near critical brain areas.

Another project with the CHUV will start in April 2025 and focus on developing novel treatment combination and improving treatment options for patients suffering from glioblastoma ($1.5M CAD). To that objective, a multicentric clinical trial with 53 patients will be conducted.

"These projects are at the forefront of research," says Prof. Andreas Hottinger, who leads the Centre. "Together with other labs, we're driving science forward." Canadian donor support has helped build a community around Lundin's motto: "Nothing is impossible."

**The research supported is advancing the understanding of rare diseases, creating new possibilities, and improving lives. As Michèle Joanisse, Director of CHUV Foundation affirms, "Rare diseases are dependent on philanthropy. The collaboration with Myriad Canada has genuinely boosted our ability to explore new paths that were not possible before." Myriad Canada remains committed to transforming healthcare for rare disease communities worldwide, ensuring every patient receives the care they deserve.**

MYRIAD CANADA

Myriad Canada understands that philanthropy is about personal stories, and sometimes these stories take us beyond our borders, which is why we manage charitable projects worldwide.

We connect Canadians to their favourite charitable causes - anywhere in the world - by crafting personalized solutions for one-time gifts or recurring donations through donor-advised funds.

We provide the due diligence and oversight that charitable projects require so that donors can receive charitable tax receipts. We are agile but rigorous. Our focus is on direct outcomes for impactful projects and on minimizing management fees.

As a member of the Myriad Alliance for Borderless Giving, and through 44 years of relationships built through the Myriad Family, we have access to a strong international network and extensive practical expertise and knowledge.

The Myriad Alliance for Borderless Giving is a global network of trusted member organizations that facilitate cross-border giving with independent offices in Canada; the United States; Europe; Australia; New Zealand; and China; including Taiwan and Hong Kong.

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