MUSCULAR DYSTROPHY CANADA
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Newsletter
SUMMER 2021

THE TIME IS NOW: KEEPING UP THE RESEARCH MOMENTUM

Muscular Dystrophy Canada (MDC) is committed to finding cures and improving quality of life for Canadians affected by neuromuscular disorders (NMD). Because of incredible donors and supporters, MDC is able to invest in innovative research led by exceptional clinicians and scientists. In the past five years alone, collaboration by scientists, healthcare professionals and persons affected by NMDs has supported tremendous progress in research and clinical care.

To build upon this momentum and support the neuromuscular community in Canada, we invest in research focused on **managing healthcare, understanding diagnosis and disease progression, enhancing care and discovering novel treatments and therapies.** By supporting different areas of research and a broad set of neuromuscular disorders, MDC is able to support the best research to increase knowledge, build capacity, diversify the research base and gain learnings to answer future questions and needs. Below are examples of recently funded research that donors, like you, made possible.

RESEARCH IN ACTION

UNDERSTANDING DIAGNOSIS AND DISEASE PROGRESSION

Drs. David Granville and Michael Berger from the University of British Columbia have teamed up to **study neuromuscular disorders caused by autoimmune imbalances in the immune system** (for example, Myasthenia Gravis, Chronic Inflammatory Demyelinating Polyneuropathy, Myositis). This research team is looking for molecular factors in the immune system that could help predict the severity of symptoms and identify future drug targets.

DISCOVERING NOVEL TREATMENTS AND THERAPIES

Dr. James Dowling, a Pediatric Neurologist and Senior Scientist at the Hospital for Sick Children, is **exploring gene-editing (CRISPR) as a potential treatment for hereditary myopathies.** This study has potential to be the first therapy for these disorders. The approach, if proven successful, will also provide a roadmap for developing similar approaches for other types of NMDs.

ENHANCE CARE

Dr. Leanne Ward, Pediatric Endocrinologist and Professor at the Children's Hospital of Eastern Ontario (CHEO), conducted a clinical trial that **compared two treatments for chronic illness osteoporosis in boys affected by Duchenne Muscular Dystrophy (DMD).** Boys on glucocorticoids are at risk for vertebral and long bone fractures due to osteoporosis, which can cause back pain and spine deformity. In this pilot trial, the safety and feasibility of a novel therapy in the treatment of osteoporosis (denosumab) was compared to the standard of care approach (intravenous bisphosphonate therapy, zoledronic acid) in boys with DMD.

MANAGE HEALTHCARE

Led by Dr. Hanns Lochmüller, Neuromuscular Specialist and Scientist at Children's Hospital of Eastern Ontario (CHEO), **the Neuromuscular Disease Network for Canada (NMD4C)** is an integrated network for patients, scientists, and clinicians that aims to make rapid progress towards giving all Canadian patients world-class care and access to new treatments. The NMD4C raises the standard of care for NMDs by pooling expertise to improve coordination of clinical trials, biobanks and clinical registries. In addition, NMD4C is developing and sharing top-notch educational and training opportunities for individuals affected by NMDs, clinicians, academics and scientists.

Your unwavering support is instrumental as we work with research, clinical and academic communities on the core of our mission: relentlessly searching for cures through well-funded research. The incredible momentum we are currently experiencing would not be possible without your continued dedication. Thank you!

Do you have questions? MDC's Research Hotline is a helpful resource for Canadians searching for information on neuromuscular disorders. Contact us at research@muscle.ca or 1-800-567-2873 ext 9037.

DONORS SUPPORT EXCITING NEUROMUSCULAR DISORDER RESEARCH

Thanks to incredible donors and supporters, seven new research projects will be funded in 2021 through the **MDC Neuromuscular Disorder Research Grant Program**, a dedicated Canadian source of funding for neuromuscular research. This year MDC was also able to invest, alongside Canadian Institutes of Health Research (CIHR), in an international, collaborative, $1.15 million research project through the European Joint Programme on Rare Diseases (EJP RD).

"Despite an unprecedented time, MDC staff and board of directors worked relentlessly to explore all options to offer research funds in Canada, recognizing the need to maintain momentum. Through careful consideration and planning, we were able to ensure the incredible advances currently taking place in research are accelerated."

Stacey Lintern,
CEO, Muscular Dystrophy Canada

Generous donors contribute greatly to funding exceptional researchers, clinicians and academics who will lead these projects taking place in hospitals and universities across Canada, and around the world. This continued investment in life-changing research would not be possible without the ongoing commitment of donors and supporters.

"To the families and donors, I say a huge thank you. From my first day as a researcher, getting my first grant from MDC, you were there. Thank you for sticking by the research community for many, many years. Hopefully we can pay back the trust that you put in us."

Dr. Rashmi Kothary,
Deputy Scientific Director and Senior Scientist at the Ottawa Hospital Research Institute and 2019-2020 MDC research grant recipient

MDC is continuing its support towards 12 active research studies and is investing in seven new research projects this year. Congratulations to the 2020 - 2021 research grant recipients:

DR. ALEX PARKER
A Pharmacogenetic Pipeline for Charcot-Marie-Tooth Disease

DR. COLIN CRIST
Promoting Muscle Repair by Pharmacological Inhibition of eIF2a Dephosphorylation

DR. MOHAMED CHAHINE
Human iPSC-derived Neurons as a Model of Congenital Myotonic Dystrophy Type 1

DR. NADINE WIPER-BERGERON
Improving Myoblast Transplantation Outcomes via Pharmacological Reprogramming

DR. NICOLAS DUMONT
Targeting Defective Stem Cells in a Preclinical Model of Myotonic Dystrophy Type 1

DR. RIMA AL-AWAR
Safety and Efficacy of a Possible Epigenetic Therapy for Facioscapulohumeral Muscular Dystrophy

DR. TOSHIFUMI YOKOTA
Enhancing the Efficacy of Antisense Oligonucleotide Therapy for Facioscapulohumeral Muscular Dystrophy

"MDC is committed to supporting opportunities that fulfill our commitment to invest in research that leads to information on diagnosis, treatment and health management of neuromuscular disorders; and, we're confident that the research funded this year will bring us closer to cures for all children, youth, adults and families affected by neuromuscular disorders."

Stacey Lintern,
CEO, Muscular Dystrophy Canada

Learn more about MDC's research investments by visiting muscle.ca

RESEARCH CHANGED JENNA'S DIAGNOSIS – AND LIFE

Jenna Keindel searched for answers for years. With muscle biopsies and progressive muscle weakness in her hips, shoulders and neck, doctors diagnosed 16-year-old Jenna with Limb-Girdle Muscular Dystrophy. However, questions remained unanswered and doctors weren't able to identify the exact gene that was causing the disorder.

Over the years, Jenna became part of the MDC community to learn more about her diagnosis, find support from her peers, and share information.

"One day, I came across a research article that fit with the some of the questions I had about my symptoms and diagnosis, and it prompted me to ask my doctor to order a test," shared Jenna.

The results of that test showed that Jenna had an autoimmune neuromuscular disorder that mimics the symptoms of Limb-Girdle Muscular Dystrophy. She also learned there was a treatment available.

Jenna credits research with now having a confirmed diagnosis and changing the way she speaks about her diagnosis journey.

"I used to call it a misdiagnosis, but it wasn't; the doctors diagnosed me with the information they had available at the time. It is thanks to ongoing research that additional information became available and I was re-diagnosed. Research made that possible." **Jenna Keindel**

Donors, like you, make life-changing research like this possible through their gifts to MDC.

Jenna is positive about the future and determined to share her story so that others who may be in a similar situation can get the answers they seek. *"The sooner people are tested; the sooner they get diagnosed; and, if a treatment is available, the sooner they can get access to it, the better the outcomes will be for all,"* said Jenna.

This year's Tenaquip Foundation Walk4MD, which took place on June 19, brought awareness to stories like Jenna's, and highlighted the importance of continually investing in research by spotlighting a team of six neuromuscular disorder Research Champions.

This team of Research Champions know first-hand the incredible impact funds raised through events like the Walk4MD have on advancing research and supporting the neuromuscular community and continued research advancements. You can meet our Walk4MD Research Champions by visiting **Walk4MD.ca**. While you're there, please consider making a donation to ensure critical advancements in research continue!

WE'RE HERE FOR YOU, EVEN WHEN WE HAVE TO STAY APART

Regardless of where you live in this beautiful country of ours, the pandemic has undoubtedly impacted your life in big and small ways. Many of us can't see loved ones, are restricted to our homes, and are feeling more isolated than ever.

The silver lining in all this, is that the pandemic has highlighted what's really important. While this will be slightly different for everyone, I think we can agree that health, loved ones and being connected to a larger community rank at the top of the list for all of us.

Thanks to generous donors and supporters, Muscular Dystrophy Canada (MDC) was able to quickly adapt its services and programs this past year to 'virtually' support your priorities, and address some of the challenges being faced by the neuromuscular community.

NAVIGATING HEALTH CARE AND COMPLICATED SUPPORT SYSTEMS:

MDC clients can connect directly with a Service Specialist, through the Systems Navigation Program, who will help them with all areas of their non-medical needs including: funding equipment to improve daily life, providing emotional and educational support, and ensuring access to vital resources and support systems.

NETWORK MEETINGS:

MDC Let's Connect Virtual Network meetings are offered via Zoom to provide an opportunity for individuals of all ages, located across Canada, to come together, form friendships, share learnings and support each other's journeys. Meetings are held every two weeks.

EDUCATIONAL WEBINARS:

The Let's Talk NMD webinar series brings clinicians/researchers, individuals with lived experience, past MDC grant recipients together with the Canadian neuromuscular community to share trusted information on research, disorder specific knowledge and updates regarding COVID-19 and vaccinations. Webinars are hosted via Zoom every month, we hope you will join us!

All of these support programs and services, are made possible through generous gifts from donors and fundraising efforts of our supporters. If you are in a position to do so, please consider making a donation today to ensure all Canadians affected can access the support they need.

"The MDC Let's Connect Virtual Network gives me something to do since I'm stuck at home and alone in these times. It also gives me a chance to connect and meet other people, like me, who have neuromuscular disorders which is something I've always wanted to do. It makes me feel like I have a part in all of this."

Alexandra LeBoeuf, MDC client

To donate, for more information on the programs and services noted, or to register for a meeting or webinar visit muscle.ca or call 1-800-567-2873.

YOUR CHALLENGE, YOUR WAY

High Rise Challenge participants given opportunity to choose their own challenge in 2021

On October 2, Muscular Dystrophy Canada's High Rise Challenge is back virtually, and offering participants the opportunity to choose their own "challenge". That's right, if you don't want to climb stairs, like in past years, you don't have to. You can select from one of the 11 provincial or territorial challenges, or create your own challenge.

For each "provincial and/or territorial" challenge you complete leading up to the virtual celebration on October 2, you will be awarded a badge that can be used on social media to share your accomplishments with your friends and family.

Fire Fighters, paramedics, police, military, MDC families and members of the community have been participating in the High Rise Challenge to raise funds in support of Canadians affected by neuromuscular disorders. Due to the COVID-19 pandemic, this is the second year that the event has been offered virtually. Thank you to everyone who has supported this critical fundraising event during challenging times.

For more information on the High Rise Challenge, to register or make a donation, please visit **highrisechallenge.ca**

FIRE FIGHTERS STILL NEED YOUR HELP TO FILL THE BOOT

Due to COVID-19, Fire Fighters had to cancel many of their in-person Fill the Boot events. But the needs of those affected by neuromuscular disorders cannot be cancelled. MDC still needs to provide critical programs and services to our families.

For more than 66 years, Fire Fighters have been supporting Muscular Dystrophy Canada and Canadians impacted by neuromuscular disorders in an incredible way.

To support the amazing efforts of our Fire Fighter partners and to make a donation, please visit **fillthebоot.ca**

UPCOMING EVENTS

e-CME accredited webinar
July 6, 2021 - to register email research@muscle.ca

Atlantic Network
July 20, 2021

Walk4CMT
September 18, 2021

High Rise Challenge
October 2, 2021

STRONG NONPROFITS. STRONG COMMUNITIES.
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