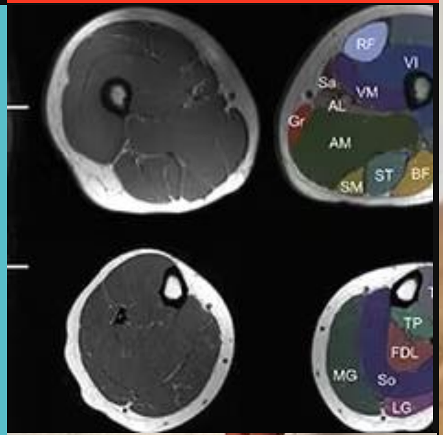
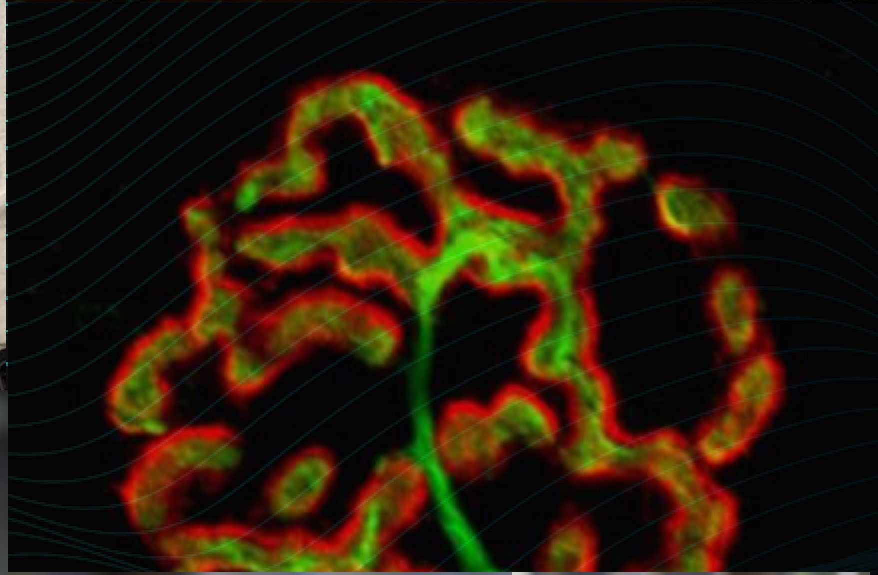
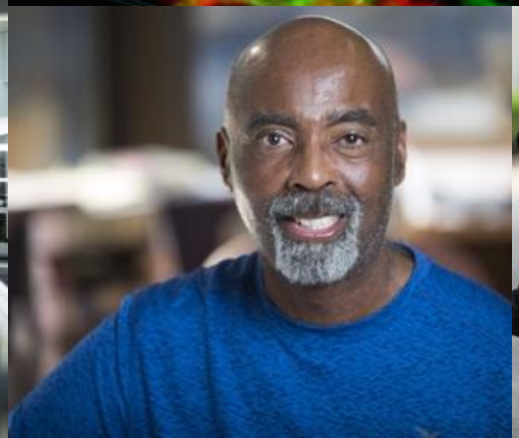
Empowering the Facioscapulohumeral muscular dystrophy (FSHD) Community:
An Overview of MDC's Projects, Programs, and Supports

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About
Muscular Dystrophy Canada

MISSION

Muscular Dystrophy Canada's mission is to enhance the lives of those affected by neuromuscular disorders by continually working to provide ongoing support and resources while relentlessly searching for a cure through well-funded research.

VISION

To find a cure for neuromuscular disorders in our lifetime.

VALUES

DETERMINATION
We are resolute. No matter what the obstacle, we will not be deterred.

COURAGE
We bravely move forward even when we are afraid or unsure where the road will lead.

PASSION
Our enthusiasm and our appetite to learn, grow and make a difference is insatiable.

CARING
We have genuine human concern for others that is never contrived and without a hint of pity or sympathy.

BREAKING DOWN BARRIERS SINCE 1954

AN Imagine Canada accredited and registered charity with Canada Revenue Agency

Ignited by Passion, Fueled by Hope

Accredited by Imagine Canada

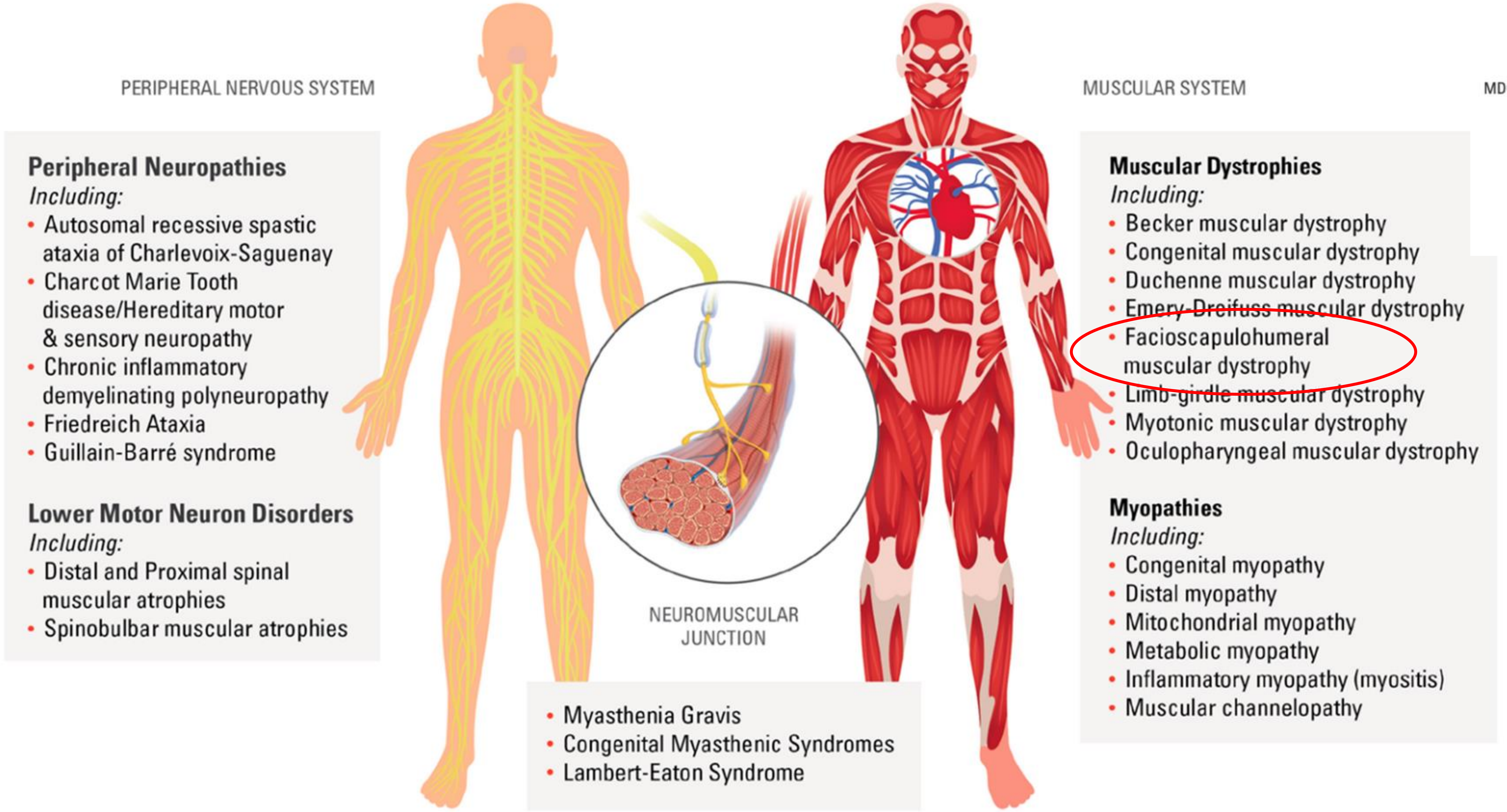
STRONG CHARITIES
ACCREDITED
IMAGINE CANADA

Supported by Diversified Revenue including Firefighters

FIRE FIGHTERS
SUPPORTING
MUSCULAR DYSTROPHY CANADA
SINCE 1954

Nothing About Us Without Us

160+ Neuromuscular Diseases Included



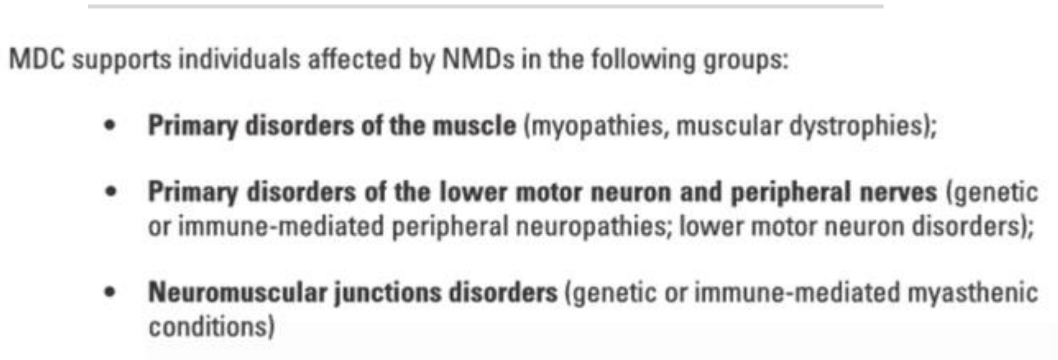
Peripheral Neuropathies
Including:
- Autosomal recessive spastic ataxia of Charlevoix-Saguenay
- Charcot Marie Tooth disease/Hereditary motor & sensory neuropathy
- Chronic inflammatory demyelinating polyneuropathy
- Friedreich Ataxia
- Guillain-Barré syndrome

Lower Motor Neuron Disorders
Including:
- Distal and Proximal spinal muscular atrophies
- Spinobulbar muscular atrophies

- Myasthenia Gravis
- Congenital Myasthenic Syndromes
- Lambert-Eaton Syndrome

Muscular Dystrophies
Including:
- Becker muscular dystrophy
- Congenital muscular dystrophy
- Duchenne muscular dystrophy
- ~~Emery-Dreifuss muscular dystrophy~~
- Facioscapulohumeral muscular dystrophy
- ~~Limb-girdle muscular dystrophy~~
- Myotonic muscular dystrophy
- Oculopharyngeal muscular dystrophy

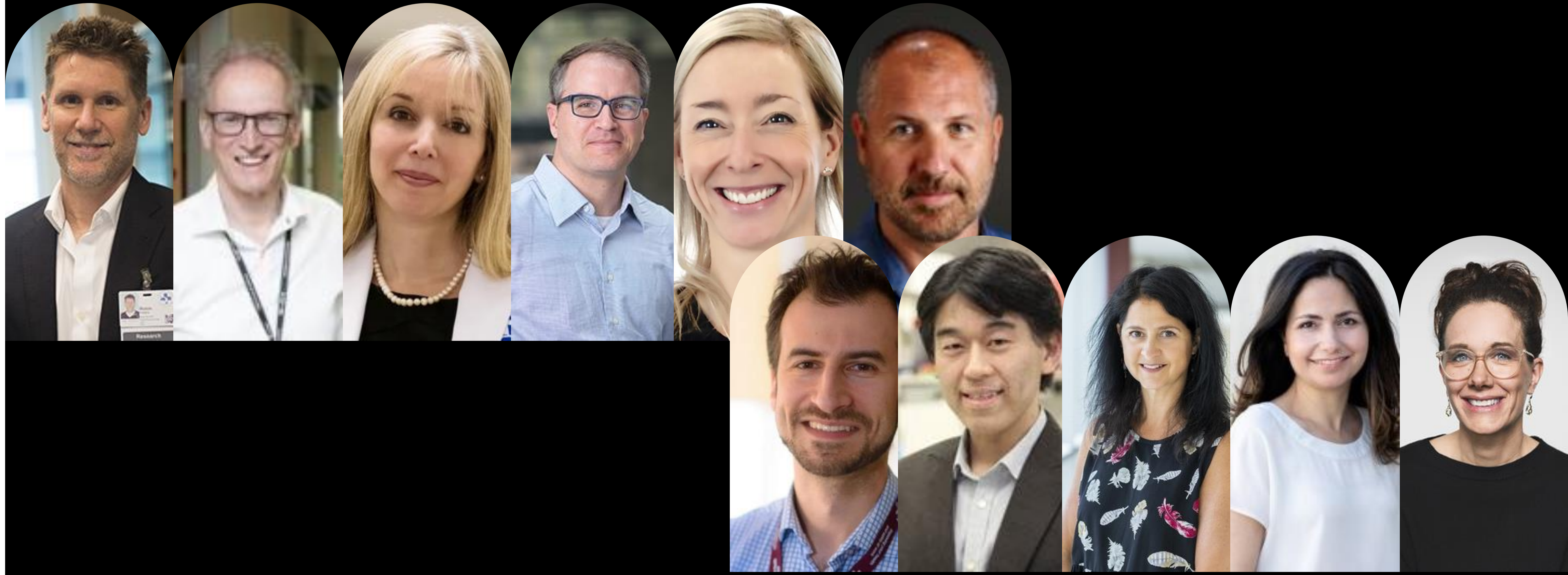
Myopathies
Including:
- Congenital myopathy
- Distal myopathy
- Mitochondrial myopathy
- Metabolic myopathy
- Inflammatory myopathy (myositis)
- Muscular channelopathy


MDC supports individuals affected by NMDs in the following groups:

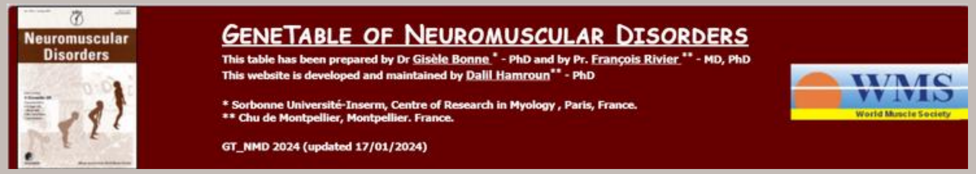
- **Primary disorders of the muscle** (myopathies, muscular dystrophies);
- **Primary disorders of the lower motor neuron and peripheral nerves** (genetic or immune-mediated peripheral neuropathies; lower motor neuron disorders);
- **Neuromuscular junctions disorders** (genetic or immune-mediated myasthenic conditions)

Comprehensive list of neuromuscular diseases informed by:

Medical Scientific Advisory Committee



Neuromuscular Gene Table



GENETABLE OF NEUROMUSCULAR DISORDERS

This table has been prepared by Dr Gisèle Bonne * - PhD and by Pr. François Rivier ** - MD, PhD
This website is developed and maintained by Dalil Hamroun** - PhD

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GT_NMD 2024 (updated 17/01/2024)

Expert Consultation

Individually Rare, Collectively Common

Where the healthcare system stops, MDC begins.

When a Canadian is diagnosed with a neuromuscular disorder, we know they will need a lot of support and services. **But the systems around us – provincial healthcare, private insurance, and government policy aren't set up to give that kind of full support. These systems are also difficult for people to navigate.**

So that's why we're here – **to fill in the healthcare gaps and help people find their way through these systems.**

Sky-high cost puts drug out of reach for adults with rare disease

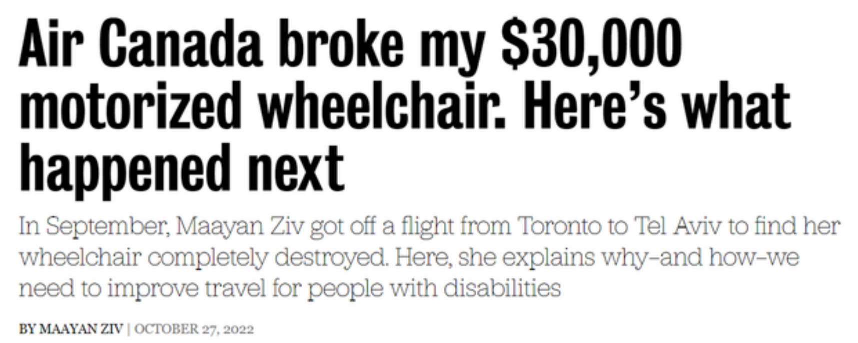
At more than $700K for 1st year, Spinraza simply isn't an option for most adults with spinal muscular atrophy

CBC News · Posted: Apr 08, 2019 4:57 PM EDT | Last Updated: April 9, 2019

Without rare-disease policy, patients in Canada face steep costs for drugs

CARLY WEEKS › HEALTH REPORTER
PUBLISHED FEBRUARY 24, 2017

This article was published more than 7 years ago. Some information may no longer be current.



Air Canada broke my $30,000 motorized wheelchair. Here's what happened next

In September, Maayan Ziv got off a flight from Toronto to Tel Aviv to find her wheelchair completely destroyed. Here, she explains why–and how–we need to improve travel for people with disabilities

BY MAAYAN ZIV | OCTOBER 27, 2022

$40K needed to retrofit Fort McMurray home for teen in wheelchair

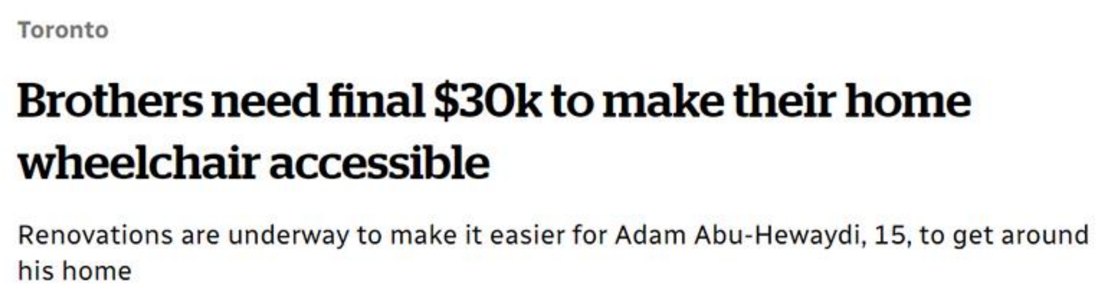
Family has turned to online fundraising as last resort to help their son

David Thurton · CBC News · Posted: Jan 03, 2018 9:00 AM EST | Last Updated: January 3, 2018

Sask. teen fighting for funding to receive 24/7 care near his post-secondary school

'I feel profoundly dehumanized and discriminated against,' 18-year-old says

Adam Hunter · CBC News · Posted: Jul 28, 2024 8:00 AM EDT | Last Updated: July 29



Toronto

Brothers need final $30k to make their home wheelchair accessible

Renovations are underway to make it easier for Adam Abu-Hewaydi, 15, to get around his home

The reality is there is a strong need for 'wrap-around' supports for people living with FSHD.

- With improved standards of care and approved treatments and therapies for some NMDs, people are living longer.
  - However, healthcare and community resources have not expanded to support individuals and their families as they **age in place.**
- Healthcare and community resources are limited and if available, have **long wait times.**
- As individuals age, supports required are **more complex and costly**.
- **This results in the neuromuscular community having the burden to deal with limited supports (in all settings i.e. community and healthcare).**
- Parents and caregivers are aging as well and often experiencing **fatigue and loss of productivity**.
- There is an impact on mental health and well being; strong desire to be **connected to peers especially due to rarity of condition.**
- Financial barriers are a significant concern, it is extremely **expensive to live with a neuromuscular disorder**.
- People with neuromuscular conditions have to pay for **medical and assistive devices** not covered by government funding, constant renovations to homes and vehicles, fee for service therapies and personal care etc.

"FSHD really is an invisible disability...my diagnosis came far too late."

"We need to do better in helping people get answers. With answers, people can start planning their lives."

Needs and Goals are Centred On:

Specialized Care and Multidisciplinary Supports

- **Need for Specialized Care:** Neuromuscular disorders require highly specialized care from multidisciplinary teams, including neurologists, physiatrists, occupational therapists, and other allied health professionals. Access to such care is limited in many regions.
- **Long-term Management**: These conditions are often progressive, meaning individuals need ongoing care throughout their lives. There is a strong need for specialized clinics and professionals who can provide continuous monitoring, personalized treatment plans, and holistic support.
- **Coordination of Care**: Coordinating between various healthcare providers, specialists, and services can be challenging. Many in the community require assistance navigating the complex healthcare system

Emotional and Adjustment Supports

- **Living with a Chronic Condition**: Neuromuscular disorders can have a profound emotional and psychological impact, both for those diagnosed and their families. The need for counseling, peer support, and mental health resources is critical to help individuals cope with anxiety, depression, and isolation.
- **Support Networks for Families**: Caregivers, often family members, experience significant stress and burnout. Emotional and practical support, including respite care and caregiver networks, can help alleviate these pressures.

Needs and Goals are Centred On:

Funding for Clinical Care

- **Access to Equipment and Services:** Many individuals require expensive assistive devices, such as mobility aids, respiratory equipment, and home modifications. Funding is needed to ensure equitable access to these essential tools, which are often not fully covered by public healthcare or insurance.

Advocacy for Policy Change

- **Access to Treatments**: Advocacy is vital to ensure that people living with neuromuscular disorders have timely and affordable access to new therapies and treatments. This includes working with policymakers to approve innovative drugs and therapies and to reduce barriers related to cost and access.
- **Inclusion in Healthcare Policy**: The neuromuscular community needs advocates who can influence healthcare policy to address gaps in coverage, ensuring that people with rare diseases are not overlooked in broader healthcare planning.
- **Disability Rights and Accessibility**: Advocacy is also required to promote inclusion and accessibility in public spaces, education, and employment for individuals with neuromuscular disorders.

Access to Information and Education

- **Need for Clear, Accessible Information**: People living with neuromuscular disorders need accurate, up-to-date information on their condition, available treatments, and research developments. Many struggle to find reliable sources of information.
- **Clinical Trials and New Treatments**: Individuals and families need clear guidance on how to access clinical trials and experimental treatments.

Social, Emotional and Practical Supports

Knowing the level of function, relief from pain and stress, and ability to participate in daily life

Capability

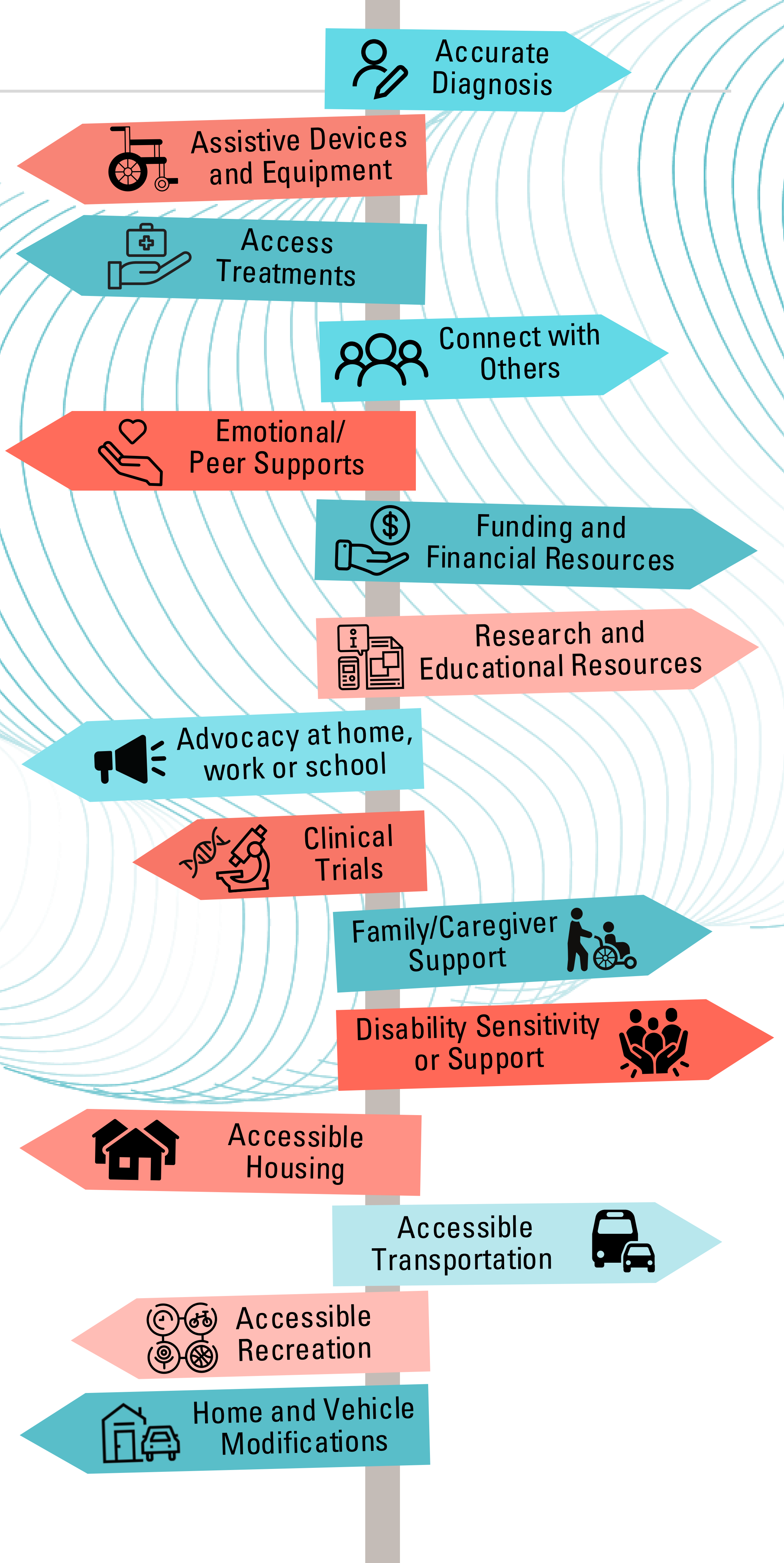
Functional status

Comfort

Relief from physical and emotional pain

Care

Can continue their life during care by considering elements like how many hours per week the patient spends scheduling and traveling to appointments, or how many hours are devoted to understanding and paying medical bills



Accurate Diagnosis

Assistive Devices and Equipment

Access Treatments

Connect with Others

Emotional/ Peer Supports

Funding and Financial Resources

Research and Educational Resources

Advocacy at home, work or school

Clinical Trials

Family/Caregiver Support

Disability Sensitivity or Support

Accessible Housing

Accessible Transportation

Accessible Recreation

Home and Vehicle Modifications

FSHD-Focused Activities

BIND STUDY

What's the full cost of living with a neuromuscular disorder in Canada?

#LetsTalkNMD
WEBINAR SERIES

FSHD 101 with Dr.Toshi Yokota

Friday, June 18, 2021

WATCH NOW

Ignited by passion, fueled by hope.

Individually rare, collectively common.
Neuromuscular Conference

Exercise for Neuromuscular Disorders, A Focus on FSHD

Exercices pour personnes atteintes de maladies neuromusculaires, accent particulier pour les personnes atteintes de FSHD

Ignited by passion, fueled by hope.

What activities are specifically related to FSHD in 2025?

For Canadians, By Canadians

Peer Support:

1. Here2Hear Peer Support Program
2. Caregiver and Family Retreats

Generating Evidence:

1. FSHD Journey Mapping Project
2. FSHD Cost of Illness in Canada

Raising Clinical Capacity:

1. NMD4C
2. FSHD Masterclass

Influencing Policy:

1. Access to Reimbursement for Treatments
2. Early, timely and accurate diagnosis

Raising Awareness and Knowledge Exchange:

1. Hope on the Horizon: FSHD Treatment, Trials, Therapies Event
2. Mini-Regional Conferences on FSHD

Together we can ensure people with FSHD can receive comprehensive support

As the needs of the neuromuscular community grow, so does the demand for resources and support services.

Your partnership with MDC is crucial in helping us generate the revenue needed to maintain and expand programs that support the growing and evolving needs of people with neuromuscular disorders and their families.

Thank You & Stay Connected

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