Canadian Neuromuscular Disease Registry (CNDR)

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CLINICAL NEURO SCIENCES
CALGARY CANADA

HOTCHKISS BRAIN INSTITUTE

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Canadian Neuromuscular Disease Registry

Canadian Neuromuscular Disease Registry (CNDR)

CNDR ORG

Canadian Neuromuscular Disease Registry

A registry is a collection of standardized information about a group of individuals, such as those living with the same disease, that is used for a variety of specific purposes.

The CNDR facilitates research that benefits patients, families, and caregivers, and promotes the development of effective therapies for neuromuscular diseases.

The CNDR: Who We Are

A Multi-Centre, National Collaborative Program

UNIVERSITY OF CALGARY

THE UNIVERSITY OF BRITISH COLUMBIA

University of Manitoba

UNIVERSITY OF ALBERTA

UNIVERSITY OF SASKATCHEWAN

UNIVERSITY OF TORONTO

Queen's UNIVERSITY

Western UNIVERSITY · CANADA

McGill UNIVERSITY

McMaster University

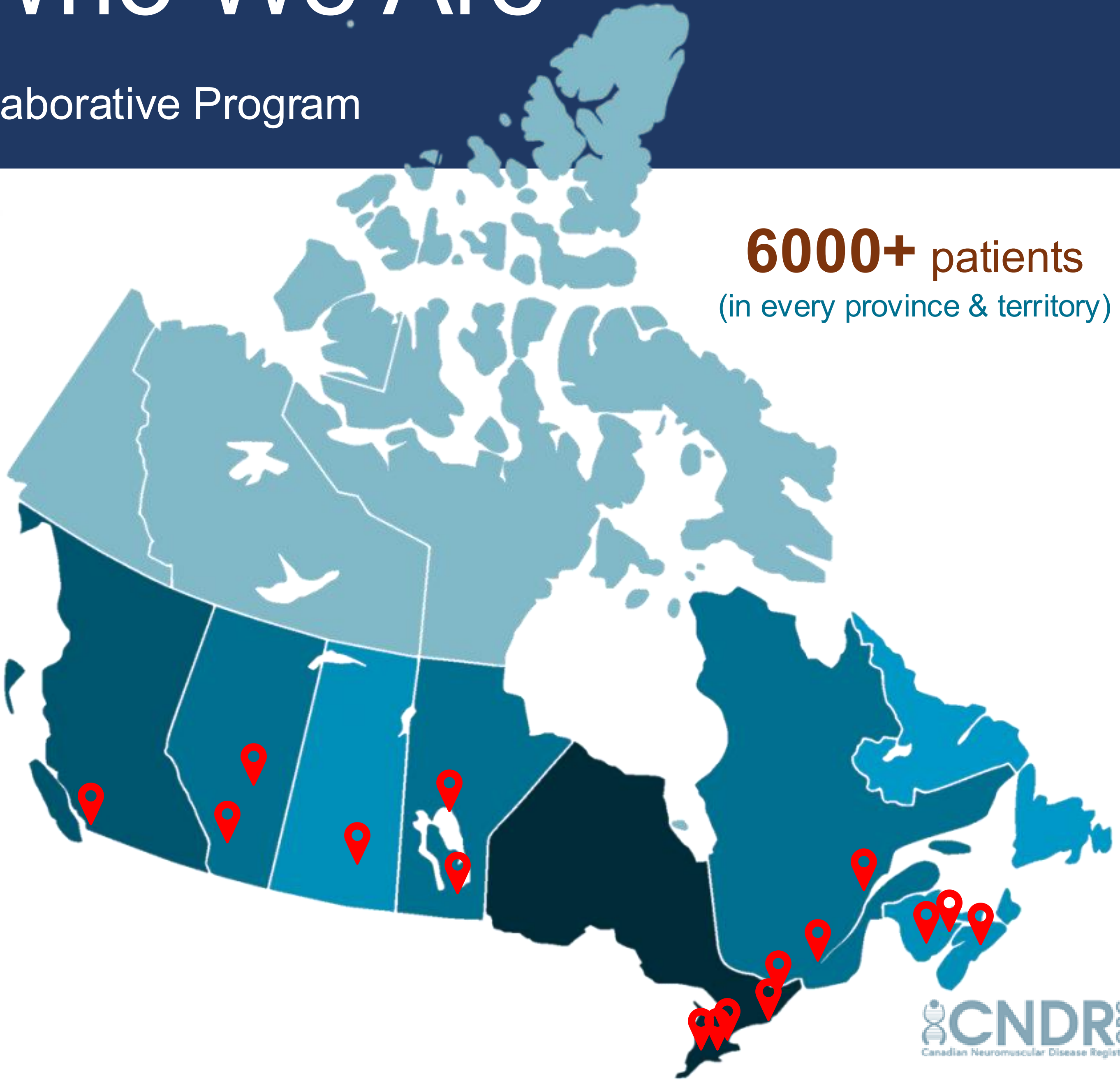
UNIVERSITÉ LAVAL

UNIVERSITÉ DE SHERBROOKE

uOttawa

Université de Montréal

DALHOUSIE UNIVERSITY



6000+ patients
(in every province & territory)

136+ investigators

52 clinics

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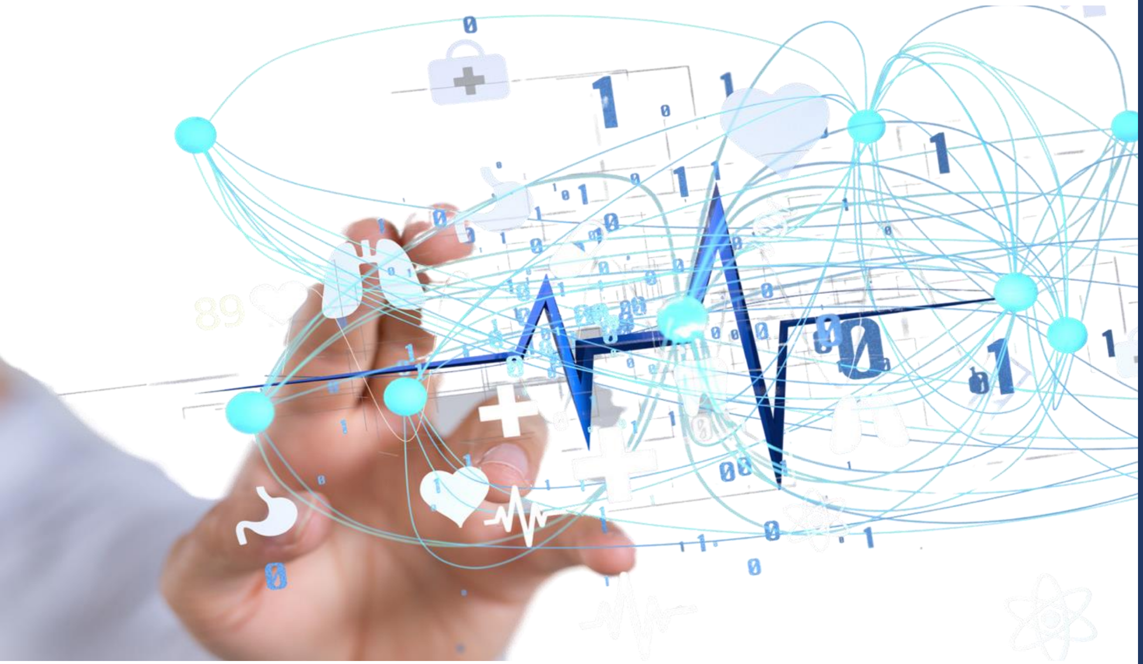
OUR GOAL:

CONFRONTING CHALLENGES FOR RARE DISEASES

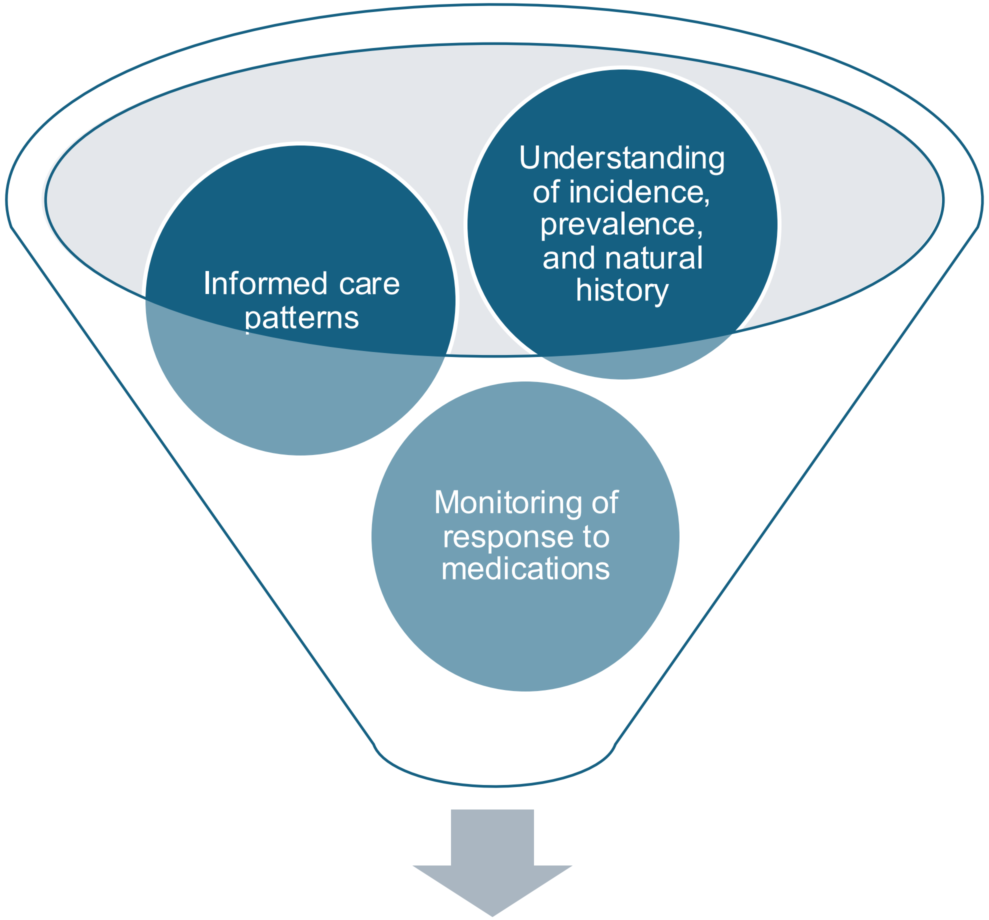


RARE DISEASE CHALLENGES:

- Limited understanding of population demographics
- Varying quality and consistency of care across Canada
- Lengthy time to diagnosis
- Quality of life data is limited
- Patient voice and advocacy
- Access to treatment options

CNDR Impact

When data is used by the community:

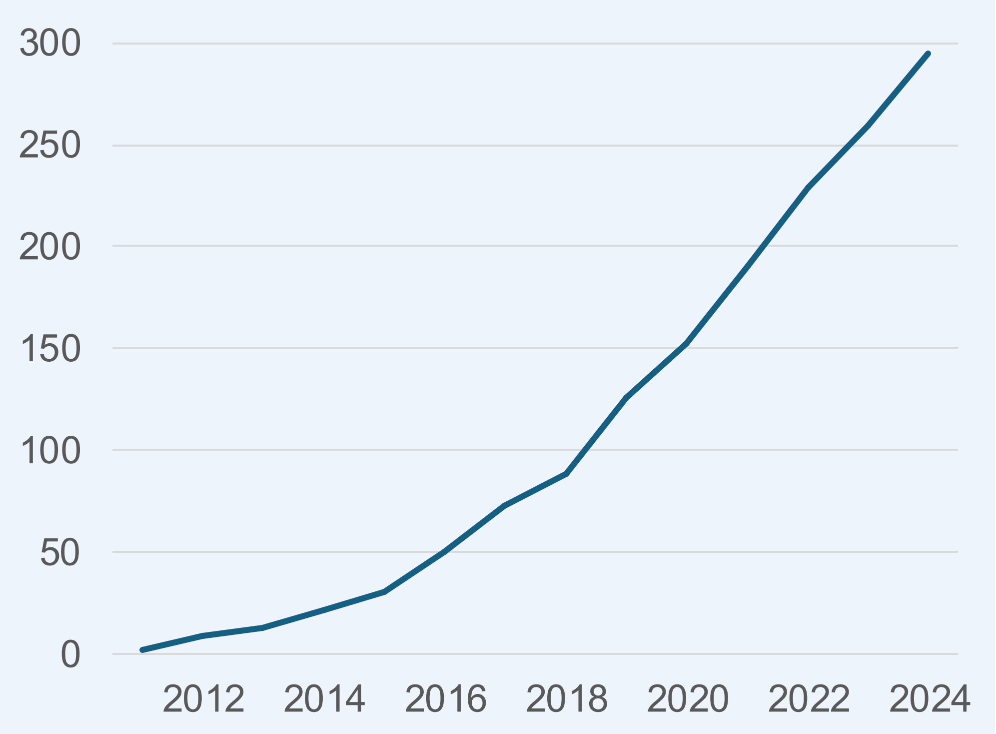


Understanding of incidence, prevalence, and natural history

Informed care patterns

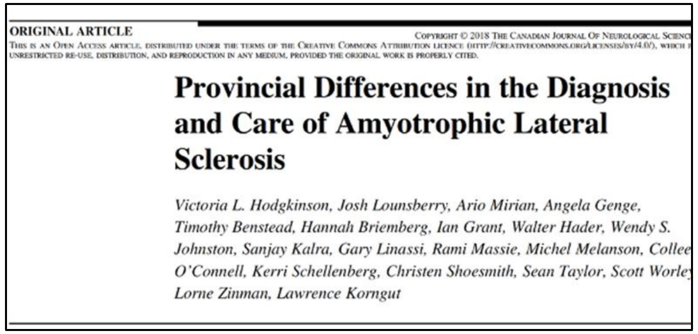
Monitoring of response to medications

Improved outcomes for people living with FSHD



300+ data usages so far, for research studies, feasibility assessments, international projects, research notifications, and more!

CNDR Impact – Outcomes


ORIGINAL ARTICLE

Provincial Differences in the Diagnosis and Care of Amyotrophic Lateral Sclerosis

Victoria L. Hodgkinson, Josh Lounsberry, Ario Mirian, Angela Genge, Timothy Benstead, Hannah Briemberg, Ian Grant, Walter Hader, Wendy S. Johnston, Sanjay Kalra, Gary Linassi, Rami Massie, Michel Melanson, Collee O'Connell, Kerri Schellenberg, Christen Shoesmith, Sean Taylor, Scott Worle Lorne Zinman, Lawrence Korngut

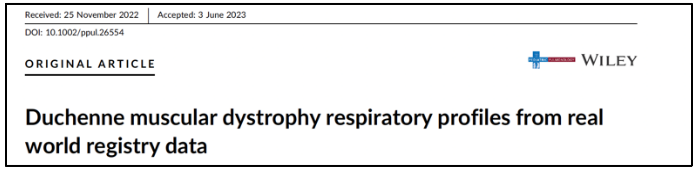

The Canadian Journal of Neurological Sciences (2024), 1–12
doi:10.1017/cjn.2024.1

Canadian Journal of Neurological Sciences
Journal Canadien des Sciences Neurologiques

Original Article

A Study on the Incidence and Prevalence of 5q Spinal Muscular Atrophy in Canada Using Multiple Data Sources

Tiffany R. Price[1], Victoria Hodgkinson[2], Grace Westbury[2], Lawrence Korngut[2], Micheil A. Innes[3], Christian R. Marshall[4,5], Tanya N. Nelson[6,7], Lijia Huang[8], Jillian Parboosingh[9] and Jean K. Mah[1]


Received: 25 November 2022 | Accepted: 3 June 2023
DOI: 10.1002/ppul.26554

ORIGINAL ARTICLE

WILEY

Duchenne muscular dystrophy respiratory profiles from real world registry data

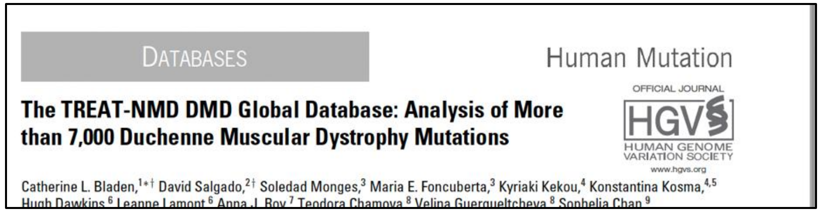

The Canadian Journal of Neurological Sciences (2025), **52**, 119–128
doi:10.1017/cjn.2024.49

Canadian Journal of Neurological Sciences
Journal Canadien des Sciences Neurologiques

Original Article

A Real-World Study of Nusinersen Effects in Adults with Spinal Muscular Atrophy Type 2 and 3

Isabelle Côté[1], Victoria Hodgkinson[2], Marianne Nury[3], Louis Bastenier-Boutin[3] and Xavier Rodrigue[3,4]

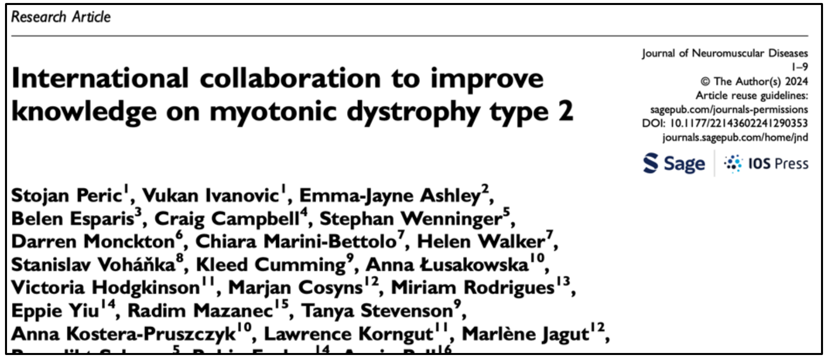

DATABASES

Human Mutation

OFFICIAL JOURNAL
HGVS
HUMAN GENOME VARIATION SOCIETY
www.hgvs.org

The TREAT-NMD DMD Global Database: Analysis of More than 7,000 Duchenne Muscular Dystrophy Mutations

Catherine L. Bladen,[1*†] David Salgado,[2†] Soledad Monges,[3] Maria E. Foncuberta,[3] Kyriaki Kekou,[4] Konstantina Kosma,[4,5]
Hugh Dawkins,[6] Leanne Lamont,[6] Anna J. Roy,[7] Teodora Chamova,[8] Velina Guergueltcheva,[8] Sophelia Chan,[9]


Research Article

Journal of Neuromuscular Diseases
1–9
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DOI: 10.1177/22143602241290353
journals.sagepub.com/home/jnd
Sage | IOS Press

International collaboration to improve knowledge on myotonic dystrophy type 2

Stojan Peric[1], Vukan Ivanovic[1], Emma-Jayne Ashley[2], Belen Esparis[3], Craig Campbell[4], Stephan Wenninger[5], Darren Monckton[6], Chiara Marini-Bettolo[7], Helen Walker[7], Stanislav Voháňka[8], Kleed Cumming[9], Anna Łusakowska[10], Victoria Hodgkinson[11], Marjan Cosyns[12], Miriam Rodrigues[13], Eppie Yiu[14], Radim Mazanec[15], Tanya Stevenson[9], Anna Kostera-Pruszczyk[10], Lawrence Korngut[11], Marlène Jagut[12],


Factors Associated With Health-Related Quality of Life in Children With Duchenne Muscular Dystrophy

Journal of Child Neurology
2016, Vol. 31(7) 879-886
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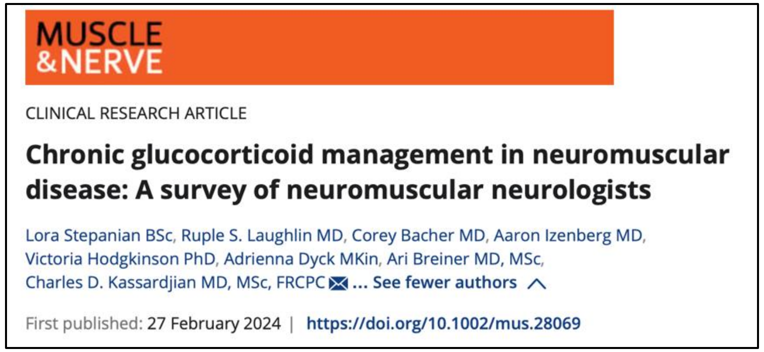
DOI: 10.1177/0883073815627879
jcn.sagepub.com
SAGE


ORIGINAL ARTICLE

Survey of Canadian Myotonic Dystrophy Patients' Access to Computer Technology

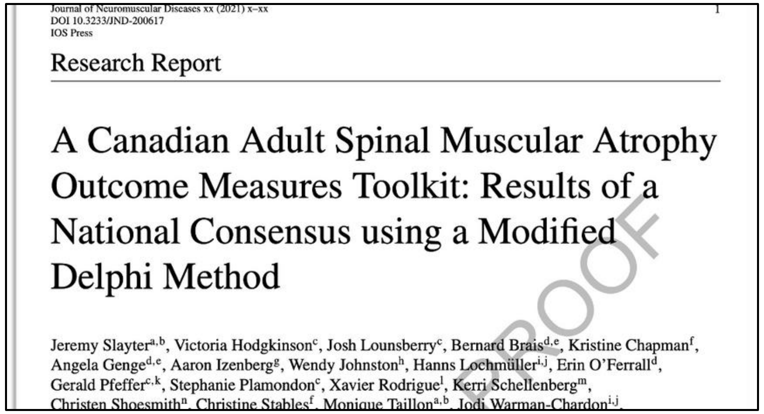

MUSCLE & NERVE

CLINICAL RESEARCH ARTICLE

Chronic glucocorticoid management in neuromuscular disease: A survey of neuromuscular neurologists

Lora Stepanian BSc, Ruple S. Laughlin MD, Corey Bacher MD, Aaron Izenberg MD, Victoria Hodgkinson PhD, Adrienna Dyck MKin, Ari Breiner MD, MSc, Charles D. Kassardjian MD, MSc, FRCPC ✉ ... See fewer authors ^

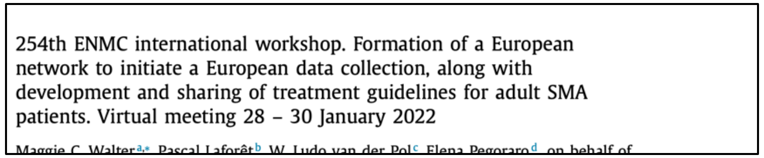
First published: 27 February 2024 | https://doi.org/10.1002/mus.28069


Journal of Neuromuscular Diseases xx (2021) x–xx
DOI 10.3233/JND-200617
IOS Press

Research Report

A Canadian Adult Spinal Muscular Atrophy Outcome Measures Toolkit: Results of a National Consensus using a Modified Delphi Method

Jeremy Slayter[a,b], Victoria Hodgkinson[c], Josh Lounsberry[c], Bernard Brais[d,e], Kristine Chapman[f], Angela Genge[d,e], Aaron Izenberg[g], Wendy Johnston[h], Hanns Lochmüller[i,j], Erin O'Ferrall[d], Gerald Pfeffer[c,k], Stephanie Plamondon[c], Xavier Rodrigue[l], Kerri Schellenberg[m], Christen Shoesmith[n], Christine Stables[f], Monique Taillon[a,b], Jodi Warman-Chardon[i,j],


254th ENMC international workshop. Formation of a European network to initiate a European data collection, along with development and sharing of treatment guidelines for adult SMA patients. Virtual meeting 28 – 30 January 2022

Maggie C. Walter[a,*], Pascal Laforêt[b], W. Ludo van der Pol[c], Elena Pegoraro[d], on behalf of

CNDR’s FSHD Program

Made possible by

FSHD CANADA FOUNDATION
Curing the #1 Form of Muscular Dystrophy

An FSHD-specific Canadian data program has launched!

Clinical Data

- Diagnosis
- Genetics
- Mobility
- Breathing tests
- Other conditions (eye, hearing, etc.)

Understand # of Canadians living with FSHD and geographic distribution

Facilitate FSHD clinical trials coming to Canada

Understand differences in care for ALL Canadians with FSHD

Understand disease progression (natural history)

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CNDR FSHD Program **Progress**

The current dataset will be revised as necessary to prepare for monitoring of therapy use and to align with the international community

2023:

Community engagement around what data should be collected

2024:

Initial administrative approvals to capture this data

2025:

Program launch ✅
National program roll-out ✅
BetterLife Canada integration (soon!)

2026:

Goal of data captured from 500 Canadians living with FSHD to inform research and advocacy

Through a collaborative partnership between CNDR, FSHD Canada Foundation, FSHD Society, and Lumiio…

BetterLife FSHD

is coming to 🇨🇦

Made possible by

FSHD CANADA FOUNDATION
Curing the #1 Form of Muscular Dystrophy

FSHD SOCIETY

CNDR.ORG
Canadian Neuromuscular Disease Registry

A new patient engagement solution that accelerates real-world data and supports patient journey & care pathways

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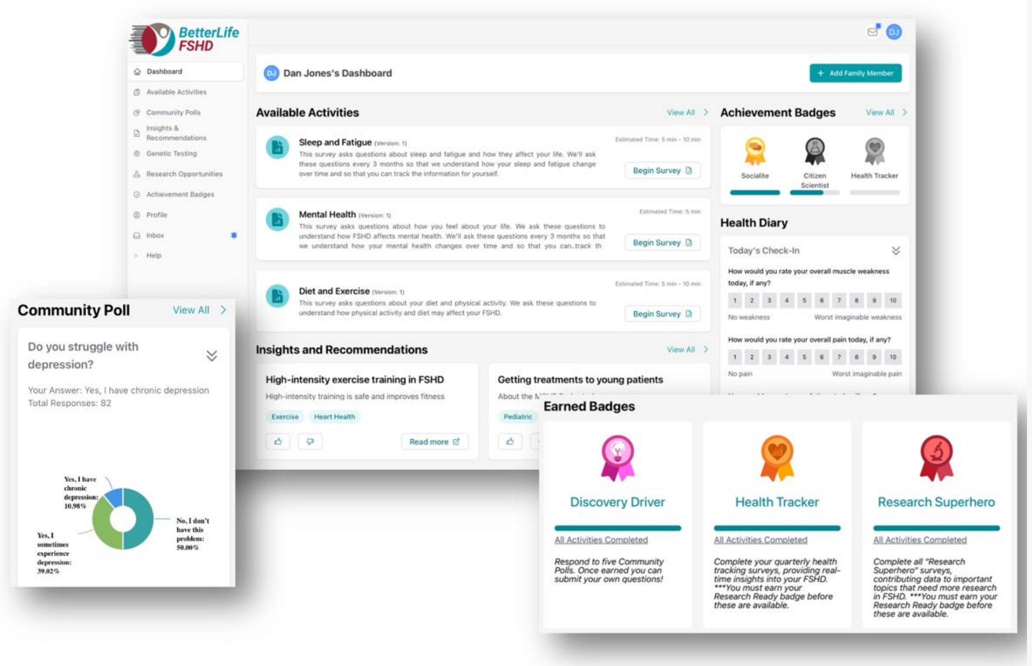
BetterLife FSHD



Dan Jones's Dashboard

Available Activities

Sleep and Fatigue (Version: 1) Estimated Time: 5 min - 10 min
This survey asks questions about sleep and fatigue and how they affect your life. We'll ask these questions every 3 months so that we understand how your sleep and fatigue change over time and so that you can track the information for yourself.

Mental Health (Version: 1) Estimated Time: 5 min
This survey asks questions about how you feel about your life. We ask these questions to understand how FSHD affects mental health. We'll ask these questions every 3 months so that we understand how your mental health changes over time and so that you can..track th

Diet and Exercise (Version: 1) Estimated Time: 5 min - 10 min
This survey asks questions about your diet and physical activity. We ask these questions to understand how physical activity and diet may affect your FSHD.

Achievement Badges

Health Diary

Today's Check-In

How would you rate your overall muscle weakness today, if any?
1 2 3 4 5 6 7 8 9 10
No weakness — Worst imaginable weakness

How would you rate your overall pain today, if any?
1 2 3 4 5 6 7 8 9 10
No pain — Worst imaginable pain

Community Poll

Do you struggle with depression?

Your Answer: Yes, I have chronic depression
Total Responses: 82

Yes, I have chronic depression: 10.98%
Yes, I sometimes experience depression: 39.02%
No, I don't have this problem: 50.00%

Insights and Recommendations

High-intensity exercise training in FSHD
High-intensity training is safe and improves fitness

Getting treatments to young patients
About the N

Earned Badges

Discovery Driver
All Activities Completed
Respond to five Community Polls. Once earned you can submit your own questions!

Health Tracker
All Activities Completed
*Complete your quarterly health tracking surveys, providing real-time insights into your FSHD. ***You must earn your Research Ready badge before these are available.*

Research Superhero
All Activities Completed
*Complete all "Research Superhero" surveys, contributing data to important topics that need more research in FSHD. ***You must earn your Research Ready badge before these are available.*

A patient-driven health platform and research registry

Designed by and for patients, to meet needs at every step in the patient journey

Built-in health and symptom tracking, exports for HCPs aligned w/care guidelines coming soon

Modern data infrastructure, core dataset alignment, use of validated PROs

Research portal to facilitate data requests and access

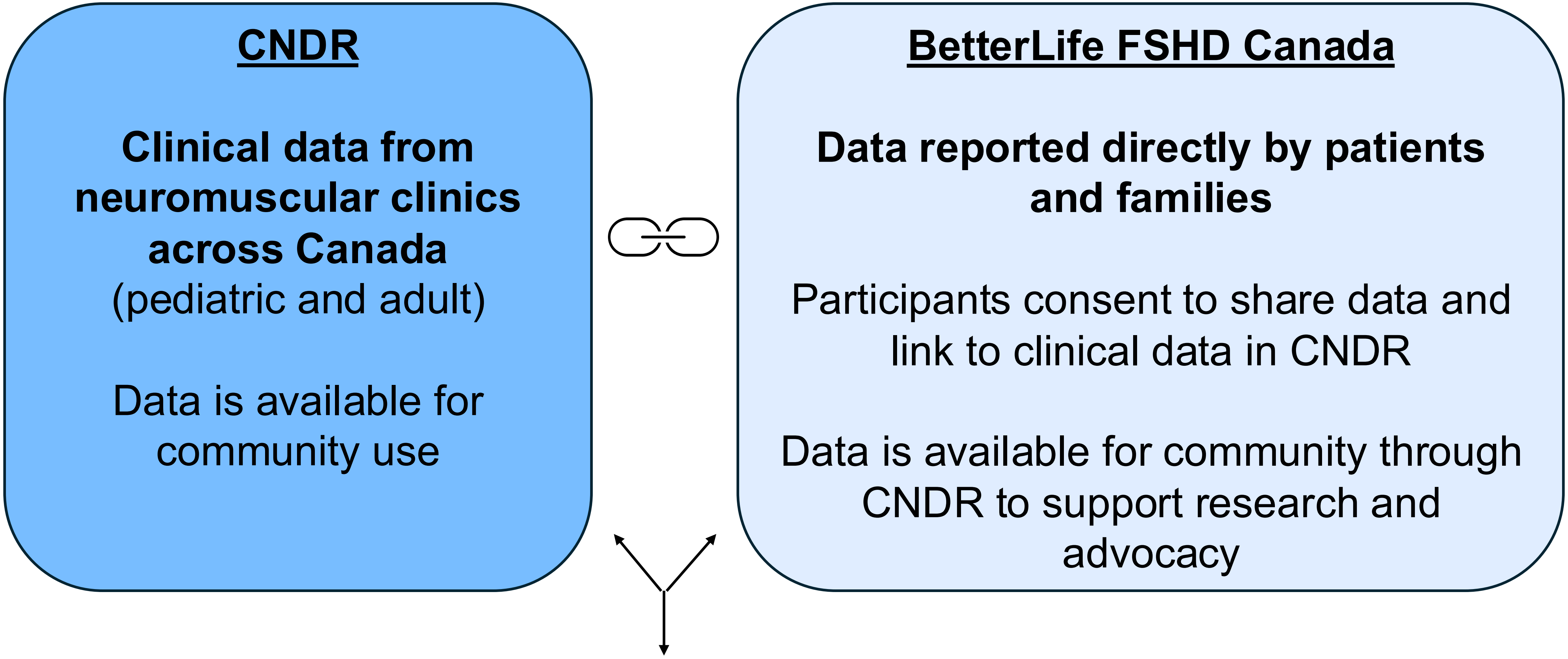
Help patients live a better life with FSHD

- Aim 1: Provide patients with personalized resources and data tracking
- Aim 2: Engage and connect patients with personalized research opportunities

Speed and improve FSHD research and therapeutic development

- Aim 3: Elevate the patient voice by performing patient experience-centered research
- Aim 4: Enable multi-disciplinary research by securely sharing data with other researchers working to advance disease understanding, clinical trials, clinical care, and access in FSHD

How will this work in Canada?



CNDR

Clinical data from neuromuscular clinics across Canada
(pediatric and adult)

Data is available for community use

BetterLife FSHD Canada

Data reported directly by patients and families

Participants consent to share data and link to clinical data in CNDR

Data is available for community through CNDR to support research and advocacy

National and international collaborations and linkages

CNDR.ORG
Canadian Neuromuscular Disease Registry

Learn more about CNDR & sign up:

Access our website:

cndr.org

Contact our office:

cndradmin@ucalgary.ca

403-210-7303

Thank you so much for your time!

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