CANADIAN NEUROMUSCULAR DISEASE REGISTRY

Impacting Patient Outcomes *using* Real-World Evidence

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The CNDR: What We Do

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 Canadian Neuromuscular Disease Registry (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

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

How it works: Core Elements

Bringing together the whole rare disease community

CNDR RESEARCH IS:

Guided by specific objectives

Overseen by governance process

Adaptable and responsive

Proper stewardship of data

How it works

1) Patients can register to share their data by completing an informed consent form for participation.

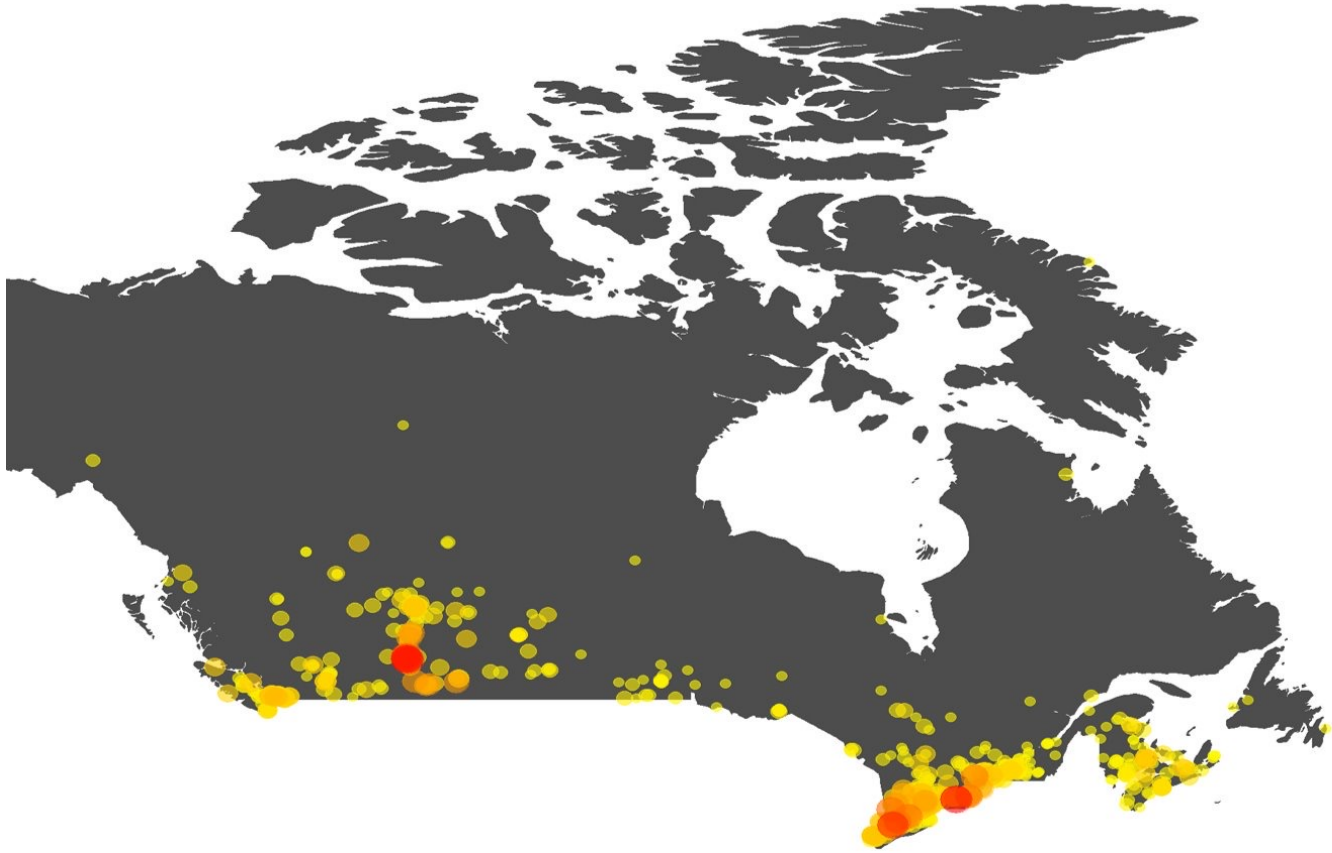
2) Your physician will provide your medical data into the registry.

3) Researchers, patient organizations, pharmaceutical partners, or government can apply to access the data

4) Following approval, aggregate analyses/numbers would be provided

5) Patients may be contacted for potential opportunities for additional research studies/trials

Over **6000** patients registered across Canada!



CNDR.ORG

New CNDR Dataset: FSHD

- Developed in collaboration with Muscular Dystrophy Canada
- Currently wrapping up clinician and patient roundtables to provide feedback on the draft dataset

CNDR is launching an FSHD-specific clinical dataset in 2024!

The dataset will collect clinical information on patients with FSHD to help facilitate research and readiness for clinical trials and potential new therapeutics

People living with FSHD are encouraged to participate in the CNDR!

CNDR Reach: Network Expertise and Advocacy

> Can J Neurol Sci. 2018 Sep;45(5):516-517. doi: 10.1017/cjn.2018.59. Epub 2018 Jul 24.

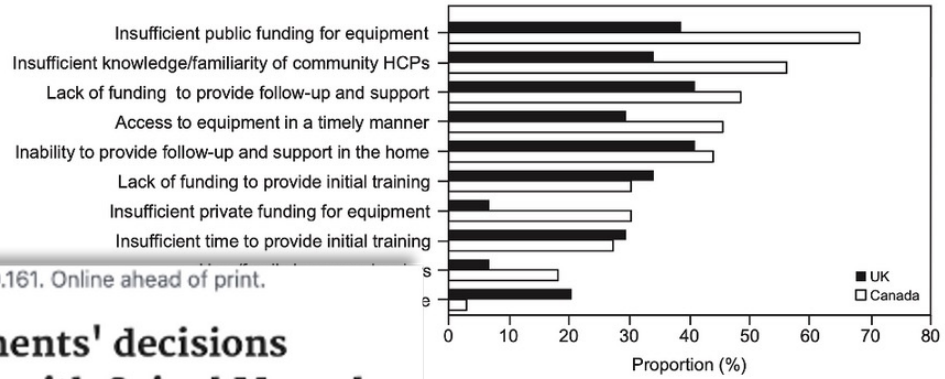
Response to the Canadian Agency for Drugs and Technologies in Health and Institut national d'excellence en santé et en services sociaux decision regarding nusinersen for Spinal Muscular Atrophy

Craig Campbell [1], Kathy Selby [2], Hugh McMillan [3], Jiri Vajsar [4], Lawrence Korngut [5], Bernard Brais [6], Alex MacKenzie [7], Maryam Oskoui [8]

Affiliations + expand

PMID: 30039778 DOI: 10.1017/cjn.2018.59

Monitoring Cough Effectiveness and Use of Airway Clearance Strategies: A Canadian and UK Survey



ovider.

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

Research Report

A Canadian Adult Spinal Muscular Outcome Measures Toolkit: Result National Consensus using a Modif 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],

> Can J Neurol Sci. 2020 Jul 27;1-10. doi: 10.1017/cjn.2020.161. Online ahead of print.

Response to provincial governments' decisions regarding monitoring for adults with Spinal Muscular Atrophy

Victoria L Hodgkinson, Kristine Chapman, Aaron Izenberg, Hanns Lochmüller, Colleen O'Connell, Erin K O'Ferrall, Maryam Oskoui, Gerald Pfeffer, Stephanie Plamondon, Xavier Rodrigue, Christen Shoesmith, Jodi Warman-Chardon, Bernard Brais, Lawrence Korngut, Kerri Schellenberg

PMID: 32713403 DOI: 10.1017/cjn.2020.161

ST. MICHAEL'S
UNITY HEALTH TORONTO

Managing chronic corticosteroids in neuromuscular disease: A Canadian Survey

CNDR Reach: Engaging with Patients

UNIVERSITY OF ALBERTA

DEPARTMENT OF MEDICINE
DIVISION OF NEUROLOGY

Research study: The ALS Talk Project

ALS Information Needs and Their Implications for Clinical Communication: An Online Focus Group Approach

You are invited to take part in an online focus group. The focus group is for **people diagnosed with amyotrophic lateral sclerosis** (ALS). We are interested in your experience talking to health care professionals about your diagnosis, treatment and advanced care planning. We want to learn about the information and information sources that you find helpful. You may feel supported by sharing ideas with other people with ALS. You may gain useful information from other people in the focus group.

Children's Hospital
London Health Sciences Centre

Western
UNIVERSITY · CANADA

Title: Health related quality of life in children and adolescents with Spinal Muscular Atrophy: A longitudinal study in Canada.

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

Journal of Child Neurology
2016, Vol. 31(7) 879-886

DOI: 10.1177/0883073815627879
jcn.sagepub.com
SAGE

Published in final edited form as:
Dev Med Child Neurol. 2016 July ; 58(7): 698–705. doi:10.1111/dmcn.12948.

NMD4C

MDC Muscular Dystrophy Canada / Dystrophie musculaire Canada

COVID-19 and Neuromuscular Patients

April 3, 2020

There is an abundance of information available on COVID-19, but little guidance specific to Canadians with neuromuscular disease (NMD), their families, and their caregivers. We at the Neuromuscular Disease Network for Canada (NMD4C) and Muscular Dystrophy Canada (MDC) hope to support the community by compiling this information, recommendations, and links to additional sources. Information is provided to the best of

Parent-reported multi-national study of the impact of congenital and childhood onset myotonic dystrophy

DALHOUSIE UNIVERSITY

Information Form

Study Title: A survey of Canadian youth with Duchenne and Becker Muscular Dystrophy exploring gender identity, sexuality, sexual health questions and concerns.

ORIGINAL ARTICLE

Survey of Canadian Myotonic Dystrophy Patients' Access to Computer Technology

CNDR Reach: Patient Engagement Survey

UNDERSTANDING THE PATIENT VOICE IN REGISTRY RESEARCH

Through a CNDR patient preference survey

SURVEY RESULTS:

Most Important Items

1. Medications & Treatments
2. Independence in daily activities
3. Mobility and Motor Function
4. Fatigue/Energy Levels

Motivations for Registry Participation

1. Improve my own care
2. Participate in CT's
3. Contribute to knowledge and understanding
4. Desire to help others
5. Participate in other research (i.e., surveys)

Registering to Participate in the CNDR

What do I do?

Scan to access cndr.org

STEP 1 | Connect with the CNDR national office (www.cndr.org) and/or your ALS physician

STEP 2 | Read an informed consent form that explains what's required of you, and how the registry works.

STEP 3 | Contact the CNDR national office or your physician if you have questions

STEP 4 | Sign up to request your medical information to be shared!

Funders & Partners

Past & Present

- Mitsubishi Tanabe Pharma Canada
- Amylyx
- Biogen
- Cytokinetics
- Roche
- Novartis
- Dynacure
- Defeat Duchenne Canada
- Pfizer
- Sanofi Genzyme
- Cure SMA
- ALS Society of Canada
- Starratt Family Foundation
- The Marigold Foundation
- Muscular Dystrophy Canada
- NMD4C
- CALS
- TREAT-NMD
- CDC
- Statistics Canada
- CADTH

Contacting the CNDR

Access our website:

cndr.org

Contact our office:

cndradmin@ucalgary.ca

403-210-7303