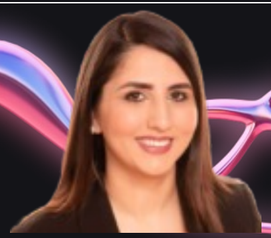
MUSCULAR DYSTROPHY CANADA
DYSTROPHIE MUSCULAIRE CANADA

DOCUMENTING SOCIOECONOMIC AND QUALITY OF LIFE IMPACT OF NEUROMUSCULAR DISORDERS



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MUSCULAR DYSTROPHY CANADA
DYSTROPHIE MUSCULAIRE CANADA

Let's Find Your Pathway of Possibilities

MDC works in partnership with individuals affected by neuromuscular disorders and their families to navigate and access resources at every stage of life, regardless of type of NMD, age, stage, or location in Canada.

ACHIEVE YOUR PERSONAL GOALS WITH OUR SERVICES.
WE CAN SUPPORT YOU WITH:

- Obtaining an accurate **diagnosis**
- **Managing and adapting** to your diagnosis
- Exploring and participating in **clinical trials**
- **Finding specialists** and multidisciplinary healthcare supports
- Gaining **trustworthy evidence-based information** to enable informed decisions through MDC's expertise and networks
- Researching and accessing **treatment and rehabilitation options**
- Accessing **financial resources**
- Obtaining **appropriate assistive devices and equipment** for safety, inclusion and full participation
- Accessing **community supports** for housing, job opportunities, education, and volunteering – including application assistance and navigating wait times
- Building **advocacy skills** to influence decisions at the local, provincial, and national levels
- **Connecting with peers** for mutual support and shared experiences
- **Adapting to ongoing changes** due to the progressive nature of neuromuscular disorders or the aging process

OUR IMPACT:

- Enhanced **healthcare outcomes** and experiences (↓ time to diagnosis and treatment, ↓ admissions to hospitals, ↓ serious complications)
- Enhanced knowledge for **informed decision-making**
- Enhanced access to **options and solutions**
- Enhanced **safety** and independence
- Enhanced **quality of life**

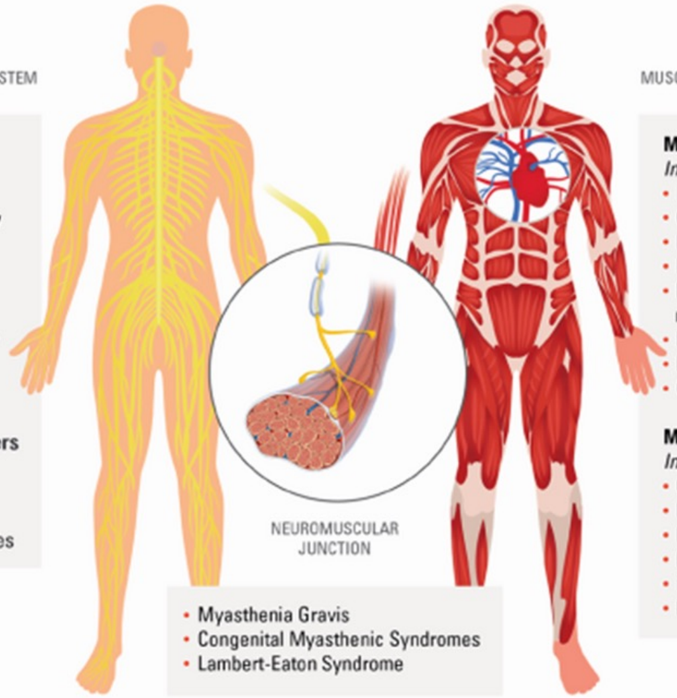
Muscular Dystrophy Canada Covers Disorders Affecting the:

Peripheral Nervous & Muscular Systems

PERIPHERAL NERVOUS SYSTEM

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



NEUROMUSCULAR JUNCTION

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

MUSCULAR SYSTEM

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

Ottawa

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

CANADA

Most expensive medication in the world now offered in Quebec

MONTREAL – The most expensive medication in the world, Zolgensma, will be available in Quebec as of Oct. 20, La Presse has learned. About 10 families across the province were pushing for the gene therapy medication, which costs $2.8 million a dose, to be offered to their children with spinal muscular atrophy, like in Ontario and Alberta.

British Columbia

B.C. funding decision on pricey drug leaves teen with rare disease facing 'scary' future

Miles Ambridge just misses cut-off age to access Spinraza, a potentially life-changing treatment

CANADA

At $2.8 million it's the world's most expensive drug. For these children, it may be the price for a normal life

Zolgensma, a treatment for Spinal Muscular Atrophy, is a life-changing drug for children. But timing is critical — it must be received before age 2. And it's not yet approved in Canada.

Local News

Ontario to cover cost of drug for rare neuromuscular disease on a 'case-by-case basis'

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

RED DEER | News

Red Deer parents start $32K-GoFundMe to buy wheelchair for son with neuromuscular disorder

Edmonton

Alberta family race to raise $2.8M to treat son 'Mighty' Max's rare disease

Max was diagnosed with type two of spinal muscular atrophy (SMA) in November

NEUROMUSCULAR DISORDERS HAVE GAINED MORE ATTENTION IN RECENT YEARS DUE TO HIGH COSTS ASSOCIATED WITH HEALTH CANADA APPROVED THERAPIES.

do the expenses incurred from not having therapies outweigh the costs of drugs for rare diseases?

Walk Example

argenx

argenx Announces VYVGART (efgartigimod alfa) Authorized for Sale by Health Canada for Generalized Myasthenia Gravis

Significant Impact on Productivity (at Work and Home)

*"I am **unable to work**, need to rest frequently, need help with activities like washing my hair, etc."*

*"I had to **retire** from my job because of MG."*

"I retired because the stress of my job plus MG did not mix well."

"I have worked while experiencing a MG crisis – it was horrible. I am fearful for the next crisis."

"I am on disability leave because of my MG."

"I am no longer able to work and rely fully on my husband for my meals, clean home and being moved from one place to another."

"I wasn't able to work today because I was very tired. It gets in the way of my ability to work. And this impacts my finances in a huge way."

"I am not able to do the work I was once able to because I can't strain my eyes and read for more than 20 minutes."

"I feel useless at home. Everything now falls on my husband. Taking care of the children, cleaning, cooking and taking care of everything that revolves around IVIG treatment. MG is unreliable and my ability to support is unreliable."

CADTH Ruling on SPINRAZA™ (nusinersen) Extinguishes Hope for Adults Needing Treatment for Spinal Muscular Atrophy (SMA) Français

Biogen

Impact on Work Participation

*"I have very limited use of my four limbs. My dexterity is extremely difficult to even bend my fingers. **I used to work for the Toronto star for 25 years. When I turned 51 I became very fatigued so I had to go on long-term disability.** I am in a motorized chair and I do have PSW's come in to help in the morning and night."*

*"It has been progressive. I am a father of three and especially over the last two years, I cannot do any long distance walks (a block), I can no longer climb stairs. **I had to advise work of my condition. I had to share with everyone that in the new future I would need some assistance.** The impact is the slow degradation and loss of the ability to do things. Also, the inability to do things with my family."*

*SMA affects every aspect of my life. Living confined to a wheelchair with extremely weak muscles limits my ability to do anything normally. I rely on others 24 hours a day to help me with most aspects of my life. **I am able to continue to hold down a job and function fairly well at it with help. As my muscles weaken, I don't know how long that will continue.**"*

*"**Maintaining a career or position outside of my home is incredibly difficult**, as I would have to pay someone to be with me most of the time. So I have chosen not to pursue a typical nine to five job and take advantage of opportunities*

"A neuromuscular disorder (NMD) can affect everything. 100% of our lives. Every day it impacts pretty much every aspect of our family life. How we plan our day, working full-time, caregiver commitments and responsibilities, stress, and concern for overall mental and physical health. It impacts our finances, emotions...everything. We are fortunate he is a smart, strong-willed, resilient, independent young man, but the neuromuscular disorder still gets in the way, in some form, every day... for all of us."

Parent of a child affected by spinal muscular atrophy (SMA)

LIVING WITH A NEUROMUSCULAR DISORDER IN CANADA CAN BE EXPENSIVE

01 SOCIETAL IMPACT

02 INDIRECT AND OUT OF POCKET EXPENSES

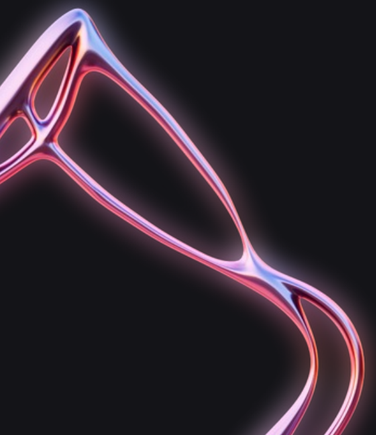
03 VALUE OF EARLY DIAGNOSIS

04 DISABILITY WITHOUT POVERY

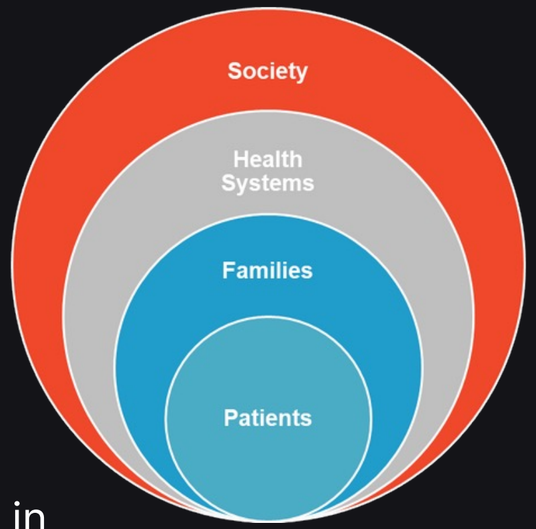
05 HOW TO CAPTURE TRUE COSTS OF RARE DISEASES?

SETTING THE STAGE

- Although individually rare, more than 70,000 Canadians are affected by one of the 600 genetic and autoimmune neuromuscular disease (NMD) subtypes. Many NMDs are characterized by profound weakness and/or sensory loss and also have multisystem involvement including cardiac and respiratory failure or intellectual delay
- New disease-modifying therapies are emerging from clinical trials, robust changes happening to standards of care and persons with genetic and acquired NMDs are receiving more complex care at home
- In Canada, approximately 70% of health care expenditures, including physician services, diagnostic tests, and hospitalization expenses, are covered by government funding. Direct cost include “hospital care expenditure, physician care expenditures, prescription drug expenditures, dental services, vision care services and formal caregiving”.
- The remaining 30% of health care expenses are either covered by private health plans or become out-of-pocket expense for patients.



MEASURING THE SOCIETAL IMPACT OF NEUROMUSCULAR DISORDERS



01 LIMITED DATA SOURCES

Research on the costs of neuromuscular disorders is limited, in part because of the difficulty in accessing accurate information on healthcare resource utilization from public health administrative datasets, patient registries, claims data, etc.

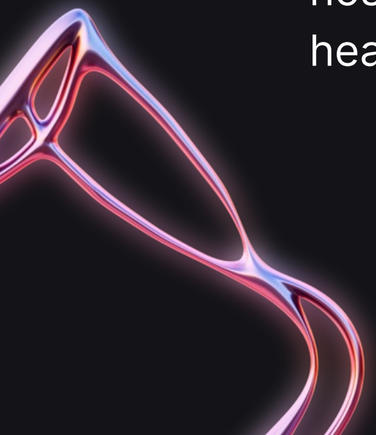
02 COMPREHENSIVE COSTS

There is increasing evidence that costs of diseases not only arise from the utilization of healthcare but also from non-healthcare or indirect factors, ultimately causing financial hardship. Therefore, identifying the areas in which the socio-economic burden falls on is important for effective resource planning and allocation.

THERE IS INCREASING EVIDENCE THAT COSTS OF DISEASES NOT ONLY ARISE FROM THE UTILIZATION OF HEALTHCARE BUT ALSO FROM NON-HEALTHCARE OR INDIRECT FACTORS, ULTIMATELY CAUSING FINANCIAL HARDSHIP.

01 DIRECT COSTS

• Direct costs include the cost of treatment, medical procedures, hospitalizations, physician visits, home healthcare, and other medical costs.



02 INDIRECT COSTS

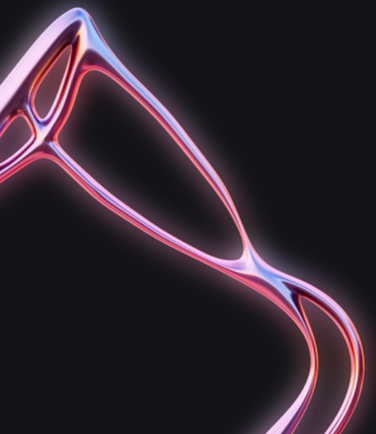
• Indirect costs include patient and caregiver productivity loss, work loss, home and vehicle changes, traveling and accommodation for medical visits, assistive devices and technologies.

Neuromuscular Disorder

- Duchenne Muscular Dystrophy
- Myotonic Dystrophy
- Spinal Muscular Atrophy
  - Subtype 1
  - Subtype 2
  - Subtype 3
- Facioscapulohumeral Muscular Dystrophy
- Charcot-Marie-Tooth Disease
- Myasthenia Gravis

Healthcare Resource & Other Cost Categories

- Physician Visits
- Emergency Room Visits
- Surgeries/Interventional Procedures
- Inpatient Stays
- Assistive Devices
- Modifications
- Allied Healthcare Professionals
- Professional/Paid Caregivers
- Lost Caregiver Productivity
- Lost Patient Productivity
- Medications
- Monitoring Tests
- Novel or Special Access Program Medications



COST TO SOCIETY FOR SIX NEUROMUSCULAR DISORDERS IS $4.7 BILLION/YEAR

Total Costs of All NMD in the Total Cohort Over a 1-Year Time Horizon

Total Economic Co: $4,694,944,427.86 otal Cohort: 33,781

- Physician Visits
- Emergency Room Visits
- Surgical/Interventional Procedures
- Inpatient Stays
- Assistive Devices
- Modifications
- Allied Healthcare Professionals
- Professional/Paid Caregiver
- Caregiver Employment
- Patient Employment
- Medications
- Monitoring Tests

Healthcare Resource	Economic Cost	% Breakdown
Physician Visits	$20,622,432	0.44%
Emergency Room Visits	$9,561,225	0.20%
Procedures	$675,081,754	14.38%
Inpatient Stays	$1,313,916,304	27.99%
Assistive Devices	$219,672,024	4.68%
Home/Vehicle Modifications	$172,194,090	3.67%
Allied Healthcare Professionals	$55,911,558	1.19%
Professional/Paid Caregiver	$235,399,935	5.01%
Caregiver Lost Productivity	$883,575,241	18.82%
Patient Lost Productivity	$1,102,072,472	23.47%
Medications	$5,150,313	0.11%
Monitoring Tests	$1,787,079	0.04%
Novel or Special Access Program Medications	$0	N/Ap

- **The largest cost category was caregiver and patient lost productivity** (42% of total) followed by inpatient stays (28% of total).
- This study only investigated the direct and indirect costs of NMDs for a small subset of NMDs and the contribution of indirect costs has not been assessed comprehensively in all NMDs.
- Individuals with NMDs and their caregivers incur considerable indirect, out-of-pocket, non-medical costs including purchasing of medical services (e.g., personal support workers, home rehabilitation services), travel, lodging, home renovations (e.g., ramps) and wheelchair accessible vehicles. Individuals with genetic and acquired NMDs and their caregivers also experience lost earnings and productivity.

FROM A PATIENT'S PERSPECTIVE, THE ACTUAL OUT-OF-POCKET AND INDIRECT EXPENSES ARE MOST CRITICAL, BUT THEY ARE LARGELY "INVISIBLE" IN MOST ECONOMIC EVALUATIONS.

The indirect costs of a large number of NMD in Canada have not been assessed comprehensively. Fragmented healthcare and social security systems within Canada contribute to the difficulties studying the burden of rare diseases. International comparisons are not necessarily valid due to differences in public and social policy.

An improved understanding of the burden of NMDs is essential for governmental agencies, insurance providers, patient partners, and society as a whole to deliver effective supports to those individuals with NMD and their caregivers.

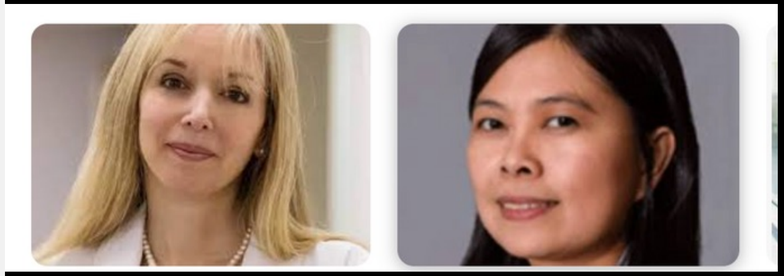
INDIRECT SOCIO-ECONOMIC BURDEN OF INHERITED NEUROMUSCULAR DISORDERS

CIHR Grant Funding for BIND Study: Assessing the Indirect Socio-Economic Burden of Inherited Neuromuscular Diseases

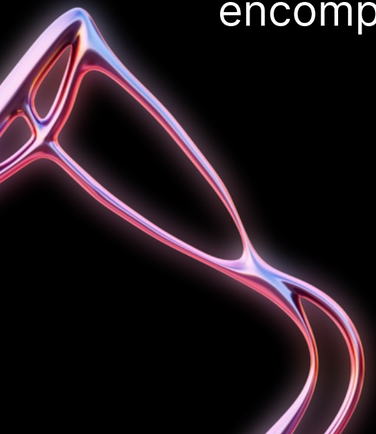
Published: 07 September 2021

The NMD4C is happy to share that a project application featuring many NMD4C members on Assessing the Indirect Socio-Economic Burden of Inherited Neuromuscular Diseases has been awarded a Canadian Institute of Health Research (CIHR) research grant in their recent round of funding decisions!

CIHR IRSC
Canadian Institutes of Health Research
Instituts de recherche en santé du Canada



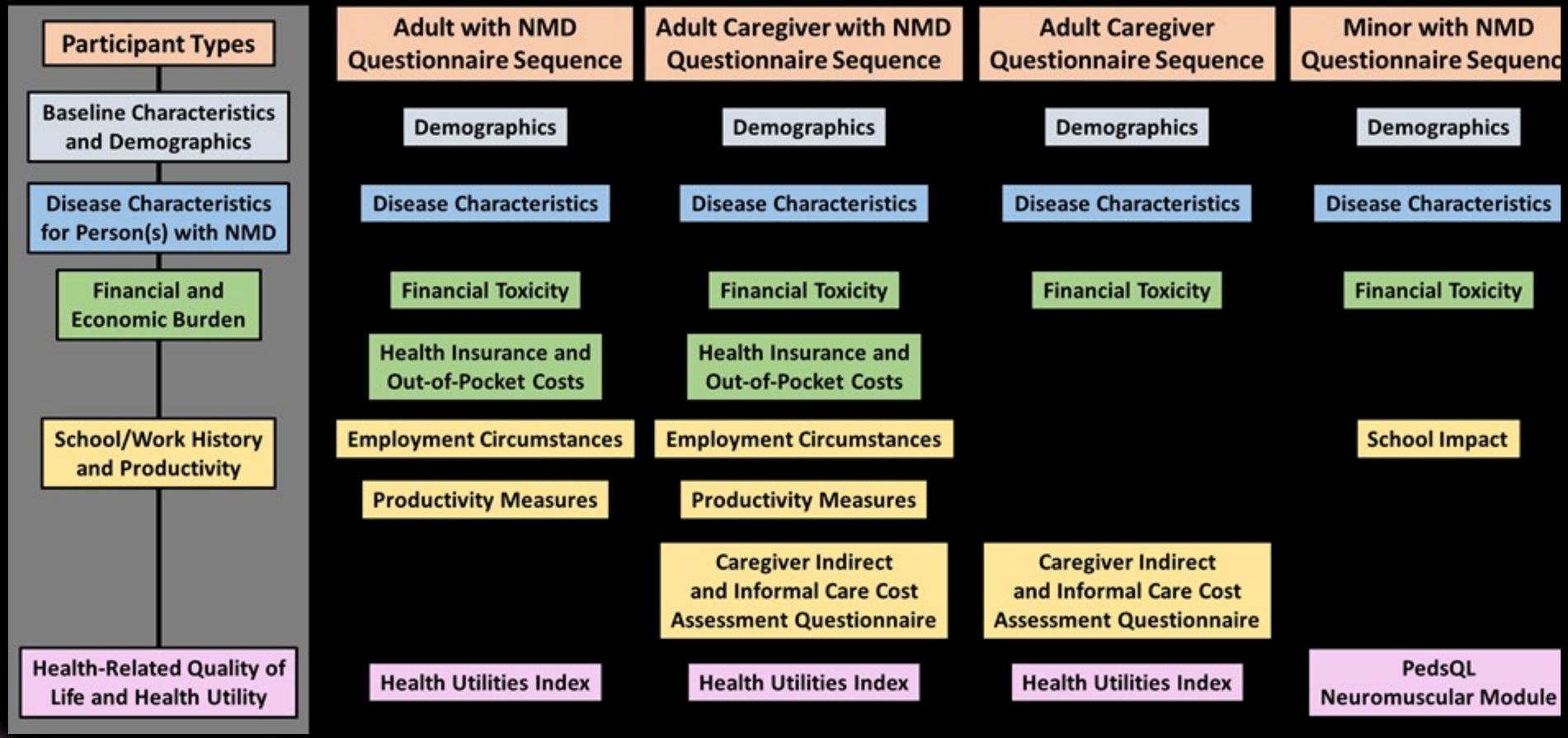
- Quantify the direct and indirect financial and social burden of patients and caregivers experience with NMD, encompassing schooling and education achievement, HRQoL, and labour force participation and productivity.



CNDR.ORG
Canadian Neuromuscular Disease Registry

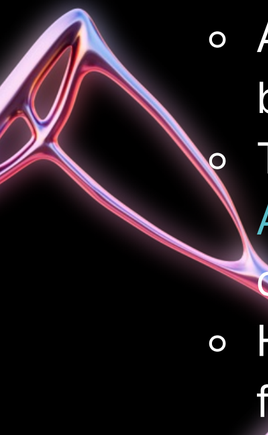
NMD4C

INDIRECT SOCIO-ECONOMIC BURDEN OF INHERITED NEUROMUSCULAR DISORDERS



INDIRECT SOCIO-ECONOMIC BURDEN OF INHERITED NEUROMUSCULAR DISORDERS

- The economic burden is conceptualized as psychosocial as well as direct and indirect costs.
- In this study, we focus on the impact of NMDs on schooling, education attainment, labour force participation, quality-adjusted life expectancy (QALE), and indirect costs (lost earnings and productivity by the patient or caregivers attributed to NMD diagnosis). Study questionnaires are based on the existing validated and standardized questionnaires to measure the disease burden
  - To gauge the financial distress experienced by individuals with NMD and their caregivers, participants will be asked to complete the FACIT – COST measure of financial toxicity.
  - To assess health-related quality of life and health utility, we will use a validated and standardized questionnaire, the Health Utilities Index (HUI®) to describe health status and to obtain utility scores of multi-attribute health-status classification systems
  - Adults with NMDs are asked about absenteeism, presenteeism as well as the impairments in unpaid activity because of NMDs using the Work Productivity and Activity Impairment questionnaire (WPAI)
  - To assess the economic cost to caregivers, we will use the Caregiver Indirect and Informal Care Cost Assessment Questionnaire (CIIQ) to measure, value, and estimate caregiver indirect (productivity) and informal care costs
  - HRQoL for pediatric population is assessed using the PedsQL Neuromuscular Module Version 3.0 Parent report for Child.



EARLY FINDINGS

THERE ARE AVOIDABLE PER PATIENT MEDICAL COSTS AND PRODUCTIVITY LOSSES ATTRIBUTABLE TO DELAYED DIAGNOSIS

TIMELY DIAGNOSIS AND SCREENING CAN SHORTEN AND POSSIBLY ELIMINATE THE DIAGNOSTIC ODYSSEY WHILE SIGNIFICANTLY REDUCING THE COST IMPACT OF NEUROMUSCULAR DISORDERS FOR INDIVIDUALS, FAMILIES, AND THE HEALTHCARE SYSTEM

MYASTHENIA GRAVIS JOURNEY MAP IN CANADA

MUSCULAR DYSTROPHY CANADA
DYSTROPHIE MUSCULAIRE CANADA

SYMPTOM ONSET

EYES
- DROOPING
- BLURRED OR DOUBLE VISION
- WEAKNESS OF THE EYE MUSCLES
- EYE FATIGUE

RATIONALIZATION...
IS IT STRESS?

START TO EXPERIENCE SYMPTOMS

CHANGES IN FACIAL EXPRESSIONS AND BULBAR FUNCTION
- DIFFICULTY SWALLOWING,
- SHORTNESS OF BREATH
- IMPAIRED SPEECH

LACK OF AWARENESS ABOUT MG

GOOGLE

AS SYMPTOMS WORSEN
PEOPLE SEARCH FOR POSSIBLE CAUSES, MG IS NOT USUALLY MENTIONED...

EVENTUALLY
THE SYMPTOMS BECOME BOTHERSOME ENOUGH TO PROMPT CONTACTING THEIR HEALTHCARE PRACTITIONER

PRE-DIAGNOSIS

MEDICAL GASLIGHTING
EXPERIENCED FROM HEALTHCARE PRACTITIONERS

IT'S ALL IN HER HEAD..

FREQUENT REFERRALS TO SPECIALISTS FOR TESTS

EMERGENCY

ON AVERAGE, PEOPLE WILL VISIT 7 DIFFERENT HEALTHCARE PROFESSIONALS AND GO TO A CLINIC OR HOSPITAL 9 TIMES BEFORE RECEIVING AN MG DIAGNOSIS

PEOPLE MIGHT ENTER A CRISIS (ICU) OR SYMPTOMS CAN CONTINUE TO WORSEN IMPACTING ABILITY TO WORK AND FUNCTION AT HOME

DIAGNOSIS

- BELL'S PALSY
- DEPRESSION
- STROKE
- CHRONIC FATIGUE
- LYME
- LUPUS
- FIBROMYALGIA
- ETC...

MANY MG PATIENTS UNDERGO A LONG JOURNEY OF TESTS AND MISDIAGNOSES:

ON AVERAGE IT CAN TAKE 5 YEARS TO RECEIVE A DIAGNOSIS AFTER 6 MISDIAGNOSES

DIAGNOSIS

MEDICAL HISTORY REVIEW, PHYSICAL EXAMINATION DIAGNOSTIC TESTS TO CHECK FOR SPECIFIC ANTIBODIES, NEUROPHYSIOLOGICAL AND IMAGING STUDIES.

MG CAN BE CLASSIFIED IN DIFFERENT WAYS

GENERALIZED MG

EARLY-ONSET MG

THYMOMA-ASSOCIATED MG

OCULAR MG

MG WITH ANTI-MUSK AND LRP4+ ANTIBODIES

MG WITH NO DETECTABLE ANTIBODIES

PREDNISONE AND MESTINON TYPICALLY PRESCRIBED

MISSING THE DIAGNOSIS HAS NEGATIVE SOCIAL, PSYCHOLOGICAL, MEDICAL, AND ECONOMIC IMPACTS

TREATMENT

IVIG

MEDICATIONS ARE PRESCRIBED BY TRUSTED PROFESSIONALS THAT SPECIALIZE IN MG

THERE IS NO CURE FOR MG. THERE ARE MEDICATIONS THAT ARE EITHER HEALTH-CANADA APPROVED FOR MG OR OFF-LABEL PRESCRIBED TO HELP ADDRESS/MINIMIZE SYMPTOMS

TRADE OFFS

TREATMENT BURDEN
- WEIGHT GAIN
- MOOD CHANGES
- GASTROINTESTINAL HEALTH

TREATMENT EFFICACY

MG CAN BECOME CONTROLLED WITH MEDICATIONS. TREATMENT PATHWAYS FOR MG ARE NOT STANDARDIZED

TREATMENTS ARE VERY SLOW TO TAKE EFFECT: PEOPLE ARE TOLD TO WAIT SEVERAL MONTHS AND YEARS BEFORE IMPROVEMENTS CAN TAKE PLACE

LACK OF COMMUNICATION

THERE IS A NEED FOR IMPROVED TREATMENT OPTIONS FOR MG

POST-DIAGNOSIS

NEVER GIVE UP!

PEOPLE FEEL LIKE THEY ARE CONSTANTLY LOOKING FOR INFORMATION ON MG, NOT SATISFIED WITH THE CARE THEY ARE BEING PROVIDED, AND FEEL LIKE THEY ARE NOT IN CONTROL OVER THEIR HEALTHCARE – ADVOCATING AND TAKING CHARGE IS IMPORTANT

MUSCLE WEAKNESS IN MG IS UNSTABLE, FLUCTUATING AND UNPREDICTABLE, WHICH IS DIFFICULT TO LIVE WITH AND REDUCES QUALITY OF LIFE

EMOTIONAL AND PSYCHOLOGICAL SUPPORT IS IMPORTANT

PEOPLE WITH MG LEAN ON INFORMAL CAREGIVING AND RECEIVE HELP FROM FAMILY MEMBERS

FATIGUE
SLEEP↓
GOOD DAYS
BAD DAYS
SOCIAL ACTIVITIES CHALLENGE
GRIEVING LIFE BEFORE DIAGNOSIS

DEVELOPING COPING STRATEGIES TO HELP WITH FLUCTUATIONS...

RESILIENCE IS KEY!

OVERARCHING THEMES

- MEDICAL GASLIGHTING
- WITHHOLDING SYMPTOMS/ SELF DOUBT
- CULTURAL CAPITAL AND SOCIAL INEQUALITY IN HEALTHCARE
- HIDDEN AND NON-VISIBLE DISABILITY
- SNOWBALL EFFECT
- HIGH USE OF HEALTHCARE RESOURCES
- IMPACT ON WORK AND SOCIAL PARTICIPATION
- INFORMAL CAREGIVING
- UNPREDICTABILITY AND UNCERTAINTY OF DISEASE
- PATIENT BEING REQUIRED TO ADVOCATE

Think Link graphics

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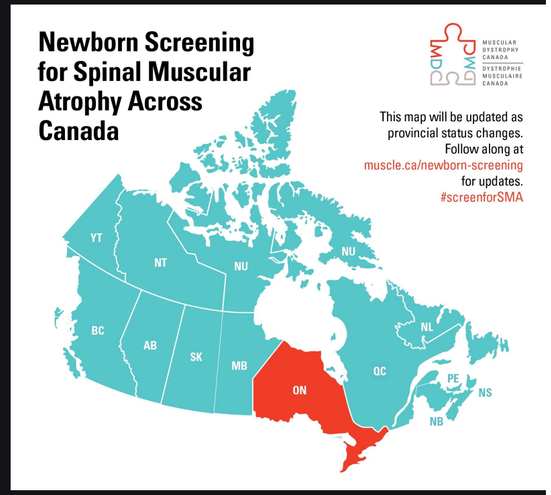
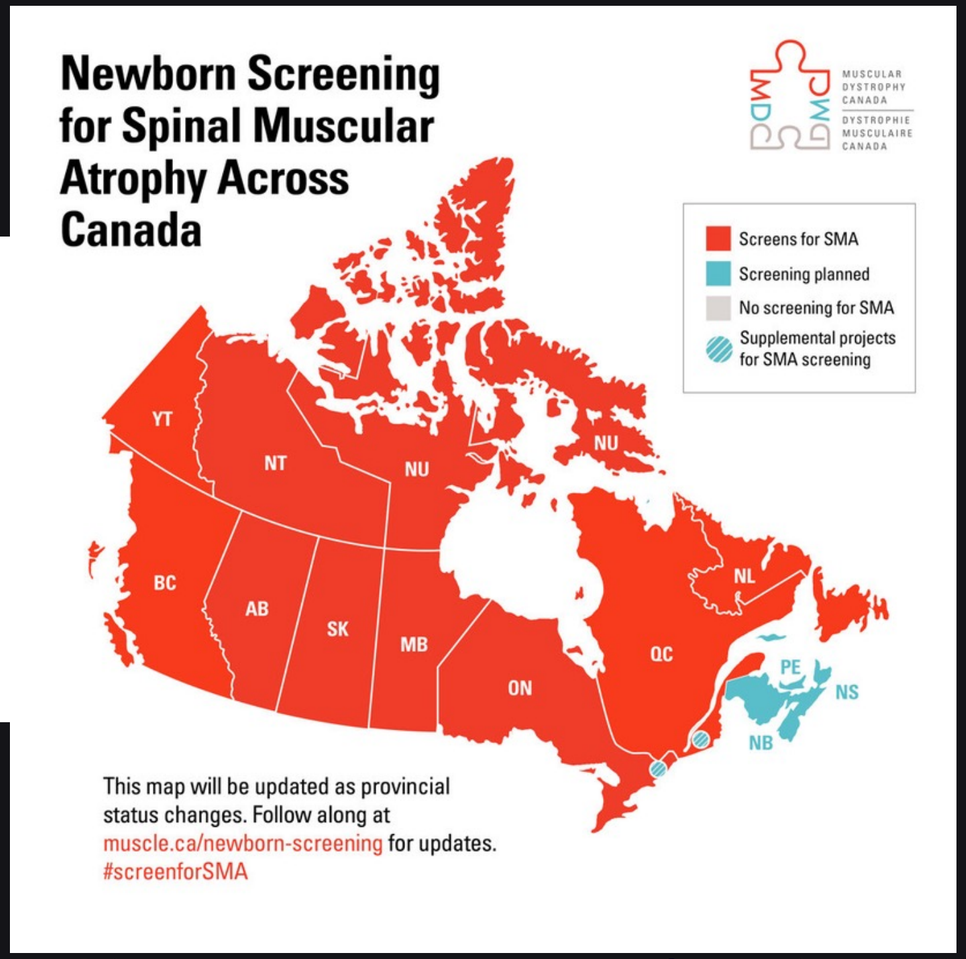




Economic Evaluation

Cost-Effectiveness of Newborn Screening for Spinal Muscular Atrophy in The Netherlands

Rimma Velikanova MSc [1 2], Simon van der Schans MSc [3], Matthias Bischof PhD [4], Rudolf Walther van Olden MD, PhD [4], Maarten Postma PhD [1 3 5], Cornelis Boersma PhD [1 3 6]

Cost-Effectiveness of Newborn Screening for Spinal Muscular Atrophy in England

ORIGINAL RESEARCH | Open access | Published: 24 May 2023 | 12, 1205–1220 (2023)

P.44 Cost-effectiveness of spinal muscular atrophy newborn screening in Belgium

T. Dangouloff [1], P. Thokala [2], A. Daron [3], S. Delstanche [3], L. Servais [1], M. Hiligsmann [4]

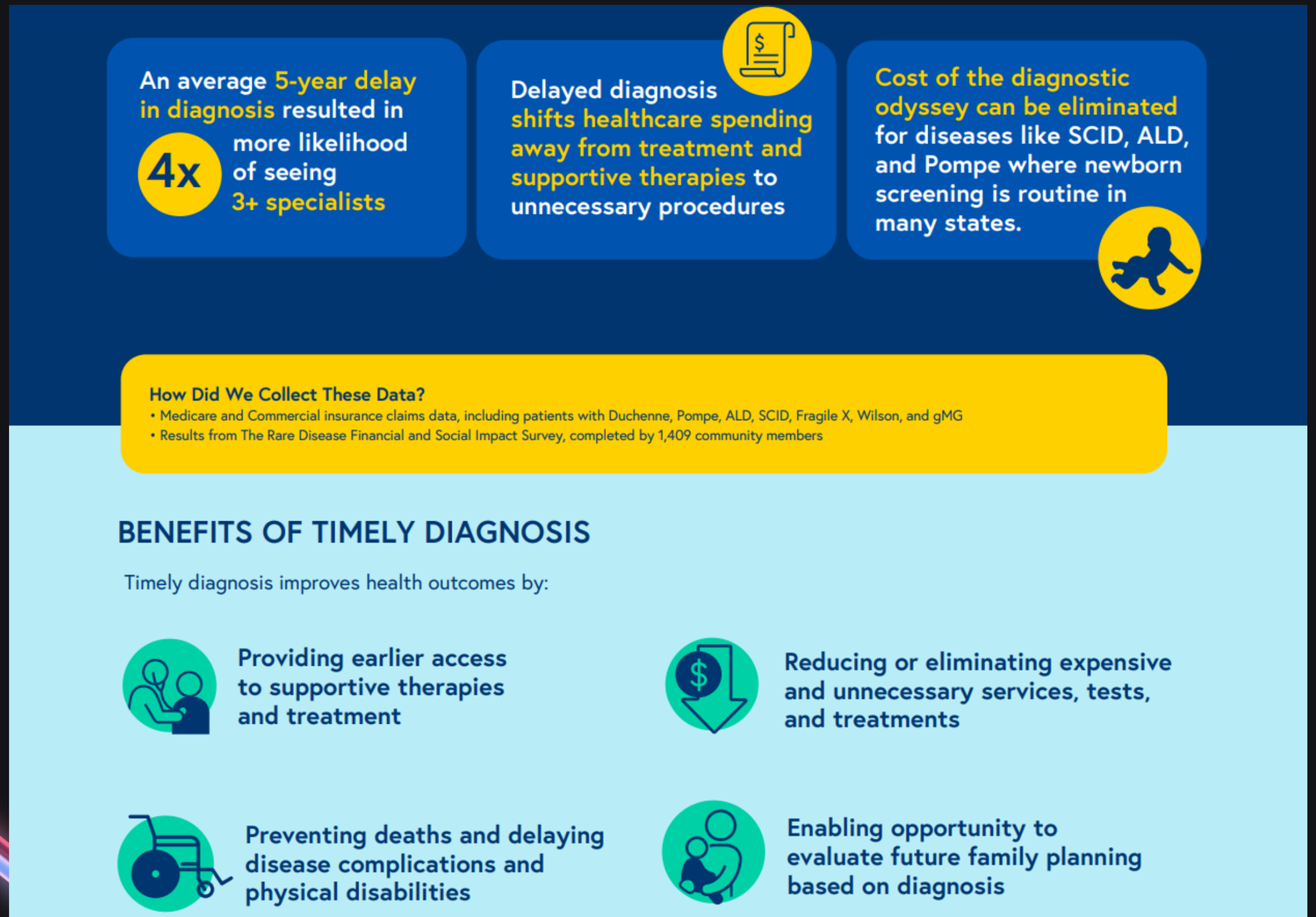
NBS improves health outcomes for patients with SMA and is less costly compared with no screening; therefore, it is a cost-effective use of resources.

NBS for early identification and treatment of SMA versus later symptomatic treatment after clinical diagnosis improves health outcomes and is less costly and, therefore, is a cost-effective use of resources.

Results were robust in sensitivity and scenario analyses.

THE COST OF A DELAYED DIAGNOSIS

Diagnostic inefficiencies not only results in potential of leading to costly and unecessary tests and treatments, but they can also push the patient beyond treatment windows, a major concern for rare diseases. not only results in potential of leading to costly and unecessary tests and treatments, but they can also push the patient beyond treatment windows, a major concern for rare diseases.



THE SOCIOECONOMIC IMPACT OF NEUROMUSCULAR DISORDERS EXTENDS BEYOND THE INDIVIDUAL PATIENT

THE IMPACT OF NMDS ON PATIENTS AND CAREGIVERS IS SEEN IN MANY DIFFERENT FACETS OF THEIR LIVES. ACCORDING TO NUMEROUS FAMILIES, THEIR FINANCIAL SITUATIONS HAVE BEEN NEGATIVELY AFFECTED: MANY CAREGIVERS END UP FORGOING OPPORTUNITIES IN BOTH EMPLOYMENT AND EDUCATION. MANY ALSO OPT TO WORK PART-TIME INSTEAD OF FULL-TIME.

"I had to quit my full time job to become my son's primary caretaker and do homeschooling since he was getting sick all the time. This was a loss of income."

-- Parent of a child with Congenital muscular dystrophy

Manuscript under review

THE ECONOMIC IMPACT OF CAREGIVING

Many caregivers are feeling the financial strain that comes with caring for a loved one, which can be even more pronounced when budgets are tight. Whether it's the need to pay for customized supplies, modifications to the home, respite care, or the fact that they must adjust their regular work hours to provide care, today's unpaid family caregivers are absolutely feeling the weight that can come with this work.

There is a growing need for unpaid carers, with the cost of professional care prohibitive (and insufficient) for many.

In our study, caregivers of patients with neuromuscular disorders were found to experience heightened feelings of emotional distress such as anxiety and depression, but also chronic health conditions and pain.

"My child has had onasemnogene abeparvove but had to move to US to access this drug which was costly. We are still in debt and there were a lot of out-of-pocket expenses. For Spinraza, it wasn't too bad as our family lives near Toronto but travel to hospital, parking, and time off work adds up"

"With the near-constant rotation of caregivers, it is difficult to maintain a routine and receive quality care, as it takes a bit to learn. A side effect is the time lost. It often feels as though I have to choose between school, work and a social life, and maintaining my physical health."

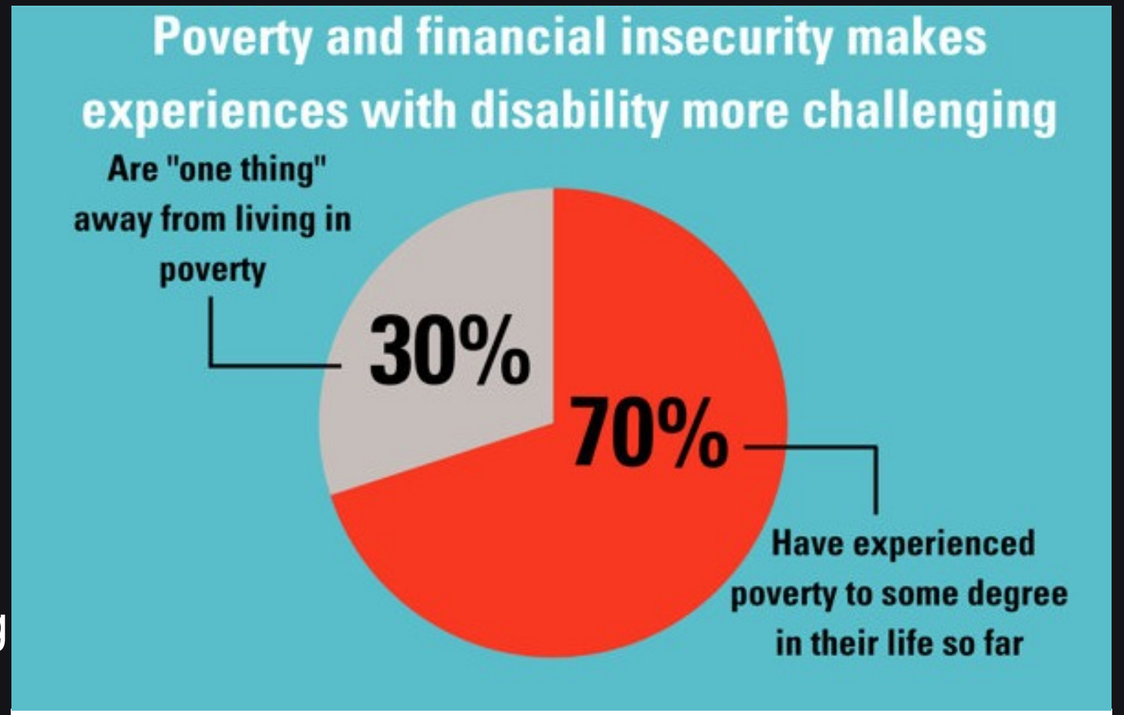
LIVING WITH A NEUROMUSCULAR DISORDER EXACERBATES POVERTY:: AN INTERSECTIONAL LENS

“One of the issues that I've always sort of been faced with and lived with is that as soon as you're earning a certain amount, you are not eligible for anything.”

-- Person with SMA

“The time required to chase money for equipment is substantial. We have been in receipt of much new equipment lately and the amount of time spent searching for funding, applying for funding, and chasing insurance companies for money is hours and hours for each piece.”

-- Parent of a child with DMD



Promised federal disability benefit could make ‘a huge dent’ in poverty rate if done right, say advocates

Bill C-22, which would create a framework for the Canada Disability Benefit, will be a House priority the week of June 12, says Government House Leader Mark Holland.

DISABILITY WITHOUT POVERTY
QUICK CASE STUDY: DEFLAZACORT/CALCORT

KEY MESSAGES

- From a patient's perspective, the actual out-of-pocket and indirect expenses are most critical but they are largely “invisible” in most economic evaluations.

- Although NMDs are rare, individuals affected by NMDs (and other rare disorders) are “high-users” of the system. Patients with NMDs want stabilization of disease - and ‘high cost” drugs might slow down progression of disease and reduce need for direct medical expenses.

- Inclusion of caregiver outcomes and costs of informal caregiving is essential to estimate the true impact of new medical products from the societal perspective, particularly for conditions with a greater demand for caregiving, such as neuromuscular disorders.

- The costs of neuromuscular disorders are likely underestimates: organizations like MDC step in to fill in gaps (e.g., fund equipment); industry partners support through PSPs;

NEED FOR DATA. WHERE DO WE GO FROM HERE?

How do we capture the costs of rare diseases? How do we look at value of health-related quality of life? What information matters most? How can we link data sets? What matters most?

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