Response to the Federal Government on Building a National Strategy for High-Cost Drugs for Rare Diseases

The Federal Government has issued a discussion paper[1] on a national strategy for high-cost drugs for rare diseases and explores three key issues:

1. How to improve patient access to high-cost drugs for rare diseases and ensure that access is consistent across the country.
2. How to ensure decisions on funding high-cost drugs for rare diseases are informed by the best available evidence.
3. How to ensure spending on high-cost drugs for rare diseases does not put pressure on the sustainability of the Canadian health care system.

Muscular Dystrophy Canada (MDC) in partnership with the Neuromuscular Disease Network for Canada (NMD4C) and The Foundation for Gene & Cell Therapy (Jesse's Journey) are pleased to provide the following summary of our recommendations for consideration into the National Strategy for Rare Diseases. As part of the invited consultation with Dr. Hoskins on February 2nd, 2021, it became clear to MDC that many of the challenging experiences of patients and families affected by rare neuromuscular disease, such as Spinal Muscular Atrophy, Duchenne Muscular Dystrophy, Pompe Disease (to name a few), are shared by many Canadians with other rare diseases and that our recommendations are highly consistent with patient organizations, clinician groups, and national organizations for other rare diseases. This highlights the need to **prioritize the unmet needs of Canadians with rare diseases.** We propose the following 8 recommendations that, if implemented, would place Canada in a better position to address the barriers to accessing healthcare for those affected by rare neuromuscular disease.

Recommendation #1: Need for a comprehensive Rare Disease Strategy | We believe the discussion paper is focused on "high cost" drugs. While we acknowledge that money/cost is important, it needs to be seen within the context of chronic, disabling, life-long diseases which untreated also come with both direct and indirect as well as societal costs. Not only should the term "high cost" be removed from the National Strategy, but we should shift our focus from cost of drugs for rare diseases to a more comprehensive plan that includes screening for and prevention of rare diseases, improved diagnostic procedures and early intervention, improved coordination of care, clinical experts and data to inform decisions, research into rare diseases (e.g. creating disease registries and encouraging clinical studies), and collaboration with drug developers to ultimately improve cost-effective access to drugs for rare diseases.

Recommendation #2: Revisit the Ontario Rare Disease Working Group Report | In 2017, Dr. Ronald Cohn, neuromuscular specialist and CEO of SickKids, chaired a working group on Rare Diseases[2] and noted *"I believe this report will provide a blueprint towards a rare disease strategy, not only for Ontario, but will also serve as a catalyst to leverage efforts in rare disease collaborations on a national level. I am excited to now work with colleagues and patient advocates to address the identified recommendations which will undoubtedly improve our diagnosis and management of patients living with rare diseases in Ontario."* The report outlined 5 key priorities: diagnosis capacity and early detection; timely access to equitable and evidence-informed care, including supports to primary care providers in identifying and managing rare diseases; access to complex care and rehabilitation; access to clinical trials and potential promising therapies; promoting innovative research. While the current framework for evaluating drugs for rare diseases in Ontario is far from perfect, it includes a detailed understanding of the disease and the potential

value of the drug for rare disease, reviewing the assessment with disease experts and the public and, re-assessment as new information becomes available about the disease and the benefits.

Recommendation #3: Put patients and families at the forefront | Undeniably, patient and clinician engagement must be improved to increase awareness of policies and programs related to drugs for rare diseases. Decisions should not be made in silos: clinicians, and patient/patient organizations with intimate experience of the condition must have input and a vote into the decision-making process[3]. In addition to meaningful involvement of patients and clinicians working in the relevant field of practice, we ask that you immediately implement an expert panel including the patient voice as part of the decision-making process. An expert advisory committee consisting of clinicians and patients with experience in rare disease should be appointed to oversee, implement and evaluate the Canadian Rare Disease Strategy. The committee should work with Canadian Rare Clinical Networks. *(Including physicians, allied healthcare professionals, caregivers and patients).*

Recommendation #4: Quality of life | Where evidence is lacking for new treatments, because of practical or methodological difficulties, there is a risk that patients remain unable to access cost-effective care. What is considered a benefit or meaningful to the patient needs to be included. Patient reported outcomes and preference data (i.e. what constitutes a benefit versus what risks they willing to take on), needs to be integrated/considered alongside clinical data is accepted. To demand large- scale clinical trials for rare neuromuscular diseases is often not feasible. It could take years to get a large sample size to gather enough objective evidence and pharmaceutical companies do not conduct such trials. Benefit from drugs for rare diseases are, at times, based on patient-reported quality-of-life benefits, which are inevitably subjective and at times subtle, but meaningful (i.e., being able to maintain ability to use fingers offers independence). In addition, HTAs should include impact on quality of life for family members/caregivers, societal costs, and loss of productivity. Sampson and Garau (2019) suggested bespoke data collection for improving the quantity and quality of data available to inform decision makers in the context of rare diseases: condition-specific health- related quality of life instruments in the context of progressive disease should be validated and used to account for the impact of knowledge about the future. For example, in SMA, that might include explicit identification of the expected time to losing the ability to walk, to go to the toilet independently, or to breathe without support. The impact of these expectations on quality of life can be identified using questions about domains of psychological wellbeing, such as hope.

Recommendation #5: Decrease barriers for clinical trials | The federal government should encourage and incentivize clinical trials in Canada and decrease barriers to being competitive on a global stage. Additionally, the federal government should amend regulatory approval pathways for drugs for rare diseases and incentivize manufacturers in order to bring these drugs to Canada. If we were to take Duchenne muscular dystrophy (DMD) as a case example: the drug development pipeline is rich for DMD with over 35 molecules currently under investigation at various stages of development. DMD drug manufacturers are reluctant to apply for approval in Canada because our process is complex. While there is concern that high-cost drugs for rare diseases can put pressure on the sustainability of the Canadian health care system, this is not true. Currently there are no drugs approved for Duchenne, but the costs of living with DMD have the most impact on our health care system. Boys and men affected by DMD lose ambulation in childhood and lose their ability to live independently as they age. This happens at an age when they should be developing their independence, starting their entry into adulthood, making positive contributions to the work force, community, and country. Instead, they are requiring more support, in some cases accumulating substantial costs, from their caregivers (usually a parent) to complete

daily functions like dressing, feeding, bath rooming, and costs to adapting their environment so they can physically get around. Families are burdened with multiple medical appointments and as the disease progresses medical interventions are required to address bone fractures, scoliosis, heart monitoring, breathing support and feeding support. In addition, a family member is required to withdraw from the workforce and community activities to care for their loved one(s). All these costs/impact must be taken into consideration when assessing costs for medications and when providing incentives to drug developers to launch innovative therapies in Canada.

Recommendation #6: Real-world evidence | The federal government should fund and support Rare Disease Registries appropriate for gathering Real World Evidence. We ask that if a medication is approved by Health Canada, there should be a data collection infrastructure that can collect Real World Evidence during this period to support decision-making and future use of the medication. These data can inform understanding of drugs for rare diseases impact and allow for outcomes-based contracting, if required. The data collection and analysis should be at arms length with the pharmaceutical industry. Governments/payers should ensure that a framework exists to allow such data collection by the health care providers and patients (and ensure the financing of it). Furthermore, Canada should participate in independent national and international networks to build partnerships to facilitate knowledge and data sharing on real-world experience of patients.

Recommendation #7: Access no matter where you reside | Access to new therapies varies significantly depending on where patients live in Canada and how their drugs are covered because public and private drug plans make separate, often different, decisions about what drugs for rare diseases they will cover. Even though Canada has a universal health-care system, the responsibilities of administering health care lies with provinces and territories governments and coverage of the medication and medical care varies across the country. This adds a layer of complexity in developing a National Strategy, which includes prescription drugs, as there is no consistency of access to drugs across Canada even with CADTH and INESSS, a transparent coordinating body. Strong leadership, collaboration and political will is required to move this Strategy forward. There needs to be clear and simple with a transparent co-ordinating governing body that has the authority and means to enforce a decision. When Health Canada decides a drug is safe and has benefit for Canadians, it should be reimbursed at the same time, so all patients regardless of where they live in Canada can access the medication immediately. This is the case in other countries like Germany, where at the end of the day these countries do not pay more for the drugs for rare disease and are further ahead in drug development (which offers economic benefit).

Recommendation #8: PMPRB remains problematic | Health Canada should reconsider the impact that proposed changes to PMPRB regulations will have on access to drugs for rare diseases. PMPRB should not apply for Drugs for Rare Disease, similar to COVID therapeutics.

In closing, we would like to acknowledge that while the costs of individual treatment for Canadians with rare diseases may be greater than other publicly funded therapies, such that 8% of the population has a rare disease, and up to 20% of the total health care spending is used for rare diseases in developed economies, the valued benefits of the federal government stimulating a pan-Canadian strategy for rare diseases that includes changes for regulation and funding of drugs for rare diseases cannot be overstated. We thank you for the opportunity to provide our recommendations and we are very eager and keen to be involved with the development of this Strategy. For now, we leave you with this: the federal government should take on a sense of urgency where the patient need is at the forefront and cornerstone of the strategy and long-term

sustainable solutions must be determined in a prioritized manner. For our community, *time is muscle and life*.

Please do take our recommendations into consideration as you prepare the Rare Disease Strategy and we would absolutely be happy to answer any questions you might have. For any opportunities for further discussion and consultation, please contact Dr. Homira Osman, PhD [homira.osman@muscle.ca].

Kind Regards,

Stacey Lintern
Interim CEO, **Muscular Dystrophy Canada**

Nicola Worsfold
Director, Research & Advocacy, **Jesse's Journey**

Dr. Hanns Lochmuller, MD
Principal Investigator, **Neuromuscular Disease Network for Canada**

References

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