DEFEAT DUCHENNE. CHANGE LIVES.

Why Does Canada Need a Rare Disease Strategy?
Perspective from the Duchenne community

Duchenne muscular dystrophy is a genetic, rare, progressive muscle-wasting condition. It affects 1 in every 5,000 male births and a child is typically diagnosed between the ages of 3-6.

Children with this disorder weaken as they age, require a wheelchair by their mid-teens or in some cases much sooner, and eventually lose their battle to this devastating condition by their 30's due to lung or heart failure.

There are currently no treatments for Duchenne patients in Canada, and care is solely focused on disease management. There are, however, five treatments approved in the USA and Europe. The first of these treatments was approved in 2014 in Europe, and in 2016 families could access this medication in the UK through a managed access agreement. Six years later, Canadian families are still waiting.

It is heartbreaking to watch your child's health decline, knowing there are other children able to access medications in their respective countries. If families are lucky enough, they might qualify and be willing to travel thousands of miles to enroll in a clinical trial. But with clinical trials comes risks. The medication might not work or could have an unknown side effect. But these are the risks many Duchenne families are willing to take if it means there is some ounce of hope of slowing the progression or maintaining some level of function so they can continue walking, eating independently, and living longer.

Health Canada's Special Access Program (SAP) is another mechanism a physician can use to access a medication not yet approved for Duchenne families. However, this program is meant for urgent acute care, not an ongoing stop-gap as we wait for medications to come to Canada. In some cases, it also requires a hefty out of pocket payment for families who are already burdened with high expenses as they adapt their environment to accommodate a child who will or can no longer walk or live independently.

The challenge of accessing affordable treatments leads to increased morbidity, loss of life or lower quality of life, loss of parent/caregiver employment and income, and increased costs to our families, the health care system, and the Canadian economy. And the frustration for Canadian donors and charities like Jesse's Journey, who invest heavily into research, only to be left waiting while our Duchenne families watch their children's health deteriorate.

But research is bringing hope to the Duchenne community. As of October 2020, we have a large pipeline with 35 different molecules or biologics under investigation. We have 38 active industry-sponsored clinical trials globally, ranging from Phase I - III by 16 various industry sponsors.

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Now is the time to act and make a difference to help improve the lives of Canadian Duchenne families.

This will take collaboration and coordination amongst all levels of government, patients, healthcare professionals, researchers, and industry partners to develop and implement a regulatory framework for specific orphan drugs.

Our recommendations on areas of focus:

- Building upon what has been adopted in other jurisdictions, develop different ways to design clinical trials and collect data to support new drug submissions and reimbursement. Provide resourcing and incentives to promote data generation initiatives.
- Formalize a role for patient/patient organizations to provide meaningful input into all aspects from drug discovery, to clinical trial development, to drug approval and reimbursement. And provide our Canadian patient organizations with the appropriate resourcing to help them do this.
- Leverage resources and engage in parallel reviews with the first to market jurisdictions such as the FDA (USA) and/or EMA (Europe) to cut down redundancy, lengthy wait times, and review times.
- Develop a dedicated funding approach for rare disease drugs and develop innovative mechanisms like managed access programs that allow for equitable and timely access.

For more information about Jesse's Journey and our contribution towards a Canadian rare disease strategy, please contact:

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