DEFEAT DUCHENNE. CHANGE LIVES.

March 5th, 2021

Manager, Office of Paediatrics and Patient Involvement
Centre for Regulatory Excellence, Statistics and Trials (CREST)
Health Products and Food Branch
Health Canada / Government of Canada

Dear Dr. Alysha Croker and ICH Coordinator,

On behalf of Canada's Duchenne muscular dystrophy (DMD) community Jesse's Journey, Canada's leading DMD patient advocacy organisation, would like to thank Health Canada and the Office of Paediatrics and Patient Involvement for the opportunity to contribute feedback on the International Council for Harmonisation (ICH) consultation process. Our coalition of patients and caregivers wholeheartedly agree the patient's perspective can only help improve outcomes of drug development. It's also our belief the most efficient and effective way to ensure success is under a globally harmonized framework.

Feedback of specific areas of the ICH Reflection Paper and ICH guidance are attached in the excel document provided. The following information provides additional feedback of our position on behalf of Canada's Duchenne community.

Incorporation of patient perspective into Drug Development

Jesses' Journey supports and advocates for the inclusion of patient perspectives at all stages of drug development and approval.

Patients and their caregivers (typically a parent in the case of childhood conditions) are the experts on their disease and have direct experience on what it means to live with their condition. They also happen to be the end users for medications being developed, so who better to advise on drug development than the patients themselves.

The reason patient advocacy and patient engagement is growing across rare disease is because this is an area of high unmet need. Our Duchenne patient community for example have yet to be approached by regulators despite several sponsors engaging Health Canada on their drug development plans and new drug submissions. It is therefore left up to the patient organizations that are knowledgeable about the processes to be proactive and find innovative ways to approach our regulators. If this patient engagement into the drug development process was formalized as suggested in this reflection paper, you would eliminate the burden on patient

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groups, allow for smaller less organized groups to provide their input, and ultimately standardize the collection of patient data.

Potential benefits

- Adopting and standardizing methods for formalized patient input will ensure patients living with a disease are involved in the scientific discussions, help bridge the gap between clinical trial data and real-world data, increase transparency and trust, and increase understanding and quality of patient reported outcomes.
- Different ways patient input can bring beneficial perspective to drug development that were not mentioned in the paper:
    - ensure appropriate endpoints are being utilized
    - help define a target population (inclusion/exclusion criteria)
    - inform sponsors on appropriate comparators
    - bring real life practical input to study duration, treatment administration, formulation, and dosage
    - can inform on clinical relevance versus statistical significance
    - bring light to significant benefits (added value) over existing therapies
    - inform on ethical aspects including informed consent

Potential challenges

- Developing a methodologically-sound data collection tool that is widely accepted and highly accounted with regulatory reviews is critical. But to ensure these methods are adopted it will be important to work in collaboration with all stakeholders (including sponsors, clinicians, regulators, and patient groups) in the development of these tools. It will also be critical to ensure you have the right experts for each condition and medication with a wholistic understanding of the condition. In some instances, this may be challenging to find in more ultra rare diseases where there may not be an organized group to represent.
- One size does not fit all, and it will be important to ensure data collection and tools are aligned to the needs of each condition and medication the tool is intended for. Even within a condition like Duchenne there is variability in disease progression and patient preferences, any questionnaires or tools will need to be adapted on a case-by-case basis.
- Developing a standardized outcome measures can be challenging. Even within the Duchenne international community there is much work needed in this area. Collaborate with international networks and groups will be imperative as this work progresses. Evaluation of outcomes by both Canadian patients and clinicians will be important to ensure these are appropriate and feasible methods to adopt. For example, EQ-5D may be a measure widely used in the EU but may not be widely utilized in Canada.

- We need to make sure the data we are collecting is of scientific rigor, a consensus on what data is collected, and how it is acquired. All data should fall under the FAIR principles (Fundable, Accessible, Interoperable, and Reusable).

Potential barriers

- Leveraging data collection through disease specific registries that already exist will be important and an easy way to obtain data already being collected. Some patient organizations may not have the capacity or resources to collect data and may need additional resourcing to do this.
- Some conditions, especially with rare diseases, may not have well organized patient organizations knowledgeable enough about all aspects of drug development or appropriate/available outcome measures to advise. In this case you may need to rely on individual patient preferences and disease experience that may not reflect the opinions and needs of the entire population effected. This will be important to consider when obtaining this input and every effort should be made to gather input from a collection of individuals and clinicians.
- Additional potential barriers/unanswered questions:
    - Who is collecting the data points and are the regulatory agencies taking the lead?
    - How will this data be collected?
    - Will there be funding/resourcing provided to patient organizations to aid in data collection in their communities or will this be left to the sponsors to manage/absorb cost?
    - This initiative would fall nicely under a rare disease framework however, Canada remains without a national rare disease strategy. As Health Canada works toward development of a rare disease strategy it would be helpful to know how this will align and how it will be incorporated?

Potential impacts should the paper's approach / guidance be adopted by Canada

Our view is incorporation of the patient perspective must start now. Yet we worry whether implementation/execution of any guidance will be adopted by Canada absent a national rare disease strategy. Our view is patient focused drug development would almost certainly fall within a strategic priority of a robust national plan. Without it, is there a risk implementation may be delayed or fails? As advocates of a community in a race against time we remain excited by the consideration to include the patient perspective in drug development.

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How we see the Reflection Paper being implemented in Canada, if pursued by ICH.
These recommendations put forth in this reflection paper would fit very nicely into a rare disease strategy for Canada. However, given we are not aware how soon a federal rare disease strategy will be implemented, we would like to see many of these methods incorporated into the current processes. This is an important initiative for Canadians and should be considered regardless of the decision from the ICH.

We thank you for taking our point of view into consideration. We're encouraged by the intent of the ICH reflection paper and are hopeful to see the patient voice prioritized here in Canada. I want to reiterate as a national patient advocacy organization we want to work in collaboration with Health Canada to ensure our patient perspective is considered in a helpful, meaningful, and productive way that will benefit Canadians living with disease.

Sincerely,

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