March 1, 2024

The Hon. Mark Holland
Ministry of Health
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The Hon. François-Philippe Champagne
Minister of Innovation, Science and Industry
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RE: Amendment to Schedule 1 of the Patent Act - Inclusion of Risdiplam

Dear Honourable Ministers,

I am writing on behalf of Knowledge Ecology International to bring to your attention a matter of importance to the health of individuals globally. We respectfully request the expeditious consideration of the addition of risdiplam, a drug for the treatment of spinal muscular atrophy, to Schedule 1 of the Patent Act.

Schedule 1 is a list of patented pharmaceutical products eligible for export under the Canadian Access to Medicines Regime (CAMR). A product is eligible to be added to Schedule 1 if it is a "patented product that may be used to address public health problems afflicting many developing and least-developed countries, especially those resulting from HIV/AIDS, tuberculosis, malaria and other epidemics" (s. 21.03 *Patent Act*).

Spinal muscular atrophy (SMA) is a hereditary genetic disease caused by a defect or mutation in the SMN1 gene. An estimated 2 percent of the population are considered carriers. SMA Type 1 is considered the most aggressive type of SMA and is the leading genetic cause of death in early infancy.

Three treatments for SMA that have been approved by Health Canada and regulatory agencies abroad, Zolgensma (onasemnogene abeparvovec), Spinraza (nusinersen), and Evrysdi (Risdiplam). Despite receiving substantial financial support for their development from governments and charities, all three of these drugs are marked by excessive prices. This has led to unequal access worldwide, with even some high-income countries unable to afford the excessive cost. In Canada, for instance, coverage is not widespread, with provinces providing varying degrees of coverage, ranging from none at all to exceptional cases, or specific patients under certain age criteria.

What makes risdiplam a particularly important drug is that, at present, it provides the best chance to make an affordable generic available. Not only is the drug easier to manufacture than Spinraza (nusinersen) or

the gene therapy Zolgensma (onasemnogene abeparvovec), but it does not require the patient to go to hospital for treatment, reducing the time and financial burden on caregivers and patients. Additionally, risdiplam is the first and only oral treatment for SMA, making it the least intrusive treatment for patients. Given the global challenges in access to SMA treatments, coupled with the unique benefits of risdiplam, including its ease of generic manufacturing, make it a compelling and necessary addition to Schedule 1. A step that is crucial to the well-being of families and individuals grappling with the disease.

We therefore request a timely response regarding the following actions:

- A meeting to discuss the issue and discuss next steps; and
- The Minister of Health and Minister of Innovation, Science and Industry to promptly offer their recommendation for the addition of risdiplam to Schedule 1; and
- The Governor in Council to issue an order to amend Schedule 1 within the next 60 days.

The shortcomings of CAMR during the COVID-19 pandemic underscore the need to make CAMR adaptable and effective. By including risdiplam, Schedule 1 can be poised to meet the needs of patients worldwide and provide the opportunity for the Government of Canada to demonstrate decisive leadership in ensuring affordable access to medicines for rare diseases. We believe this step will not only address the current gaps in access but also position Canada as a leader in providing affordable and equitable access to life-changing medications, starting with risdiplam.

We look forward to your consideration of this request. We are open to further discussion and would welcome the opportunity to speak with you.

Yours sincerely,

Arianna Schouten

Arianna Schouten
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