Canadian Paediatric Society / Société canadienne de pédiatrie

Hon. Mark Holland
Minister of Health
House of Commons
Ottawa, ON K1A 0A6

January 18, 2024

Subject: Advocacy for Enhanced Drug Regulation and Access in Paediatric Populations and Support for the Canadian Drug Agency

Dear Minister Holland,

On behalf of the Canadian Paediatric Society (CPS), the CPS Drug Therapy Committee, and the CPS Paediatric Drugs and Therapeutics Taskforce, we are writing to express our strong support for your commitment to improve access to safe, effective, and affordable medicines in Canada through the establishment of the Canadian Drug Agency (CDA). This marks a pivotal advancement for Canada, and we are particularly encouraged by the potential positive impacts that can be realized in paediatric medicine from this investment.

Access to safe, effective, and affordable medications is essential to child and youth health and a core priority for the CPS. To address this critical need, we established the Paediatric Drugs and Therapeutics Taskforce in 2019. In collaboration with 16 national child and youth health organizations, the focus of our collective work aligns with the CDA's objectives to enhance the pharmaceutical system and adopt data-driven decision-making with an eye to optimize both individual and health system-level outcomes.

Recognizing the alignment between our interests and objectives, as the CDA moves forward, we urge the following critical points be considered:

1. **Enhancement of the Drug Approval Process**: We advocate for a rational, streamlined, and transparent drug approval process that deliberately considers both our small market size and the priorities of vulnerable sub-populations within Canada, including paediatrics, in its design. At present, Canada has a dysfunctional dependence on off-label prescribing for paediatric patients, and an inefficient reliance on our national Special Access Program for essential medications for infants, children, and youth. Access to medicines to treat rare and life-threatening childhood illnesses, and access to safe and effective child-friendly formulations, both lag behind comparator jurisdictions, and there are no effective policies or incentives in place to attract manufacturers of paediatric products to the Canadian market. To ensure that Canadian children have access to safe, effective, on-label, life-saving medications, the drug approval process must specifically attend to their unique needs.

2. **Advance Paediatric-Specific Regulatory Reforms:** Following long-standing international best practices, Canada should mandate the submission of paediatric data for drug approvals where paediatric use is expected or anticipated. Consideration of paediatric use should align with both adult disease-based indications and indication-agnostic molecular targets (that may be specific

to paediatric disease). Additionally, the development and submission of paediatric-friendly dosage forms should be required to ensure safe and effective administration of approved medications for all paediatric patients.

3. **Use of Foreign Reviews and Trusted Foreign Decisions**: We strongly recommend revising Health Canada's Use of Foreign Reviews guidance to include reliance on decisions from trusted foreign jurisdictions. This should include not only review of data for new products, but also expanded indications for products already on the Canadian market. This approach will streamline the approval process for paediatric medications and formulations and start the process of "right-sizing" labels for evidence-based paediatric use. New, powerful and attractive policies leveraging the experience of trusted foreign jurisdictions are essential to reduce the significant barriers to market entry for essential paediatric medications.

4. **Health Technology Assessments for Paediatric Populations, Paediatric-Specific Indications and Formulations**: Approval of new medications and formulations must thoughtfully align with paediatric-specific health technology assessments (HTAs). Formal paediatric HTAs must be de-coupled from Health Canada-approved indications until Canada's regulatory framework mandates submission of paediatric data and guarantees appropriate consideration of paediatric use. All value assessments must account for patient age and other patient-group specific attributes, and the incremental safety benefit of child-friendly formulations must be considered. Collaboration across Health Canada, the CDA, pricing authorities, and public formulary managers will be vital to ensure evidence-based public coverage of appropriately priced evidence-based paediatric medications and child-friendly formulations.

5. **Protection Against Unreasonable Price Increases**: In parallel with the expansion of child-friendly HTAs, measures to prevent drastic price hikes in essential paediatric medications and child-friendly formulations are vital. Recent examples of new child-friendly formations entering the Canadian market at extreme price points (including liquid suspensions of glycopyrrolate, propranolol and levetiracetam) proved to disrupt, rather than enhance, access to safe and effective paediatric medications.

6. **Data-Driven Decision Making**: Robust data collection and analytics in paediatric pharmacology are critical for informed healthcare decisions and policymaking. Understanding the nuances of applying adult data to paediatric populations, and the specific challenges associated with orphan patient groups, including infants and children, is critical. Robust paediatric expertise must be present at all CDA tables, and a dedicated paediatric expert advisory committee should be formed to ensure paediatric considerations are integrated into all relevant decision-making.

7. **Robust Investment in Paediatric Drug Research**: In alignment with the advocacy of the Maternal Infant Child and Youth Research Network (MICYRN), we call for substantial investment in paediatric drug research and research infrastructure.

As the national association of paediatricians, the CPS is committed to working collaboratively with the CDA and our healthcare partners to implement the above recommendations and strengthen the pharmaceutical system in Canada for children, youth, and their families. We believe that our expertise, experience and longstanding dedication to child and youth health will greatly benefit the CDA, as the organization moves forward its ambitious mandate. We would greatly appreciate the opportunity to meet to discuss the above recommendations and the robust evidence-base behind them in greater

detail. We appreciate your consideration of this request and look forward to a productive partnership with the CDA, and to contributing to promising positive advancements in paediatric healthcare.

Sincerely,

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