POSTER #51

An Urgent Need for Canada to Have Access to Commercial Pediatric Formulations: Children Deserve the Same Standards as Adults

Andrea Gilpin[1], Sophie Bérubé[1], Jean-Marie Leclerc[1,2], Catherine Litalien[1,2]

[1]The Rosalind & Morris Goodman Family Pediatric Formulations Centre of the CHU Sainte-Justine,
[2]Department of Pediatrics, CHU Sainte-Justine, Montréal, Québec Canada

Introduction

- Canada lags behind the G7 when it comes to availability and access to suitable pediatric formulations due to several reasons including limited commercial opportunity and a complex regulatory system that has little accommodation for pediatric submissions.
- Manipulation of dosage forms designed for adults by healthcare providers and parents (compounding) falls outside of the Federal regulatory approval process, resulting in off-label use.
- The GFPC is a not-for-profit organization dedicated to promoting the development and access to child-friendly medicines to improve safety and efficacy in pediatrics. The GPFC has established a priority list of drugs currently compounded that are in need of commercial pediatric formulations in Canada.

Objectives

- To provide an overview of the ongoing advocacy efforts for regulatory and reimbursement changes in Canada to stimulate pediatric submissions.
- To facilitate access to pediatric formulations currently approved in the UK and/or Australia, given their similar healthcare systems to the Canadian one (Table 1).

Contributing to Improving Access to Pediatric Drugs

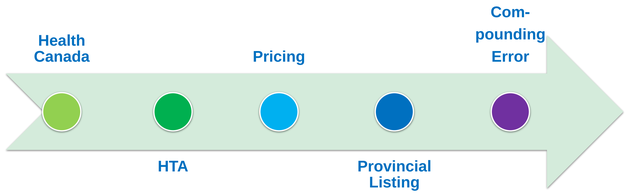
The Canadian healthcare system is managed at both federal and provincial levels and therefore any legislative changes to include pediatric-sensitive parameters should include both levels of government. The GPFC has increased awareness of the changes needed in pediatric drug approvals by using the following approaches:

- Actively participating in discussions/consultations on policies regarding drug approval process in Canada including: Proposed Health Canada Fees Structure, Regulatory Modernization, Trusted Foreign Review and Accelerated Review representing a pediatric lens.
- Meeting with key senior officials and politicians to discuss specific cases and identifying hurdles to regulatory approval and market access (Figure 1).
- Galvanizing the pediatric community to advocate together and to align on messaging to improve access to pediatric formulations and drugs.
- Accompanying pharmaceutical companies to Health Canada consultation meetings for drugs on the GPFC priority list to facilitate the process.

Table 1: Priority list of the GPFC

Drug	Availability of pediatric formulation	Pediatric indication in Canada
Lamotrigine	UK/Australia	Yes
Metronidazole	UK/Australia	Yes
Rifampicine	UK/Australia	Yes
6-Mercaptopurine	UK/Australia	Yes
Phytonadione	UK/Australia	Yes
Tacrolimus	UK/US	Yes
Ursodiol	UK/Australia	No
Caffeine	UK/Australia	No
Captopril	UK/Australia	No
Nitrofurantoin	UK	Yes
Dexamethasone	UK	Yes
Baclofen	UK	Yes
Amitriptylin	UK	Yes
Folic acid	UK	Yes
Hydrocortisone	UK (EU)	Yes
Topiramate	UK	Yes
Hydroxyurea	UK(EU)/US	No

Figure 1: Challenges at Every Junction of the Drug Approval and Market Access Process



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Advocacy of Improvements to be Made to Increase Access to Pediatric Formulations in Canada

Pediatric-specific regulatory submission fees and accelerated review

- Regulatory submissions for new indications or new formulations can be submitted using a policy relying on third-party data. Although an abbreviated submission, the fees for submission and review time are the same as those for a full regulatory submission. There is a need for a lower fee structure and an accelerated review for the submission of pediatric formulations.

Alignment of the entire process from drug approval to market access

- A new formulation for infantile hemangioma gained regulatory approval from Health Canada yet reimbursement agencies did not initially support the proposed price based on their health economic evaluation and perceived value of the medication.

Budget impact analysis using a more appropriate price as the comparator

- Oftentimes, a pediatric formulation is not available in Canada leaving the pharmacist or caregiver to rely on compounding.
- When evaluating the value of a pediatric commercial formulation for a drug currently for use in children, the price comparator used is the compounded medication which is much cheaper that the commercialized form.
- Compounded medications do not follow the rigorous Health Canada submission requirements nor can they have the same manufacture standards as industry.

Alignment between Canadian provinces for reimbursement

- Not all provinces have the same standards for public listing of medications.
- The result is that manufacturers sometimes have to negotiate different terms in various provinces or even some provinces may require additional data.

Decrease the need to rely on compounding, which is not without risk

- A liver transplant recipient child developed organ rejection as his tacrolimus levels were barely detectable and lack of adherence by the parent was suspected
- Tacrolimus compounded medication was found to be 1/10 of the expected concentration, an error made when the compounding was performed
- The risk of errors is real and can have a significant detrimental impact the treatment success

Summary

- Advocating and aligning messages with the pediatric community have provided a unified voice to key decision-makers and change is underway
- Draft guidance for Accelerated Review, Trusted Foreign Review and the proposed National Pharmacare program all now mention pediatrics in their documentation
- We are seeking to collaborate with manufacturers to commercialize the products in Table 1 and to support the approach in Canada
- Together we can improve access to pediatric drugs for Canadian children