Health Canada's Pediatric Drug Action Plan

Alysha Croker, PhD
Health Products and Food Branch

Pediatric Drug Access in Canada Development – Challenges

- **Lack of authorized medicines for children**
  - 50-80% of medicines are prescribed off-label to children
  - Drug compounding is cheaper than reimbursing authorized pediatric formulations

- **Few incentives for stakeholders**
  - Comparatively small pediatric population means smaller market
- **Difficulties in conducting clinical trials in children**
  - Additional ethical and safety concerns
  - Parental consent and agreement (assent) from child
  - Logistical barriers to enrollment in pediatric trials (especially for rare diseases)
  - Insufficient infrastructure for conducting pediatric clinical trials

Pediatric Drug Action Plan

Vision: Children and youth (aged 0-17) in Canada have access to the medicines they need in age-appropriate formulations

We propose to work across Health Canada, other governmental departments, and with our external (national and international) partners to accomplish the following three goals:

1. Increase the **development** of essential pediatric medicines and formulations
2. Improve **access** to pediatric medicines and formulations
3. Provide more information to people in Canada

Key Priorities (2023-2026)

- Implement a policy-based **Pediatric Pilot** that will ask sponsors to provide pediatric plans with certain submissions for adult medicines that are expected to be used in the pediatric population in Canada
- Develop a **National Priority List** of urgently needed medicines for children that need to come to Canada
- Explore **mechanisms** to facilitate access to certain pediatric drugs

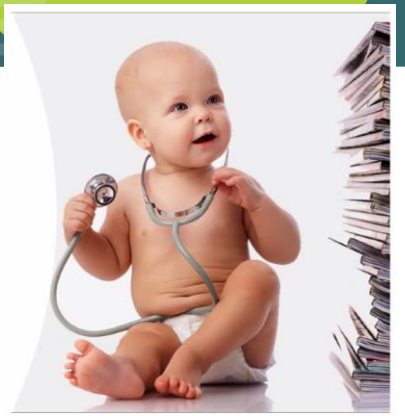
Pediatric Legislation / Regulation Internationally

Requirement to submit pediatric study results	When seeking an adult indication, pediatric study results must also be submitted to address use of the drug in the entire pediatric population (0-17 years)
Option to defer pediatric requirement by submitting a pediatric plan	In place of pediatric study results, sponsors can defer the requirement by providing a **pediatric plan,** which details the approach to generating pediatric data and provides a timeline for study completion
Option to waive pediatric requirement by including a waiver in the pediatric plan	In specific circumstances, the **pediatric plan can include a waiver** request, which provides a rationale for why studies are not advisable for all or part of the pediatric population
Requirement to submit completed study results to regulator	The results of the studies described in the pediatric plan must be submitted to the regulator once they are complete

Pediatric Regulations – differences between regulators

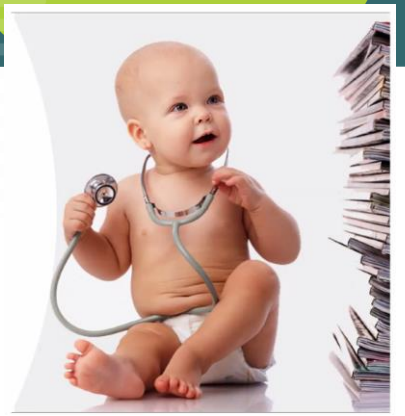
	INDEPENDENT pediatric plan review model	RELIANCE pediatric plan review model
Regulators	FDA (2003), EMA (2007), MHRA (after Brexit)	Swissmedic (2016)
Pediatric Plan submission	During adult drug development program	Only when the drug submission is made
Pediatric Plan format & content	Regulator-specific	Accept pediatric plans approved by the FDA or EMA. If no foreign plan exists, a sponsor must submit a Swissmedic-specific pediatric plan, which will be fully reviewed
Pediatric Plan review	The FDA and EMA have teams of experts who conduct thorough reviews and revisions of the pediatric plans prior to their approval	The foreign-approved plan is verified by Swissmedic • Confirm that it aligns with Swiss requirements • Can seek clarification on parts of the foreign approved plan • No changes to the foreign-approved plan are sought

Overview of the Pediatric Pilot



- The Pediatric Pilot launched on February 26th, 2024.
- Objectives of the Pediatric Pilot:
  - encourage sponsors to submit safety and efficacy information for drugs expected to be used in pediatric populations in a timely manner;
  - provide more information on the safety, efficacy and dosage of drugs used in pediatric populations to health practitioners, patients and the families of patients;
  - collect data to better understand the pediatric drug landscape in Canada; and
  - inform future policy and regulatory initiatives.
- Participation in the pilot is voluntary. All sponsors with in-scope submissions are encouraged to participate.
- Sponsors with in-scope submissions are asked to complete a **short survey** and include it at the time of filing their submission, even if they do not wish to participate in the pilot.

Overview of the Pediatric Pilot



- The policy behind Health Canada's pediatric pilot aligns with international approaches
- The pilot applies to all new drug submissions (NDS) and supplements to a new drug submission (SNDS) for any:
  - new indication
  - new dosage form
  - new route of administration
- Participation in the pilot will not affect the outcome or timeline of the (S)NDS review
- The policy is based on a “Reliance” review model
- Sponsors who wish to participate in the pilot may submit any of the following documents:
  - an approved EU-PIP (including waivers)
  - an agreed iPSP (including waivers)
  - a Canadian-specific pediatric development plan (C-PDP)

Overview of the Pediatric Pilot

- Under the “Reliance” model, Health Canada’s verification of an **EU-PIP or iPSP** entails:
  - confirmation that the foreign-approved plan contains Canadian guidance-recommended content
  - possibility for Health Canada to seek clarification on parts of the plans
  - adoption of the foreign-approved plan by Health Canada, without making changes to it
- If the foreign plan does not meet Canadian content recommendations, the sponsor may withdraw from the pilot, or submit a **Canadian-specific plan** in place of the foreign plan.
  - Canada-specific plans will be fully reviewed by Health Canada
- While Health Canada encourages sponsors to submit finalised studies from their approved plans, participation in the pilot does not create an obligation to do so.
- Health Canada's adoption of a pediatric plan does not indicate future approval of a pediatric indication or concurrence with a decision by the FDA or EMA on the results of the studies described in the plan.

For more information about the pediatric pilot, please visit:

- Pilot on pediatric development plans and studies
  - https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/pediatrics/pilot-development-plans-studies.html
  - https://www.canada.ca/fr/sante-canada/services/medicaments-produits-sante/medicaments/pediatriques/pilote-plans-developpement-etudes.html
- Guidance on submitting pediatric development plans and pediatric studies
  - https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/pediatrics/guidance-submitting-development-plans-studies.html
  - https://www.canada.ca/fr/sante-canada/services/medicaments-produits-sante/medicaments/pediatriques/lignes-directrices-presentation-plans-developpement-etudes.html

National Priority List of Pediatric Drugs

- The objective of the **National Priority List of Pediatric Drugs (NPLPD)** is to identify drugs that address areas of unmet pediatric need in Canada, and to help facilitate their access for pediatric patients.
- A targeted 60-day nomination process was held with the pediatric medical community in early spring 2023.
- In order to be eligible for consideration on the NPLPD, the nomination had to:
  - address an area of high unmet need in Canada;
  - be approved in a select foreign jurisdiction; **and**
  - not be currently approved for sale in Canada in the proposed indication or formulation, or both.

National Priority List of Pediatric Drugs

- In total, Health Canada received **over 900 nominations** through the nomination submission process (with over 300 unique drugs being nominated).
- All therapeutic areas were covered, but most nominations were in the areas of infectious diseases, neurology, hematology and oncology, and cardiovascular diseases.
- A Pediatric External Reference Group (PERG) made up of experts from the pediatric medical and pharmacist communities from across Canada provided expert advice and recommendations on the draft priority list.
  - 119 drugs were screened-in and referred to the PERG for prioritization
  - 42 drugs were selected by Health Canada for consultation, based on PERG's prioritized list

NPLPD: Consultation – How to Participate (Public)

- On October 21, 2024, Health Canada launched a 60-day consultation seeking public feedback on the draft NPLPD.
- The consultation provides people in Canada with an opportunity to comment on the initiative and the proposed priority list, as well nominate additional drugs for consideration
  - Main consultation page: https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/pediatrics/about-national-priority-list-pediatric-drugs.html
- The consultation is open for 60 days and provides interested Canadians with an opportunity to submit feedback on the initiative and the proposed list of drugs.

NPLPD: Consultation – How to Participate (Industry)

- Concurrent to this consultation, Health Canada is launching targeted consultations with industry, as well as with health system partners, to obtain feedback on the priority list. An online questionnaire has been shared with industry and includes questions such as:
  - Are there any drugs on the list for which you have market authorisation issued by a foreign regulator;
  - Have you previously explored the Canadian market for any of the drugs on the list;
  - Please share any factors that may have influenced your decision not to pursue market authorization, and are there ways to mitigate these factors; and
  - Do you have any additional feedback? Are you willing to meet with Health Canada to discuss your feedback in more detail.

- Industry partners wishing to participate in the consultation who have not received information about this consultation should contact brdd-cppic_brdd-cppci@hc-sc.gc.ca for more information.

Regulatory Flexibilities

- Based on feedback received from industry and other stakeholders, regulatory flexibilities may be needed to support increased filings of pediatric submissions to Canada, including:
  - **Precision Regulating**: opportunity to consider the use of the new authorities to the *Food and Drugs Act*, announced in Budget 2024, to support the review of certain pediatric indications and child-friendly drug formats, such as those included on the final NPLPD.
  - **Priority Pathways:** prioritization of pediatric products / submissions under new or existing review pathways.

Precision Regulating

- The amendments to the FDA will enable the Minister of Health to take timely action in a variety of situations
- Specifically, the authorities enable the Minister to make an order (a type of regulation) to:
  - **Put in place targeted exemptions** from specific regulatory requirements for certain therapeutic products and food, adding conditions as appropriate to ensure that health and safety standards are met
  - **Put in place supplementary rules** for certain therapeutic products to protect against potential health risks from unintended product use or adverse effects to health or the environment
  - **Rely on information or decisions** from select foreign regulatory authorities to satisfy specific regulatory requirements for therapeutic products and food

Precision Regulating: Reliance Authority

- There are gaps in the Canadian market for certain types of products.
- Products intended for small sub-populations can be particularly affected by market dynamics, including, for example, drugs intended for use in children or child-specific drug dosage forms.
- Exploring feasibility of relying on decisions made by other select international regulators to demonstrate Canadian regulatory requirements are met could help address one of the barriers to product access in Canada.
  - Would allow the Minister to make orders for select classes of products **where there is an unmet need** in Canada, such as certain pediatric drugs
  - Would increase our ability to **address certain barriers to submission** for certain types of products

Questions?

Please contact us!

brdd-cppic_brdd-cppci@hc-sc.gc.ca