Health Canada's Proposed Pediatric Drug Action Plan: Insights and Feedback

as part of
MICRYRN's Webinar Series

MICYRN
better health for mothers and children

February 3, 2021

centre de formulations pédiatriques
de la famille Rosalind et Morris Goodman du CHU Sainte-Justine

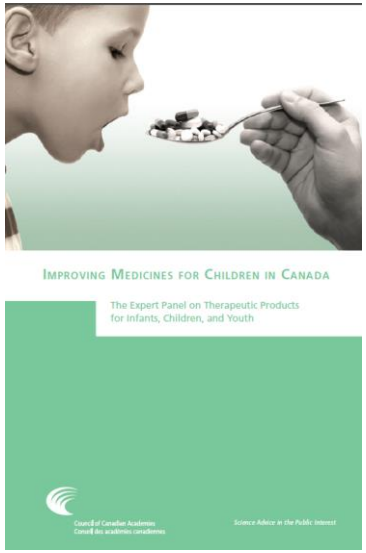
The Rosalind & Morris Goodman Family pediatric formulations centre of the CHU Sainte-Justine

Agenda

10:00am	Welcome and Introduction to Panelists
	Andrea Gilpin, GPFC
10:05am	**Alysha Croker**
	Office of Pediatrics and Patient Involvement
	Health Canada
10:25am	**Catherine Litalien, GPFC**
	Summary of Results from Survey
10:55am	Polling Questions to Audience
	Charlotte Moore-Hepburn, CPS
	Questions and Answer Period
	Andrea Gilpin, GPFC
11:30am	Conclusion of Workshop

Background to Survey

2014



2019

Paediatrics & Child Health, 2019, 333–335
doi: 10.1093/pch/pxz079
Position Statement

Position Statement

Improving paediatric medications: A prescription for Canadian children and youth

Charlotte Moore Hepburn MD[1], Andrea Gilpin PhD MBA[3], Julie Autmizguine MD MSc[2], Avram Denburg MD PhD, L. Lee Dupuis R.Ph. MScPhm PhD, Yaron Finkelstein MD, Emily Gruenwoldt MHA, Shinya Ito MD, Geert 't Jong MD PhD, Thierry Lacaze-Masmonteil MD PhD, Deborah Levy MD MS, Stuart MacLeod MD PhD, Steven P. Miller MD CM MAS, Martin Offringa MD PhD, Maury Pinsk MD, Barry Power PharmD, Michael Rieder MD PhD, Catherine Litalien MD[2]

2020-21

During the Canadian Society for Pharmaceutical Sciences in November 2020, Health Canada presented a new Pediatric Drug Action Plan (PDAP).

The GPFC and MICYRN wanted to provide feedback on the PDAP to Health Canada from the pediatric community and therefore organized this workshop.

In preparation for this workshop, the GPFC has sent a survey to gain comments and feedback on the proposed PDAP.

Focus of this session is on PDAP, with the exception of Clinical Trials issues, as MICYRN led a clinical trial workshop in January.

Alysha Croker
Office of Pediatrics and Patient Involvement

Overview of proposed Pediatric Drug Action Plan by Health Canada

Catherine Litalien, Co-Founder of GPFC and Pediatrician

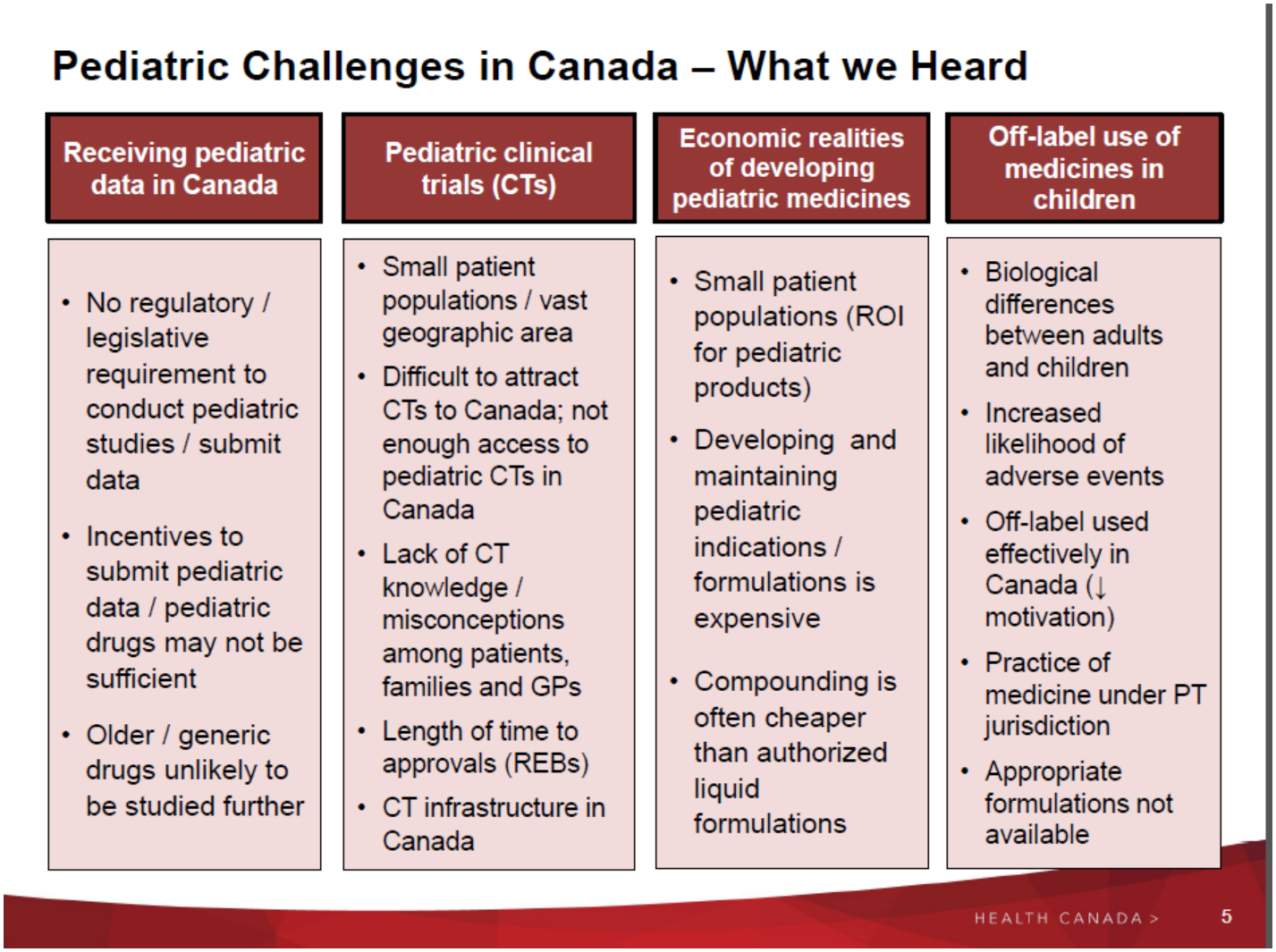
Summary of Survey results conducted by the Goodman Pediatric Formulations Centre.

Methodology

- The survey was conducted using Google Forms
- The questionnaire (8 questions) was sent to over 25 people representing over 19 organizations with the encouragement to share the survey with others in the pediatric community
- January 8-18, 2021
- 26 surveys were completed representing over 30 people (some people did one survey but were two people)
- 16 organizations responded
- Subject matter experts

- Academic institutions/universities
- Pediatric Associations
- Physicians/Clinicians
- Hospital Pharmacists
- Pharmacists
- Researchers
- Geographical diversity

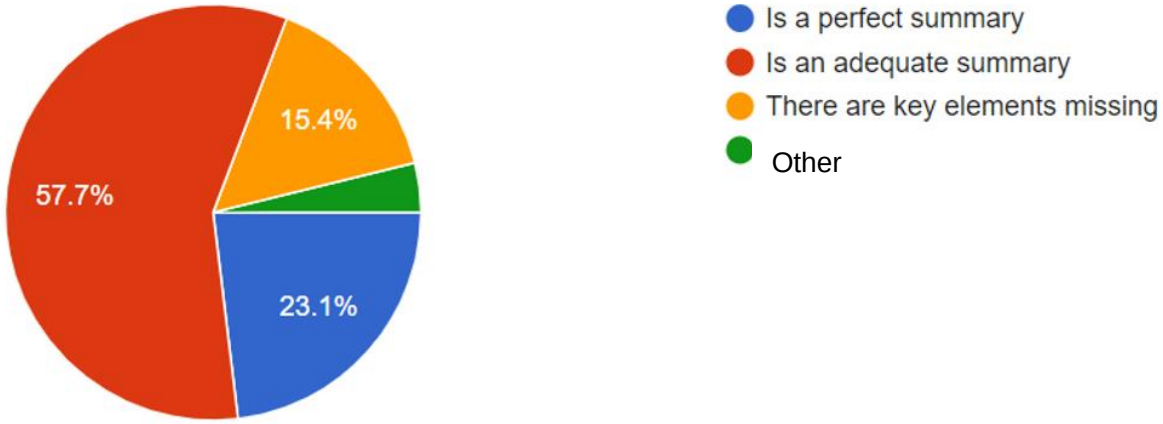
1. Was the summary of Health Canada's "What we Heard" complete ?



Health Canada's "What we Heard" captured many of the key points…

I believe that Health Canada's summary of the Challenges "What we Heard":

26 responses

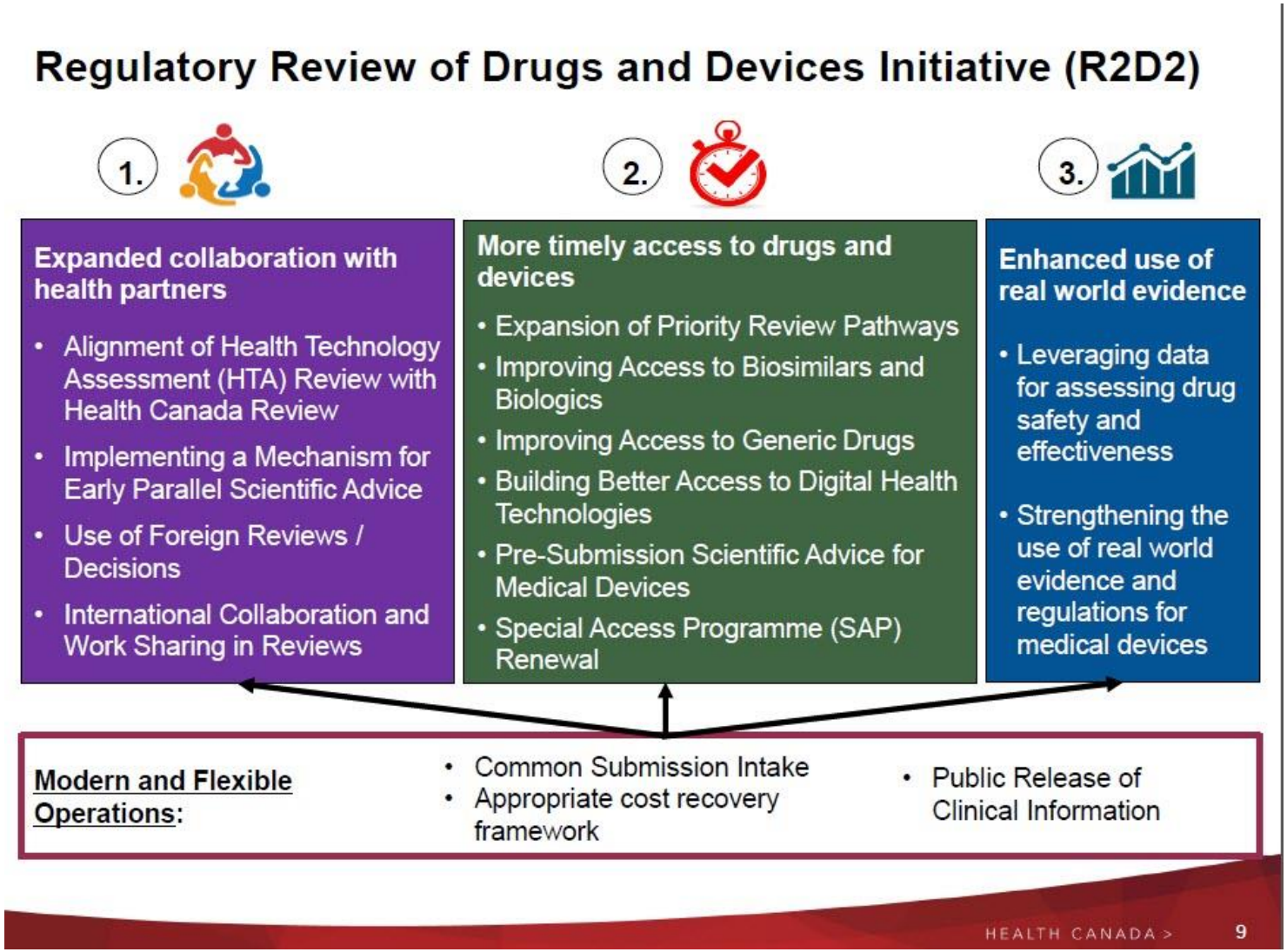


"What we Heard" Comments

Receiving pediatric data in Canada	Pediatric Clinical Trials	Economic Realities of developing pediatric medicines	Off-Label use and compounding of medicines in children
•Incentives to submit pediatric data/drugs **are insufficient** •Older/generic drugs unlikely to submitted for new indications or formulations •Awaiting the use of Trusted Foreign Decisions	•Legislation to require pediatric trials •Accept pediatric international trials •CHEER an asset	•Compounding may not be as cheap as we think •Inclusion of all costs for compounding (direct & indirect) •Compounding not equivalent to commercial formulations •Rare disease framework is missing •Smaller market=attractive incentives needed	•Lack of data capture from off-label use and compounding (and lack of funding) •No current standards for compounding across Canada •Access to appropriate formulations at a fair price

Transversal Key Components	
	• **Lack of Expert Pediatric Advisory Board (EPAB) at the Ministerial Level** • Misalignment with market access, reimbursement, pricing and provincial listing (disincentive) • Lack of pediatric specific criteria for HTA evaluation • Lack of harmonization from PTs to prioritize pediatric drugs/formulations • Lack of clear & consistent pathways and costs for entire process

2. R2D2


Regulatory Review of Drugs and Devices Initiative (R2D2)
1.
2.
3.
Expanded collaboration with health partners
• Alignment of Health Technology Assessment (HTA) Review with Health Canada Review
• Implementing a Mechanism for Early Parallel Scientific Advice
• Use of Foreign Reviews / Decisions
• International Collaboration and Work Sharing in Reviews
More timely access to drugs and devices
• Expansion of Priority Review Pathways
• Improving Access to Biosimilars and Biologics
• Improving Access to Generic Drugs
• Building Better Access to Digital Health Technologies
• Pre-Submission Scientific Advice for Medical Devices
• Special Access Programme (SAP) Renewal
Enhanced use of real world evidence
• Leveraging data for assessing drug safety and effectiveness
• Strengthening the use of real world evidence and regulations for medical devices
Modern and Flexible Operations:
• Common Submission Intake
• Appropriate cost recovery framework
• Public Release of Clinical Information
HEALTH CANADA >
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Summarized Comments- Pediatric Specific

1. **Expanded collaboration with health partners**

- Alignment of HTA review, PT listing and pricing decision with HC
- Use of foreign decisions (pediatrics may have own requirements given unique needs)
- Pathways to ensure rapid access to life saving pediatric medications for certain critical diseases
- Inclusion of patients and HCP

2. **Timely access to drugs and devices**

- Pediatric-specific considerations throughout the drug approval and market access process including provincial listing
- Address provincial barriers to list

3. **Enhanced use of real-world evidence**

- Develop process to capture extensive RWE with pediatric drug use (compounding and off-label)
- Funding needed for data registries
- How to engage all qualified stakeholders to report AEs (parents, HCP, pharmacists)

Modern and Flexible Operations:

- Unique fee structure for ped formulations
- Use of RWE in compounding medications to support commercial submissions
- Clear role for OPPI in broader HC modernization efforts
- Optimal positioning of OPPI within HC to bridge between regulation and clinical trial reform

3 PDAP Methods

Health Canada is exploring ways to potentially increase the development of essential pediatric medicines and formulations. Some potential actions include:

1. Developing a national priority drug list for pediatric diseases / molecular targets (including formulations) that will guide pediatric medicine development in Canada
2. Implementing a bench-to-bedside (pre-clinical, CTs, authorization) platform to develop the drugs on Canada's priority list (with stakeholder support)
3. Developing incentive models for pediatric drug and formulation development
4. Improving the national infrastructure for pediatric CTs in Canada
5. Working across regulators to support alignment of pediatric CT requirements
6. Applying CT Modernization projects / activities to pediatric trials

3 PDAP Methods -Comments

Health Canada is exploring ways to increase the development of essential pediatric medicines and formulations. Some potential actions include:

- Developing a **national priority drugs list** for pediatric diseases/molecular targets (including formulations) that will guide pediatric medicine development in Canada.
  - Governance of committee, funding and organization – when to be established and who is leading this effort?
  - Recognition of list and priority by HTA and PT
  - Includes rare disease and formulations

- Developing **incentive models** for pediatric drug and formulation development.
  - Off-patent medications need special considerations (reimbursement exclusivity)
  - Longer data protection or patent for branded pediatric medicines and formulations
  - Work with international partners

4 PDAP Proposed Actions

Health Canada is exploring ways to potentially improve access to pediatric medicines and formulations. Some potential actions include:

1. Implementing a pediatric regulation (and associated guidance document) that will bring Health Canada in alignment with the FDA and EMA in requiring sponsors to develop a pediatric plan and submit the data created by conducting the studies in the plan
2. Applying international worksharing and collaboration to pediatric submissions (ACSS, Project Orbis)
3. Incentivizing the submission and maintenance of pediatric drugs / formulations in Canada
4. Working across regulators to support the alignment of pediatric drug authorization requirements across regulators
5. Developing mechanisms with HTAs and PTs to ensure that (expensive) drugs and formulations for pediatric populations are given serious consideration for inclusion in PT formularies
6. Applying Agile Licensing Framework (ALF) regulations to bring pediatric indications to Canada (e.g., Use of Foreign Decisions / Real World Evidence) and to monitor their safety / effectiveness

PDAP Proposed Actions - Feedback

Health Canada is exploring ways to potentially improve access to pediatric medicines and formulations. Some potential actions include:

- The requirement of two pediatric committees to provide expertise for this ambitious plan
  - Expert Pediatric Advisory Board (EPAB) at the ministerial level to harmonize and prioritize pediatric medicines across all agencies and jurisdictions.
  - Health Canada Pediatric Advisory Board to support Health Canada and the OPPI in all pediatric initiatives and provide pediatric expertise where needed.
- Proposed Pediatric Regulation – Obligation to Submit is Important
  - Mandate, time line, funding and who is the lead for this effort?
  - No Canadian PIP or PSP – use what was already submitted in the US or Europe as is
- Modern approach to rare diseases- allow development of a therapy for an entire family of rare diseases
- Require communication on progress, annual benchmarking and progress reports for international worksharing and collaboration to pediatric submissions
- Regarding incentives need to keep in mind small-mid size companies who are most likely to commercialize in Canada
- Acceptance of Trusted Foreign Decisions (“Accept data package as is”) will have a significant impact to improving access to pediatric medications/formulations in Canada

Communication Strategies Proposed

Health Canada is exploring ways to provide more information to Canadians on relevant pediatric topics. Some potential actions include:

1. Launching a pediatrics website
2. Actively engaging with HC's pediatric stakeholders and the general public in order to keep them up-to-date regarding pediatric activities and information
3. Implementing a pediatric data strategy to create and maintain relevant databases and publish regular analyses and reports
4. Launching information / awareness campaigns to encourage the participation of pediatric populations in CTs / increase knowledge of open pediatric CTs in Canada
5. Establishing the Office of Pediatrics and Patient Involvement (OPPI) as Health Canada's centre of expertise and single point of contact for pediatrics

Communications- Feedback

Health Canada is exploring ways to provide more information to Canadians on relevant pediatric topics. Some potential actions include:

- Concern that certain aspects of this proposed initiative will detract resources from the important actions outlined in the PDAP
- A priority must be for the OPPI to publish its project plan with deliverables and timelines with annual progress reports to the public.
- Communicating the mandate and governance of both the EPAB and Health Canada Pediatric Committee
- It was reported that actively engaging with HC's pediatric stakeholders is key and should include parent/patients, hospital pharmacists, pharmacists and the general public
- Funding is critical to create and maintain databases to monitor and quantitfy medication incident reporting, working with ISMP
- Develop formal mechanism of communication and prioritization of approvals of pediatric medicines with HTA and PTs
- Need to understand the extent pediatric drugs are compounded and funding for standardization of compounding
- Use successful awareness campaigns launched in other countries.

Most Important Actions to Improve Access to Pediatric Medications

- **Expert Pediatric Advisory Board at Federal Ministry** to harmonize and prioritize across the entire drug approval process
  - Alignment across all decision points is critical
- Establish **HC Pediatric Committee** governance and mandate with input from pediatric community
  - Priority pediatric medicines list
- Accept **Trusted Foreign Decisions**
- Institute **Pediatric Regulation** using mandatory submissions “as is” from other countries
- Deploy **incentives for commercialization** in Canada both brand and off-patent medications

Government of Canada

Health Canada

The publication of the OPPI’s plan with clear objectives, metrics, funding, and a timeline for implementation.

Polling Questions

Charlotte Moore-Hepburn,

Pediatrician and

Medical Affairs Director,
Canadian Paediatrics Society

63 people attended the workshop and participated in the polling questions. Participants were from varied backgrounds and disciplines.

Polling Question 1 Results:

1. In your opinion, how important is the implementation of a pediatric regulation to increase access to pediatric medications and formulations in Canada?

Polling Question 2 Results:

1. Trusted Foreign Decisions as currently drafted indicates that 15 years of foreign market experience is needed for this to be used in Canada. Should this be different for pediatrics?

a) 15 years market experience in pediatrics is appropriate (0) 0%

b) 5-7 years experience is sufficient (13) 33%

c) 2 years experience is sufficient (14) 36%

d) foreign approval is sufficient with 0 years experience (12) 31%

Polling Question 3 Results:

1. In your opinion what are the three most important actions to take to improve access to pediatric medications/formulations in Canada? (please choose three of the following):(Multiple Choice)

Questions for **Health Canada**

1. The CCA report was published in 2014 and many of the recommendations in the PDAP were presented at that time. When will the OPPI present **clear objectives, with metric and targets**?
2. What is the **budget** for this initiative and how many resources will be dedicated to PDAP?
3. Recognizing that HC has internal priorities – how can the pediatric community support HC?
4. How will the decision be made to **formalize pediatric expertise** at:
    1. EPAB at the Federal Ministry of Health
    2. Health Canada Pediatric Committee
5. What approaches are possible for HC to contribute to **providing incentives to industry**?
6. How will the PDAP framework will address old off-patent drugs?

Top 3 Most Important Actions

Based on the polling questions during the workshop the following three were cited as the top most important actions.

1. Institute **Trusted Foreign Decisions 79%**
2. Mandatory **Pediatric Regulation 69%**

   using submissions “as is” from other countries
3. **Incentives for commercialization 46%**

   in Canada both brand and off-patent medications

Overall Health Canada's PDAP Responds to Many of the Needs Identified by Stakeholders

The pediatric community looks forward to continuing to support Health Canada in its objectives to bolster pediatric submissions in Canada.

Thank you for the opportunity for the pediatric community to provide Health Canada with feedback to the PDAP.