

Access to Approved Therapies: Public Listing of Drugs with Pediatric and Adolescent Indications



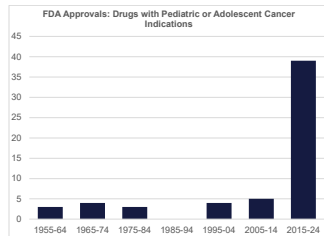
Keith McIntosh¹, Sandra Judd², Paul Gibson³

¹Advocacy for Canadian Childhood Oncology Research Network (Ac2orn), ²Department of Pharmacy, Hospital for Sick Children, ³Division of Haematology/Oncology, McMaster Children's Hospital



Background & Rationale

- Most pediatric cancer therapies prescribed **off-label**.
- Historically few cancer drugs have been approved for pediatric use. Since 2017, the U.S. Food and Drug Administration (FDA) has approved many more pediatric cancer indications.
- Regulatory approval is expected for Canadian HTA and reimbursement.
- To date, no comprehensive analysis has examined whether **Health Canada–approved pediatric cancer indications translate into routine public reimbursement** across Canada.



Objectives

Phase 1 – Regulatory approval:

- Identify cancer drugs with **regulatory-approved pediatric and adolescent indications** in the US, Canada, Europe, and Australia.
- Compare the **timing** of approvals across jurisdictions.

Phase 2 – Reimbursement:

- Examine whether approved pediatric cancer indications translate into **predictable and routine public reimbursement** across Canada.
- Assess how pediatric indications have been considered within HTA and reimbursement frameworks

Methods

Study Design

- **Phase 1:** Cross-jurisdictional regulatory approval analysis
- **Phase 2:** Canadian public reimbursement analysis

Phase 1: Regulatory Approval Analysis

- Reference dataset: FDA list of oncology drugs with approved pediatric indications. Timeframe: **2000–2024**
- Unit of analysis: Drug ingredient
- Indication groups:
 - **Group 1 (Pediatric):** < 18 years
 - **Group 2 (Adolescent):** 12 to < 18 years
- Comparator regulators: Health Canada, European Medicines Agency (EMA), Therapeutic Goods Administration (TGA), Australia

Phase 2: Canadian Reimbursement Analysis

- Study cohort: Drugs with a Health Canada–approved pediatric or adolescent indication
- Review of provincial drug formularies, with validation and review through consultation with the Canadian Pediatric Oncology Pharmacy Community of Practice.
- Reimbursement categories:
 - 1: Routine listing; 2: Restricted listing; 3: Exceptional access; 4: No public coverage
- Data collection completed **October 2025**

