JPPT | National Survey

High-Cost Drug Policies in Canadian Children's Hospitals: An Exploratory Study

Aidan Pucchio, BSc and Michael Rieder, MD, PhD

OBJECTIVE Over the past decade a number of effective but costly drugs have entered the therapeutic arena. Ethical and logistical challenges associated with including children in research and policy have produced variability in public policy on funding pediatric drugs, with inconsistent coverage across Canada. The purpose of this study was to explore the processes for funding high-cost pediatric drugs in Canadian children's hospitals.

METHODS We conducted a cross-sectional, text-based survey of all 19 chairs of Canadian departments of pediatrics about the funding and accessibility of high-cost drugs. Thematic qualitative analysis was performed to organize, sort, and code verbatim written responses and follow-up correspondence.

RESULTS Responses were received from all 19 Canadian departments of pediatrics surveyed (100% response rate). Three major themes emerged about pediatric high-cost drug policies: inconsistency between funding processes, variability in funding sources, and frustration with the current system. In aggregate, a clear concern emerged that current funding options were heterogenous and inadequate to meet patient needs.

CONCLUSIONS There was widespread consensus from respondents that current options for funding pediatric high-cost drugs were inadequate and that there was need for urgent action to address this problem. Policy changes are needed to sustain and improve access to high-cost drugs for Canadian children. We propose 3 solutions, including the creation of a national framework for funding high-cost pediatric drugs, increased incorporation of pediatric considerations in drug research and development, and a multidisciplinary drug summit on pediatric therapeutics.

ABBREVIATIONS SRQR, Standards for Reporting Qualitative Research

KEYWORDS health resources; hospitals; pediatric; pediatrics; resource allocation; therapeutics

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Introduction

Drug development and public policy have traditionally had a strong focus on adults, largely due to the ethical and logistical challenges associated with involving children in research.[1–5] Despite recent efforts to integrate the needs of children into pharmaceutical research, regulation, and policy, pediatric drug therapy remains understudied.[6,7] A lack of substantial research and policy development has led to significant variability in public policy on funding for pediatric drugs, resulting in inconsistent coverage between centers and across Canada.[5]

The paucity of available evidence about pediatric pharmaceuticals is even more significant in an era of rapid development of novel therapeutic entities.[8–10] The sharp increase in the development of biological and monoclonal antibody agents as therapeutics has led to their frequent approval in niche populations and subsequent use in broader patient groups, exacerbating deficits in access to funding across different patient populations.[4] This is particularly true in children, where off-label use of new drugs is common. While these drugs offer great promise, there is also potential for challenges for access; many biological agents and monoclonal antibodies are very costly and are frequently not included in hospital formularies.[11] Many of these agents can be defined as high-cost drugs, which Canada's Patent Medicine Price Review Board defines as medications that costs more than $40 CDN per day.[12] Because novel and high-cost therapies for children are most commonly used either as in-patient or ambulatory care facilities in hospitals, it is crucial that hospitals have an effective process for prescribing and funding these drugs. Further, provincial drug funding programs vary considerably in entitlement to coverage and out-of-pocket cost.[5] For example, when used in juvenile arthritis, infliximab is listed on pediatric formularies in Québec, but not in Manitoba, Ontario, or British Columbia. There are many other examples of regional disparities for funding high-cost pediatric medications.

Table. List of Children's Hospitals Surveyed

Alberta Children's Hospital (Calgary)
British Columbia Children's Hospital
Centre Hospitalier Universitaire de Sherbrooke (CHUS)
Centre Hospitalier Universitaire Sainte-Justine (Montreal)
Children's Hospital at London Health Sciences Centre
Département de Pédiatrie Faculté de Médecine de l'Université Laval
Department of Paediatrics, Northern Ontario School of Medicine
Holland Bloorview Kids Rehabilitation Hospital
IWK Health Centre
Janeway Children's Health and Rehabilitation Centre
Jim Pattison Children's Hospital
Kingston Health Sciences Centre (Queen's University)
McMaster Children's Hospital
Montreal Children's Hospital (McGill)
Shriners Hospital for Children
Stollery Children's Hospital
The Children's Hospital of Eastern Ontario (CHEO)
The Children's Hospital of Winnipeg
The Hospital for Sick Children (Toronto)

There is currently no comprehensive pan-Canadian strategy to provide access to high-cost drugs for children in a hospital setting or to address indications for use or disparities in access for evolving novel therapeutics in this population. In this exploratory study, our primary objective was to examine the processes to acquire funding approval for high-cost pediatric drugs within Canadian children's hospitals by surveying the pediatrics department chairs at Canadian children's hospitals. Our secondary objectives were to explore funding sources and barriers to access funding for high-cost pediatric drugs. Finally, we proposed potential solutions to improve access to high-cost drugs for children.

Methods

Our methods and analysis were developed in consultation with the Standards for Reporting Qualitative Research (SRQR) guidelines.[13] This study was approved by the Research Ethics Board of Western University. We conducted a cross-sectional survey of the department chairs of the 19 departments of pediatrics across Canada (Table). If the chairs were unable to answer survey questions, they were asked to provide a referral to the group responsible for pediatric drug funding decisions or the creation of the hospital's pediatric drug formulary.

A 4-question English-language survey was developed. In an effort to ensure question clarity and survey platform accessibility, the survey was piloted by a colleague of the authors who was not involved in survey instrument creation; no formal pretest was performed. All participants were contacted via email by a member of the research team (AP, MR) from January 2020 through August 2021. Informed consent was received, participation was entirely voluntary, and no monetary incentives were offered for participation. Participants were provided a text-based survey consisting of the following open-ended questions: 1) Does your hospital have a policy for how to deal with high-cost drugs that are not covered by provincial health insurance or are not on the hospital formulary; 2) If so, how does this policy work; 3) If not, how are these therapies addressed; 4) What do you think is the best approach to provide access to high-cost drugs for children. Areas of uncertainty were followed up through text-based email interviews. Responses were not anonymous to the research team, as participants were directly contacted. Verbatim written responses of both the initial survey answers and follow-up correspondence were used for qualitative analysis.

Interview responses were analyzed verbatim by using emergent thematic analysis to describe the experiences of participants with funding high-cost drugs in pediatric children's hospitals.[14] Qualitative analysis software (NVIVO 12 Pro, Melbourne, Australia) was used to organize, sort, and code verbatim written responses and follow-up interview correspondence. Inductive manual coding was performed by one author (AP). Codes were initially developed as the smallest unit of analysis. Similar codes were grouped together to form subthemes, and similar subthemes were further grouped to form themes. Themes were developed and revised in an iterative manner as patterns became apparent to ensure accurate data interpretation. Finalization of themes occurred after review and discussion by all research team members. No coding diary was maintained during the analysis process.

Results

All 19 Canadian departments of pediatrics were surveyed, and 19 responses were received (100% response rate). Sixteen of the respondents identified themselves as pediatric chairs and 3 referrals were made to pediatric pharmacy departments to complete the survey. The 3 major themes that emerged about pediatric high-cost drug policies were inconsistency between funding processes, variability in funding sources, and frustration with the current system. Subthemes included poor pharmaceutical evidence in the pediatric population as a barrier to drug funding, the need for an evidence-informed process

for evaluating new and emerging therapies in children, and the effect of high-cost drugs on hospital budgets and physician time. Additional verbatim illustrative comments that are not quoted in the results are contained in the Supplemental Table.

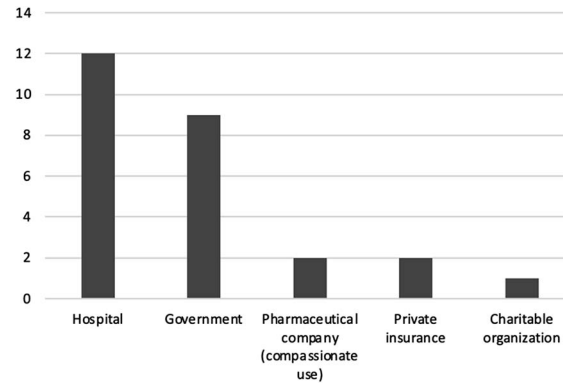
Processes for Approval of Funding for High-Cost Drugs. With regard to the process to acquire approval for institutional or external funding for high-cost drugs, 10 hospitals followed a formal process unique to their institution, 7 hospitals used a case by case system, and 2 used provincially regulated standardized processes. Sixteen respondents specified the discipline of decision makers, of which 12 centers used a multidisciplinary team consisting of both physicians and pharmacists, 3 centers used physicians as decision makers, and 1 center had a hospital administrator make all funding decisions.

There was substantial heterogeneity between funding processes. One respondent who identified a formal institutional funding process, described their system: "We have a process where the doctor in charge has to complete a form (medication, cost, disease and the problem, literature supporting the treatment and types of studies—often attached, duration, evaluation of results—how, consultation with at least another specialist, signature of a pharmacist)." Another respondent said "If the drug is costly or not approved provincially, it is evaluated by the evaluation committee. This committee asks the physician and other experts to evaluate the drug. Usually, the drugs evaluated are mostly for unrecognized indications rather than their cost." One of the respondents whose institution used a case-by-case funding process stated "We do not yet have a general policy… As a physician we have to negotiate, individually, directly with the government, without really any support of the hospital."

Most of the funding processes used an evidence-based review process that required literature supporting the treatment. Three respondents indicated that "… the evidence base for a lot of pediatric conditions is thin or non-existent." As such, an evidence-based funding process that required literature specific to the pediatric population presented a substantial barrier to funding approval, illustrated by one respondent who said that "Going on just evidence is particularly challenging." Another respondent acknowledged these barriers, but noted that "It is acknowledged that evidence is often less robust in pediatrics compared to adults… There needs to be evidence for the treated condition before funding can take place, even if this evidence is in adults."

Sources of Funding. Sources of funding were variable, with many institutions drawing on multiple sources of drug funding. Direct funding of drugs from the hospital budget was the case at 12 centers, government funding used at 9, pharmaceutical company compassionate funding at 2, private insurance at 2, and charitable organization funding at 1 (Figure). While hospital funding was the most commonly cited option for funding high-cost drugs, this solution was not without issues, with one respondent stating "If [the provincial funding system] says no, even if we think as physicians there is a medical indication, it will be no by the hospital pharmacy." In aggregate, a clear concern emerged that current funding options were heterogeneous and inadequate to meet patient needs.

Figure. Sources of high-cost drug funding (multiple sources possible per respondent).



Sentiment About Drug Funding and Suggestions. The challenging process in applying for funding and the heterogeneous sources of funding were described as a source of frustration in numerous responses, with deficiencies noted at the federal level, provincial level, and institutional levels. Five responses expressed concern with the current system, indicating that it was not adequately meeting the needs of patients or physicians, while 6 stated that the system needed to be improved: "There needs to be an evidence informed process for evaluating new and emerging therapies in children."

Beyond implications for patient care, there was agreement that drug access had a significant effect on both hospital budgets and physician time with quotes such as "It is very concerning and time-consuming for my team." and "… we are facing extremely high costs for treatments for some of our patients." There was also a consensus that a pan-Canadian approach was needed, with suggestions including a national drug formulary for children that could provide evidence and recommendations to provincial funders, or a conference to consider policy options for pediatric therapeutics. One respondent explained that "An opportunity lies in outlining what evidence is expected (drugs that show a benefit in meaningful patient outcomes or a surrogate that is demonstrated to correlate with that outcome) and determining a relative value the health system is willing to pay.… This evidence collection should not force the health care system to direct limited treatment dollars from elsewhere in the system so we can understand whether or not the drug has a benefit in patients."

A concern raised by several respondents was how the support of high-cost drugs would transition from the hospital to home care when patients moved to at-home or ambulatory settings.

Discussion

There was widespread consensus from respondents that current options for funding pediatric high-cost drugs were inadequate and that there was a need for urgent action to address this problem. As rapid development of novel therapeutics continues, paralleled by increasing drug costs, drug policy must adapt at a similar pace.

Previously, a study by Denburg et al[6] identified systemic limitations in use of pharmaceuticals for pediatric patients. They noted that evaluation of novel drugs by government committees for a pediatric indication is uncommon and that funding recommendations are primarily based on adult indications. As most hospital-specific funding processes we reported on are evidence based, this systemic lack of assessment is likely to be a barrier to receiving funding approval for high-cost drugs. Funding issues for pediatric drugs have been reported for nearly 20 years, with a 2005 report by Ungar and Witkos[5] detailing significant financial barriers to medication access in Canadian children—it is likely that these issues have been exacerbated by the increasing use of biological agents and the heterogeneity in funding models we describe.[5,11] In addition to the more ubiquitous logistical challenges associated with prescribing high-cost drugs to children, there also remains administrative challenges. As health care is provincially administered in Canada, regional variation in drug funding remains an ongoing issue. While some high-cost medications may be funded in certain provinces or territories, they may remain unfunded in others, further complicating the development of solutions that address access to these therapeutics.

A limitation of this study is that we omitted discussion of the ethical or pragmatic justification for publicly funding high-cost therapeutics, where it must be noted that funding these drugs could divert financial resources away from potentially higher-yield endeavors. Another limitation of this study is that we did not ask participants about the process for dealing with conflicts of interest when requesting the inclusion of drugs on a hospital formulary. Because we only interviewed 1 individual per institution, our evaluation of institutional policy was less vigorous than it would have been with multiple, multidisciplinary respondents at each institution. Finally, as funding for high-cost pediatric drugs is not a well-established process on any of the federal, provincial, or institutional levels, our survey instrument was open ended and high-level. While this did provide us good insights into some of the issues in these funding processes, further research is required to establish consistent shortcomings and test potential solutions.

On the basis of the preceding rationale and information, we propose several approaches to support improvement in access and affordability for pediatric drugs. First, there have been previous calls for a national framework for pediatric drug funding, which we believe our data support.[1,5,6] Such a framework could support equal opportunities for funding consideration between provinces and centers, remediating inconsistent processes unique to each hospital. Efforts to guide national drug formulary development, such as the pan-Canadian Advisory Panel on a Framework for a Prescription Drug List, have begun to address this need.[15] Alternatively, a guide for institutional decision-making with regard to high-cost drug coverage could be considered as a more pragmatic solution. Second would be policy to support the incorporation of pediatric considerations in drug approval processes, research, and development. Creating robust evidence is important to support ethical and appropriate decision-making for the pediatric population, which subsequently supports informed funding decisions. Notably, it is possible smaller-scale solutions may not adequately facilitate the incorporation of pediatric considerations in drug approval processes, research, and development. This process might require more foundational changes such as widespread recognition from pharmaceutical companies, risk-sharing agreements, efforts from large pharmaceutical vendors, or market access regulation. For example, risk-sharing agreements in partnership with pharmaceutical companies or the prospect of provisional drug approval by Health Canada pending collection of real-world data could facilitate discounts on high-cost medications.[16] However, given the paucity of evidence as to how these changes might be implemented and high financial and human cost required to implement more radical solutions, these recommendations would require more robust examination before they could be definitively suggested. As well, as noted above given that hospital funding in Canada is provincially administered, there are a number of administrative and procedural challenges to such approaches. Finally, we recommend an annual, multidisciplinary Canadian summit on pediatric drug policy and development. Receiving input from key stakeholders such as clinicians, pharmacists, policy makers, and patients in a rapidly evolving research and regulatory space could help direct policy, advocacy, and research efforts, and provide direction in an undersupported field of research.

Conclusion

Three major themes emerged about pediatric high-cost drug policies, including inconsistency between funding processes, variability in funding sources, and frustration with the current system. Respondents unanimously indicated that the current systems to fund these drugs were not meeting the needs of patients and warranted urgent action. We recommend the creation

of a national framework for high-cost pediatric drugs, increased incorporation of pediatric considerations in drug research and development, and a multidisciplinary Canadian drug summit on pediatric therapeutics.

Article Information

Affiliations. School of Medicine (AP), Queen's University, Kingston, Ontario, Canada; Departments of Paediatrics, Physiology & Pharmacology and Medicine (MR), University of Western Ontario, London, Ontario, Canada.

Correspondence. Michael Rieder, MD, PhD; mrieder@uwo.ca

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Ethical Approval and Informed Consent. The authors assert that all procedures contributing to this work comply with the ethical standards of the relevant national guidelines on human experimentation and have been approved by the appropriate committees at the Research Ethics Board of Western University. All participants provided written informed consent at enrollment.

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