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Position Statement

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Pharmacare in Canada: The paediatric perspective

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Abstract

Canada's drug insurance system is one of the most expensive in the world, yet millions of Canadians still struggle to access necessary medications. As a result, provincial, territorial, and federal governments are considering public pharmacare policy proposals to ensure that all Canadians can access the medications they need. Pharmacare policies offer an opportunity to prioritize children and youth, whose unique drug needs have long been neglected. Prescription drug use is common in this population, with approximately half of Canadian children and youth requiring at least one prescription in any given year. Drug use remains concentrated, however, among those with complex, chronic, and serious diseases. Children and youth rely heavily on compounded and off-label prescription drugs, which impacts safety, efficacy, palatability, and cost. Reimbursement decision-making bodies do not appropriately value the unique benefits of paediatric drugs, including child-friendly formulations, improved quality of life for children and families, and cost-savings outside the healthcare system. Regardless of the pharmacare model ultimately implemented, ensuring universal, comprehensive, and portable prescription drug coverage for all children and youth is essential. To accomplish this, paediatric drug experts should develop a national, evidence-informed formulary of paediatric drugs. Health Canada should also improve processes to make commercial paediatric drugs and child-friendly formulations more available and accessible. The federal government must also support paediatric drug research and development to this end.

Keywords: *Child health services; Drug costs; Drug insurance; Paediatric pharmaceuticals; Pharmacare; Pharmaceutical economics*

Canada is currently the only high-income country in the world with a universal public health insurance system that does not include prescription drug coverage (1). Drug coverage is instead provided by a patchwork system of over a hundred public, and an estimated 100,000 private, insurance plans (2). Public drug insurance is inconsistently available across provinces and territories, and is limited to select populations, such as adults over age 65, low-income families, and individuals with mental or physical disabilities (3). Nearly one in five Canadians report that they do not have prescription drug coverage, which limits their access to necessary medications. A similar number report that they, or other household

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members, have not taken a drug as prescribed within the last year due to prohibitive cost. Almost a million Canadians have reported sacrificing food or heat to afford medications for themselves and their children (4).

In addition to these fundamental problems of access and equity, Canada's patchwork system is costly, inefficient, and unsustainable. On average, drugs cost almost $1,100 per person per year, with over 20% of costs being 'out-of-pocket' (5). Canada pays the third highest per person costs for prescription drugs in the world: 62% higher than the United Kingdom, and more than twice that of Denmark, two countries with otherwise similar health care systems (6). Although drug costs for adults are higher than for children, the average drug costs for a Canadian child are nearly $500 per year (7).

As Canada contemplates policy solutions to address these disparities and inefficiencies, it is critical to address the unique challenges faced by children and youth in accessing safe, effective, prescription drugs:

1. Children and youth are a vulnerable population warranting special attention.
2. They have unique prescription drug needs that differ from those of adults.
3. They have experienced significant regulatory neglect, leading to poor availability of paediatric drugs in Canada.
4. Drug therapies for children and youth provide significant return on investment that may not be captured by current assessment processes.

A pharmacare program presents an opportunity to improve access to prescription drugs for all Canadians. As this important conversation moves forward, distinct paediatric drug issues must also be addressed to both increase equity of access for paediatric patients and to improve regulatory and reimbursement standards for paediatric drugs.

PAEDIATRIC ISSUES ARE CENTRAL TO PHARMACARE

Numerous reports have detailed the economic and social benefits of pharmacare (8,9), defined as a drug insurance system that ensures access for all Canadians to the medications they need. Most Canadians support making drug insurance more universal (10). However, significant debate exists over the specific mechanisms to expand coverage. Proposals range from a single-payer system similar to existing provincial/territorial, hospital and physician insurance plans, to a modest expansion of existing programs for low-income and other vulnerable Canadians, to mandatory private drug insurance. Some advocates, including political leaders (11) and academics (12) have developed an 'essential medicines' list, modelled after the World Health Organization's 2017 'Model List of Essential Medicines for Children' (13), with a focus on commonly prescribed medications for primary and preventive care. Others have advocated for more comprehensive formularies that include a broad range of less commonly prescribed medications.

In all prospective pharmacare models, specific attention must be paid to the paediatric population. Although children and youth account for a small fraction of total drug costs, they are not 'little adults'. They have unique medical and pharmaceutical needs that require unique consideration (14,15).

Children and youth are a vulnerable population warranting special attention

National and international laws and customs have enshrined the rights of all young people to the care and assistance they need to grow and achieve their full potential. Central to this is the right to 'the highest attainable standard of health', as ratified in the United Nations Convention on the Rights of the Child (16). Drug therapy is a critical component of healthcare for many children. Recognizing the distinct rights of children, paediatric drug coverage should be a priority in any pharmacare plan. All children and youth, no matter what their socio-economic or health status or where they live in Canada, must have access to the safe, effective medications that they may need. Most Canadians support this position, and public drug plans that prioritize children are generally popular (17).

Children and youth have unique prescription drug needs that differ from those of adults

About one-half of Canadian children require at least one prescription medication in any given year, and the average child receives four prescriptions per year (5,7). However, while prescription drug use is common in young people, patterns of use differ significantly from those of adults (18). Many adults, and especially seniors, require multiple prescription drugs for chronic health conditions, but the most commonly used drugs for children are antibiotics prescribed for short-term use (19). Young people who require medications for chronic conditions have a far more heterogeneous pattern of medication use than adults. For example, the most commonly prescribed medication for a chronic condition in paediatric patients—an inhaler to treat asthma—is used by only 6% of children (20). By contrast, the most common prescription drug for a chronic condition in adults—a statin to treat high cholesterol—is used by almost half of the patients over age 65 (18).

Drug use in children is also asymmetric, with 70% of paediatric drug use concentrated amongst the 20% of children with chronic, complex, or serious diseases (7). Moreover, a significant number of children live with a 'rare disease', defined by

Health Canada as 'a life-threatening, seriously debilitating or serious and chronic condition affecting a fairly small number of patients' (21). In general, each disease affects less than 1 in 2,000 people (22). Although these diseases are individually uncommon, when considered together they become relatively common, with the burden of rare disease particularly concentrated in the paediatric population. Approximately 1 in every 12 Canadians are affected by a rare disease, with two-thirds being under 18 years of age (23). When drug therapies exist for children with a rare disease, they are by their nature uncommonly used drugs.

To address the heterogeneous drug needs of Canadian children and youth, paediatric pharmacare proposals must be comprehensive. While the epidemiology of adult disease may tempt policy makers to adopt a more targeted, 'essential' list of covered drugs, this approach would exclude an unacceptably large number of children and youth. Recognizing that uncommon and rare diseases are, in fact, common in paediatrics, a paediatric pharmacare strategy must include coverage of medications for uncommon conditions.

Existing public and private drug insurance plans cover a select and highly variable number of drugs for children and youth. For example, some provincial or territorial drug plans cover as few as 37% of high-cost drugs for rare diseases, while others cover up to 90% (24). In some jurisdictions, drugs covered for adult conditions are not similarly covered for children or youth. This discrepancy leads to a 'postal code lottery' where young people may not receive coverage for their medications depending on where they live. This means some families are forced to uproot their lives to ensure access to vital medications for their child. Eliminating this 'postal code lottery' should be a national priority.

A further issue is that many children cannot swallow pills, and require medications in a liquid or quick-dissolving form. Flexible dosing is also essential, because dosage amounts in newborns, children, and youth are typically based on weight and/or body surface area. Many medications needed by children are not available in commercially prepared, child-friendly formulations. Therefore, pharmacists, other health care providers, or parents may need to manipulate an adult drug (e.g., a pill) to create a liquid formulation for a paediatric patient (a process known as 'compounding'). Compounded medications often have an unpleasant taste, which can affect compliance and, ultimately, therapeutic outcome. Compounding also increases risk for dosage errors, reduces drug uniformity, and may impact bioavailability, stability, and potency. Compounding methods vary from one pharmacy to another, raising risk for inconsistent composition and for dosing errors, and reducing therapeutic effectiveness.

Therefore, policy makers must actively pursue improvements to approval and reimbursement processes for commercial paediatric formulations. Commercial formulations have strict quality, manufacturing, and efficacy standards that do not apply to compounded medications. They also often taste better. And although commercial formulations may be associated with higher costs, this additional expense must be weighed against potential benefits, including decreased risk for adverse effects, improved patient safety, and optimal adherence. Children and youth should have the same access to quality medications as adults.

Children and youth have experienced significant regulatory neglect, leading to poor availability of paediatric drugs in Canada

Reimbursement and access are inextricably linked to regulatory decisions, including which drugs are approved for which indications. This impacts which drugs are ultimately available for sale in Canada. Unfortunately, regulatory neglect has compromised access to many paediatric medications, as explained in a recent statement (14).

To market a drug in Canada, a manufacturer must submit evidence for the safety and efficacy of their product to Health Canada (25). Based on this submission, Health Canada approves a drug for use in a particular population, along with distinct conditions and age ranges for use. These 'indications' are the basis for the official product monograph, often referred to as the drug 'label'. Once a medication is labelled for use in Canada, it can be prescribed by physicians for patients with indications other than those listed on the product monograph. This practice is known as 'off-label' use. Importantly, 'off-label' does not mean 'off-evidence' (14). Physicians prescribing a drug off-label must evaluate clinical and scientific evidence to determine whether benefits outweigh risks in a specific patient. Given the research and regulatory environment in Canada, off-label prescribing is the cornerstone of many effective paediatric treatment regimens.

Approximately 80% of paediatric prescriptions are off-label in Canada (15). Because physicians can prescribe medications off-label, there is little incentive for manufacturers to obtain paediatric-specific data from clinical trials. In contrast to the United States and the European Union, manufacturers are not obliged to submit paediatric data to Health Canada, and often choose not to. It is an expensive and onerous process to apply for paediatric approvals, and medication can still be used in young patients in accordance with off-label prescribing norms. Children deserve the same protections from the drug regulatory system as adults, but this standard is not being met.

Longstanding regulatory issues may negatively impact pharmacare policies. Any decision about drug coverage that is limited to Health Canada-labelled indications risks significantly reducing or preventing access to vital medications for Canadian children. Determining which medications are to

be covered under any prospective pharmacare system should be guided by paediatric experts, informed by best evidence, and decoupled from Health Canada's approved paediatric indications.

Drug therapies for children and youth provide significant returns on investment that may not be captured by current Canadian assessment processes

When a medication is approved for use by Health Canada, public and private drug insurance plans must determine whether to cover it. The Canadian Agency for Drugs and Technologies in Health (CADTH) evaluates all new drugs based on clinical, economic, and patient data, and provides reimbursement recommendations for Canada's federal, provincial, and territorial insurance plans (a process known as Health Technology Assessment or HTA). In Quebec, the 'Institute national d'excellence en santé et en services sociaux' (INESSS) makes drug reimbursement recommendations using a similar but separate HTA process.

CADTH analyzes a drug's costs and benefits and compares them with existing treatments (26). Generally, improvements in mortality and quality of life for a given drug are summarized as a cost in dollars per quality-adjusted life year (QALY), a composite measure of disease burden. Paediatric clinical and economic data are often lacking, making it difficult to perform these calculations. Even when paediatric data exist, there are unique difficulties in cost-benefit calculations for children. Valid and reliable instruments for assessing quality of life at different ages and stages of development and with different diseases are often nonexistent.

Historically, HTA processes have been based primarily on costs and savings within the healthcare system, often without considering the significant savings associated with improved patient life outcomes in other domains outside of healthcare. Many drug treatments impact the levels of required support for children at school or in their communities. For example, patients with attention deficit-hyperactivity disorder (ADHD) often need supplementary, sometimes costly, special education supports and services (27). Drug treatment may reduce these needs. A standard HTA for an ADHD drug would not necessarily capture these impacts.

Also, HTA processes currently focus only on the child, without considering the significant burden of a child's disease on caregivers and families. Parents often miss work to care for a child, which can negatively impact their own quality of life. Paediatric drug therapy can produce a valuable return on investment by improving a caregiver's quality of life and ability to work, two important benefits that are also not measured by traditional HTA processes.

Lastly, when evaluating paediatric formulations, public insurance plans often compare costs and benefits with compounded medications. This comparison equates compounded with commercially prepared formulations, without considering the many safety, quality, and palatability benefits of commercial medications. Other countries have mechanisms to consider the special needs of children in their drug approval and reimbursement processes, but Canada does not.

All of these issues may lead HTA bodies to recommend excluding important paediatric drugs from drug formularies. An appropriately child- and youth-focused pharmacare system would recognize the significant returns on investment provided by paediatric drugs, including impacts beyond the health care system and the individual patients. Coverage should not be defined by existing standards, but must take the many, unique benefits of drug therapy in children and youth into account.

CONCLUSION

Pharmacare has the potential to improve the health of all Canadians, reduce existing health disparities, and improve access to prescription drugs. Any prospective pharmacare system must address the unique pharmaceutical needs of children and youth and correct decades of accepting lower standards of care for young people. Policy makers must use this opportunity to strengthen Canada's health care system and ensure that all children and youth have access to safe, effective prescription drugs.

RECOMMENDATIONS FOR POLICYMAKERS

To ensure that pharmacare policies address the unique health care needs of Canadian children and youth, the Canadian Paediatric Society (CPS) and the Goodman Pediatric Formulations Centre (GPFC) recommend:

1. Federal, provincial, and territorial governments should develop and implement pharmacare plans that mandate universal, comprehensive, portable prescription drug coverage for children and youth. Specifically:
    - Coverage should be consistent across Canada, eliminating the 'postal code lottery' and ensuring that children and youth maintain comparable coverage when they move between jurisdictions. Child refugee claimants covered under the Interim Federal Health Program should be fully included.
    - Engagement with First Nations, Inuit, and Métis partners is necessary to ensure that the knowledge and perspectives of Indigenous peoples is integrated in planning and implementation.
    - Coverage should include medications for children with rare diseases.

2. The federal government should develop a comprehensive, evidence-informed, national list of paediatric drugs for inclusion in a national pharmacare program. All children and youth should have access to drugs on this list.

    - Decisions to include drugs must be guided by paediatric experts, as informed by the best available paediatric evidence, and not solely defined by Health Canada's approved indications.
    - A permanent, dedicated, appropriately funded Expert Paediatric Advisory Board at the Health portfolio level should be established and charged to lead this expert panel.
    - The added value of commercial paediatric formulations over compounded preparations should be recognized by prioritizing them on the national list of paediatric drugs.
    - The federal government should also support the development of detailed dosing information and compounding instructions for drugs that are not commercially available as child-friendly formulations. This policy would enhance uniformity of practice and reduce the need for hospitals and pharmacies to individually develop dosing or compounding instructions.

3. Health Canada must modernize processes for drug approvals and oversight to expand access to paediatric medications and ensure appropriate paediatric labelling for all medications marketed in Canada.

    - Health Canada should proactively request manufacturers include paediatric-specific data when paediatric use can be expected or anticipated (as is currently done by regulators in the USA and Europe), and provide manufacturers incentives to do so.
    - Health Canada should create an expedited process to support the review of paediatric products that have been labeled, marketed, and widely used in trusted foreign jurisdictions.

4. The federal government should support paediatric drug trials, cost-effectiveness research in children and youth, and the development of commercial paediatric drug formulations.

    - Paediatric outcomes data (e.g., medication safety, comorbidities, duration of use) should be gathered and studied to better inform drug development and reimbursement decisions.
    - Research budgets should include dedicated funding for paediatric drug studies proportionate to population size and reflecting the economic and social returns on investment from paediatric-focused research.

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