

RARE-SAP

Reforming the Special Access Program to Improve Access to Lifesaving Treatments for Critically Ill Children

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RareKids CAN | SickKids Child Health Policy Accelerator

Regulatory Reform to Advance Access to Essential Off-Market Therapies

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About RareKids-CAN

RareKids-CAN is a diverse national coalition of partners working to streamline and accelerate pediatric rare disease clinical trials and improve access to innovative treatments for children and youth across Canada.

About SickKids Child Health Policy Accelerator

The SickKids Child Health Policy Accelerator is the first and only hospital-based centre in Canada dedicated to improving child and youth health policy. We aim to close the gap between what is proven to improve outcomes for children, youth and their families and what is reflected in federal public policy.

Regulatory Reform to Advance Access to Essential Off-Market Therapies

Endorsements























































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Regulatory Reform to Advance Access to Essential Off-Market Therapies

Executive Summary

Children in Canada deserve access to the medications they need. Unfortunately, many safe and effective treatments are not available in Canada due to the country's relatively small market and limited commercial incentives available for manufacturers. As a result, some therapies are never introduced, others are withdrawn despite ongoing clinical need, and many innovative therapies reach Canadian patients only after approval in larger international jurisdictions. Pediatric patients, particularly those with rare diseases, are disproportionately impacted by these gaps, as they represent a small subpopulation within Canada's pharmaceutical ecosystem.

Health Canada's Special Access Program (SAP) was developed to address these challenges. The SAP enables healthcare practitioners to access drugs that are not marketed in Canada in emergency situations for the treatment of serious or life-threatening conditions. For many children with rare diseases, the SAP is essential to access life-sustaining and life-enhancing treatments.

While the SAP aims to expand access to treatment, the program also introduces administrative burdens that compromise timely care and lacks flexible, risk-based pathways that enable access to more novel treatments with emerging evidence of safety and efficacy in emergency situations. When access to a more novel treatment is denied, clinicians may be directed to establish a clinical trial for the single patient in need of care—a process that can significantly delay, and in some cases entirely prevent, the delivery of essential medical care. These delays have contributed to disease progression and, in the most tragic cases, preventable deaths.



Regulatory Reform to Advance Access to Essential Off-Market Therapies

To modernize the SAP and establish streamlined, risk-proportionate pathways for accessing therapeutic products unavailable on the Canadian market, Health Canada should:

1. **Eliminate the "fail-first" requirement that compels patients to try potentially less safe or less effective on-market therapies before accessing the optimal treatment through the SAP.**

2. **Develop streamlined, patient-centred pathways that facilitate access to essential therapies, including:**

   - **For therapies with a well-established history of use: Create a list of non-marketed therapies that are supported by robust clinical data, a history of safe and effective use, and established Canadian clinical practice, that are pre-approved for importation and use under a modernized SAP.**
   - **For therapies not included on the pre-approved list: Develop flexible and permissive access pathways that facilitate the importation and use of treatments with emerging evidence of safety and efficacy through a revised case-by-case special access framework with appropriate regulatory oversight.**

3. **Develop a modern, efficient, and transparent system that reduces unnecessary administrative burden and increases clarity for practitioners.**

Implementing these reforms will accelerate access to treatments available in other trusted jurisdictions without compromising the rigorous safety standards that define Canada's healthcare system. Enhancing transparency and implementing a risk-based regulatory approach will also align Canada with leading international regulators, ensuring that federal requirements are clear and federal oversight is proportionate. Ultimately, reducing unnecessary regulatory burdens and administrative barriers will enable more timely, equitable access to care and better support the practitioners who provide it.

Accessing Pediatric Therapies in Canada

Approximately 1 in 12 Canadians lives with a rare disease, and two thirds of them are diagnosed in childhood.[1] A rare disease is commonly defined as a life-threatening, seriously debilitating, or chronic condition affecting fewer than 1 in 2000 individuals.[1,2] More than 80% of rare diseases are genetic, and for many, no effective treatment exists.[2,3] **Unfortunately, even when treatments are available, they are often inaccessible in Canada due to the country's small market size, the even smaller pediatric sub-population, and a regulatory and reimbursement landscape with limited commercial incentives for manufacturers to seek and maintain market authorization.** As a result, children with rare diseases in Canada disproportionately rely on federal access mechanisms—most notably Health Canada's Special Access Program (SAP)—to obtain life-saving and life-sustaining medications that are otherwise unavailable on the domestic market.

Novel therapies, many of which are developed for rare diseases, frequently experience significant delays before entering the Canadian market; in some cases, essential life-saving and life-sustaining drugs are never commercialized in Canada at all. One recent study identified an average delay of 540 days between initial submission of a new drug to the U.S. Food and Drug Administration (FDA) or the European Medicines Agency (EMA) and a subsequent submission to Health Canada.[4] A second study found that approximately one third of new drugs approved by the FDA or EMA between 2016 and 2020 had not received approval in Canada by 2023, with approximately half of these therapies targeting rare diseases.[5] It is estimated that only 60% of treatments for rare diseases are authorized for sale in Canada, and most are approved up to six years later than in the United States and Europe.[1] Consequently, Canadian children with rare diseases are waiting years longer than their counterparts in the United States or the European Union to access new therapies, if access is achieved at all. For these patients and their families, delays are not abstract regulatory concerns; they carry **tangible risks of disease progression, irreversible loss of function or, in the most serious case, loss of life.**

Barriers to access are not limited to novel therapies. Children have distinct pharmaceutical needs compared to adults, including requirements for flexible, weight-based dosing and child-friendly formulations, such as easy-to-swallow liquids or rapidly dissolving tablets.[6] Unfortunately, many older, well-established drugs, including essential antibiotics and antiparasitic drugs manufactured in child-friendly formulations, have been withdrawn from the Canadian market due to limited commercial viability despite ongoing clinical need.[7] Without access to suitable commercially available formulations, pharmacists are required to compound medications for pediatric use, a process associated with an increased risk of dosing and preparation errors. Where compounding is not feasible, practitioners may again need to rely on federal emergency drug access programs to import the necessary therapies.[8]

Accessing Pediatric Therapies in Canada

The SAP was developed to help bridge these critical gaps by enabling access to therapies that are not commercially available in Canada. However, in its current form, the program is both administratively cumbersome and insufficiently flexible to meet the needs of patients with rare disease. Leading international regulators, namely the Therapeutic Goods Administration (TGA) in Australia and the Agence nationale de sécurité du médicament et des produits de santé (ANSM) in France, have implemented access frameworks with more streamlined, risk-proportionate pathways that minimize administrative requirements for low-risk, off-market therapies while ensuring timely access to more novel, emergency care. As Canada considers its own regulatory future, modernizing and strengthening risk-appropriate pathways—anchored in a thoughtfully redesigned SAP—will be essential to ensuring that children with rare diseases receive the medicines they need, when they need them, in time to optimize health outcomes.



Existing Pathways to Access Off-Market Therapies: The Special Access Program

Authorized under Food and Drug Regulations C.08.010 and C.08.011,[9] the SAP allows healthcare professionals to request access to a drug that is not available for sale in Canada in emergency situations. Health Canada defines an "emergency" as a "serious or life-threatening condition where conventional therapies have failed, are unsuitable, or unavailable in Canada."[10] The SAP receives over 12,000 requests per year, disproportionately serving children and people affected by rare disease, and enables access to both medications with a well-established history of safe and effective use as well as newer treatments that are not yet available in Canada.[11]

The SAP provides two pathways to facilitate access to non-marketed therapies. The majority of applications proceed through the patient-specific request pathway (Form A) in which practitioners must justify the clinical need for the medication, detail all prior treatments that have been trialled (or ruled out on clinical grounds), and provide supporting evidence on the drug's use, safety, and efficacy in the patient's specific context.[12] This evidence may include peer-reviewed literature, treatment guidelines, and/or documentation of authorization in other jurisdictions. Once completed, practitioners must fax the form to Health Canada for approval. If approved, a Letter of Authorization is issued by the Minister of Health, permitting the manufacturer to sell the requested drug to the practitioner, and the patient can receive the required treatment.

In 2022, Health Canada introduced new regulations establishing a second pathway, permitting SAP requests for "future-use" therapies to be stocked for use in medical emergencies (Form B).[13] By removing the requirement that each SAP request be tied to an individual patient prior to importation, the reform enabled the "pre-positioning" of a limited number of time-sensitive medications within hospital pharmacies. Practitioners, however, must still submit a justification comparable to that required for a patient-specific (Form A) request to access and/or replenish a pre-positioned drug.

Existing Pathways to Access Off-Market Therapies: The Special Access Program

Challenges with the SAP

Table 1. Key Challenges with the SAP Pathway

Issue	Description
Treatment delays	For all Form A/patient-specific requests, applications must be reviewed and approved before treatment can be accessed. This contributes to delays in essential medical care. Newer treatments require additional time for Health Canada review
Cumbersome case-by-case evidence requirements	Extensive patient-specific applications are required for drugs accessed through the SAP, including those with well-established use, safety, and efficacy profiles; products already approved for use in trusted foreign jurisdictions; therapies previously licensed in Canada; and medications recommended for use in a Health Canada-approved product monograph. Additional requests for information from Health Canada are also common, even for products that are accessed regularly.
Application redundancy	Individual applications must be prepared and submitted for each patient, even if the same treatment has been approved for the same indication, in similar patients, multiple times.
Time-limited approvals	All approvals are time-limited. For patients who require long-term treatment, practitioners must reapply at regular, frequent intervals to ensure uninterrupted care.
Clinically inappropriate "fail-first" therapeutic history requirements	Applications require evidence that all therapies available on the Canadian market have been considered and have failed (or found clinically unsuitable). This forces patients to potentially try less effective, less safe, and/or more expensive on-market options before accessing treatment through SAP.
Lack of access to novel therapies	A pathway to enable access to novel therapies under a flexible, treatment-focused, "special access" framework is not provided. When SAP access is denied, clinicians may be directed to establish a Single Patient Study—a clinical trial for one patient—to administer therapies that could be delivered safely and effectively under expert clinical supervision. The regulatory requirements to establish a clinical trial can be so burdensome that treatment becomes untenable.
Lack of transparency	Specific SAP approval standards are not public. It is often not clear why an SAP application was denied and/or directed to a research-focused pathway (Single Patient Study).
Loss of real-world evidence	Data collected through the SAP pathway is not accessible to researchers and practitioners for research and analysis purposes.

Existing Pathways to Access Off-Market Therapies: The Special Access Program

While the introduction of the future-use (Form B) pathway has decreased the time involved in accessing a small number of time-sensitive emergency use drugs, the SAP continues to contribute to delays in essential therapy and remains administratively burdensome for clinicians and pharmacists. The majority of applications require patient-specific justifications, compelling practitioners to complete time-consuming documentation to secure drug access, even when the treatment is well established, considered standard of care, and has been approved multiple times for an identical clinical scenario. Moreover, approvals are time-limited, forcing practitioners to reapply to secure ongoing access for each patient, as often as every 6 to 12 months, even if that patient requires the drug for a lifelong, chronic condition. The SAP's reliance on outdated administrative systems, including PDF forms and submission by fax machine, heightens practitioner frustration as they are inefficient and administratively cumbersome.

Cumulatively, the administrative burden associated with the SAP is so significant that some hospitals employ an additional pharmacist whose full-time role is dedicated to completing the necessary paperwork. In smaller hospitals, the administrative requirements of the SAP must be managed by already overstretched providers, straining the healthcare system and contributing to practitioner burnout. For community-based providers and pharmacies, these administrative requirements are often untenable, exacerbating geographic barriers to care.

Case study: Cumbersome case-by-case evidence requirements

Neuroblastoma is a rare pediatric cancer that develops in nerve tissue. Approximately 50 to 70 cases are diagnosed each year, most commonly in children under 5.[14,15]

Dinutuximab is a common chemotherapy treatment for high-risk neuroblastoma. The product has received Health Canada authorization and is commercially available in Canada. The Health Canada-approved product monograph for dinutuximab recommends that the drug be administered in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), a medication that supports bone marrow recovery during chemotherapy treatment.[16]

Unfortunately, GM-CSF is not available in Canada and can only be accessed through the SAP. As a result, each time a child receives standard treatment for high-risk neuroblastoma, the treating medical practitioner must submit a request and provide justification for access to a product that Health Canada has already recommended for this indication.

Existing Pathways to Access Off-Market Therapies: The Special Access Program

Case study: Time-limited approvals

Duchenne Muscular Dystrophy (DMD) is a rare neuromuscular disorder that affects approximately 70 out of every 100,000 people in Canada.[17] Diagnosed in early childhood, it is a lifelong condition that results in progressive muscle weakness and premature mortality. Average life expectancy varies from late adolescence to mid-30s.

Deflazacort is a drug used to prolong independent movement in children with DMD.[18] It is only available in Canada through the SAP.[18] Despite patients requiring access to deflazacort for life, the SAP requires physicians to reapply to the SAP at regular intervals to ensure uninterrupted treatment.

When requesting access to a drug through the SAP, Health Canada requires practitioners to demonstrate that all potentially effective on-market treatment options have previously been tried and found unsuitable for a patient.[10] Mandating a "fail-first" approach is fundamentally incompatible with practitioners' duty to deliver the highest standard of evidence-based care, as informed by current literature as well as thoughtful, individualized risk/benefit assessments. Moreover, this "fail-first" requirement causes significant moral distress for patients, families, and practitioners, who must manage the real risks associated with delayed access to optimal treatments, and the potential exposure to side-effects associated with therapeutic options available in Canada.

Case study: Clinically inappropriate "fail-first" therapeutic history requirements

Tuberculosis (TB) is an infectious disease caused by airborne bacteria that most often infect the lungs, but can also infect the brain and other organs. Infections can be latent or active and, if untreated, can cause serious disease or death. Although TB is uncommon in Canada, it continues to disproportionately impact Inuit, First Nations, and Métis populations and newcomers.[19]

Standard TB treatment involves a six-month course of a multi-drug treatment regimen. In countries with a higher TB prevalence, child-friendly formulations—such as palatable liquids and dissolvable tablets that allow for accurate, age-appropriate dosing—are widely available.[20] In Canada, however, most of these formulations are not available, as limited commercial demand makes market authorization unattractive to manufacturers. As a result, practitioners must rely on adult drug formulations that are produced in doses that may be inappropriate for children and that are difficult to swallow.

Existing Pathways to Access Off-Market Therapies: The Special Access Program

To access a child-friendly formulation of an essential TB medication through the SAP, practitioners must first prescribe the adult formulation (a tablet), ask caregivers to cut the tablet to administer the correct dose, and "prove" that the child will not tolerate the on-market treatment option. Practitioners have reported inconsistent dosing, poor adherence, and parents needing to physically restrain children to facilitate daily administration.[21] When safer, more appropriate pediatric options exist, requiring children to endure unnecessary distress and heightened risk of suboptimal treatment outcomes is unethical.

The "fail first" requirement not only impedes access to therapies that are clinically more appropriate, it can also undermine the use of more cost-effective medications when a considerably cheaper clinically suitable alternative could be accessed through the SAP. When public drug plans or insurers refuse coverage for these more expensive on-market options, the financial burden shifts to healthcare organizations—or to patients—placing additional pressures on already strained systems and families.

Case study: Clinically Inappropriate "Fail-First" Therapeutic History Requirements

Cystinosis is a rare genetic disease that affects multiple organ systems. Without treatment, cystinosis can lead to kidney failure, brain and muscle damage, and blindness. While there is no cure, cysteamine is an effective, lifelong treatment that can delay or prevent organ damage. Two forms of this important drug are manufactured: an immediate-release formulation marketed as Cystagon and a delayed-release formulation marketed as Procysbi.

While Cystagon has never been authorized in Canada, prior to 2017, Canadians affected by cystinosis were able to access Cystagon through the SAP. Cystagon costs approximately $10,000 per child per year.[22] However, when Procysbi was approved for sale in Canada in 2017, the SAP stopped approving requests for Cystagon, despite the fact that Procysbi was originally priced at more than $300,000 per child per year.[22] Although the price of Procysbi has since been lowered, Cystagon remains a significantly more cost-effective option for families, insurers, and taxpayers and should be accessible through the SAP.

A lack of transparency, in both previously approved medications under the program and the criteria used for access approval, contributes to additional frustration. Health Canada only recently published a "List of drugs most commonly requested" on the SAP website, following extensive advocacy by clinicians. Previously, limited publicly available information contributed to clinician uncertainty about which drugs were accessible through the program, resulting in inconsistent patient access. The "List of drugs most commonly requested" should be further improved by including the population and/or clinical indications for which the listed drugs have been approved. In addition, Health Canada's

standards for assessing "use, safety and efficacy" evidence are not comprehensively disclosed. Frontline clinicians and patients have reported that separate applications for the same medication for similar patients have been both refused and approved, suggesting that the criteria for evaluation are not clear.

Finally, and importantly, the SAP does not consider the risks associated with delaying or refusing access to a treatment in its decision making. In serious or life-threatening scenarios, the risk of trying a promising treatment that has limited safety data may be superior to allowing the disease to progress. Medical practitioners have argued that in these circumstances, the SAP inappropriately uses tenets of evidence-based decision making where evidence-based decision making is clinically inappropriate or unfeasible.[23]

Case study: Treatment delays

Variceal bleeding is a serious complication of end-stage liver disease (cirrhosis) caused by blood vessels rupturing in a patient's esophagus and stomach. The European Association for the Study of the Liver recommends terlipressin as a first-line treatment for variceal bleeding in patients with cirrhosis, as studies have shown it to be more effective over a longer period than octreotide, another drug commonly used to treat this condition.[24,25] Unfortunately, terlipressin is not available in Canada.

When a patient recently presented to a Canadian children's hospital with hematemesis (vomiting large volumes of blood) due to variceal bleeding, clinicians were required to trial all other medications and interventions (including complex surgical procedures aimed at banding the bleeding varices) before accessing terlipressin for this patient. While simultaneously providing this emergency care—including multiple blood transfusions—in a pediatric intensive care unit, physicians and pharmacists spent multiple hours preparing SAP paperwork and additional time awaiting SAP approval.

Once approval was granted, terlipressin, in combination with other care, successfully controlled the bleeding. The patient has since received a liver transplant and has returned to school without further complications. In clinical emergencies where the difference between life and death is minutes or hours, clinicians do not have time to trial less effective therapies, complete complex paperwork using outdated administrative systems, and wait long periods for approval.

Balancing Regulatory and Patient Risk

An effective access framework must balance the risks associated with providing access to unapproved therapies against the risks patients face when access is unavailable, delayed, or denied. At present, the SAP is principally designed to minimize potential harms associated with therapies that have not been reviewed and approved by Health Canada. This approach reflects Health Canada's mandate to assess the safety and efficacy of drugs at a population level, and to uphold consistent standards for market authorization.

However, this approach fails to accommodate for the individualized risk-benefit determinations that underpin clinical care, particularly in the context of rare, complex, or treatment-refractory conditions. In these settings, practitioners must weigh nuanced patient-specific factors, urgency, and evolving evidence when determining the most appropriate course of care—considerations that may not align with broader regulatory assessments, market dynamics, and commercial viability. As a result, the current regulator-centric framework overemphasizes the risks of treatment while under-recognizing the risks of inaction. To facilitate individualized patient-centred care, regulatory frameworks should enable, rather than constrain, the exercise of clinical judgement of highly trained physicians—particularly in urgent or life-threatening scenarios where timely decision-making is critical to patient outcomes.

The current conceptualization of risk rests on two flawed assumptions: that all non-marketed products are inherently novel or non-established, and that products available on the Canadian market necessarily present a lower risk to patients. In practice, many drugs accessed through the SAP have previously been authorized by Health Canada, have long-standing safety and efficacy profiles, are regularly used in Canadian clinical practice, and remain standard of care in comparable jurisdictions. Yet the program applies a uniform evidentiary threshold, requiring the same level of clinical justification for well-established therapies as for truly novel intervention. Similarly, the presumption that on-market therapies are always lower risk underpins the SAP's "fail-first" requirement, compelling the use of less appropriate or less effective therapies before access is granted. Together, these assumptions drive a rigid operational approach that places substantial burden on practitioners and, in some circumstances, compromises patient care.





Risk-Based Special Access to Novel Treatments

As medical science advances and precision therapies become increasingly available, the SAP must evolve to ensure Canadians can benefit from emerging treatments at a pace comparable to patients in peer nations. Achieving this will require a more flexible, treatment-focused "special access" approach that ensures timely access to novel therapies, including advanced therapeutic medicinal products (ATMPs) with credible emerging evidence of safety and efficacy.

The risks associated with novel therapies exist on a spectrum, ranging from well-established drugs repurposed for new conditions to first-in-human gene technologies.[26] As a result, regulatory oversight should be calibrated to clinical risk, the maturity of the product, and the strength of available supporting evidence.

ATMPs—including gene-, tissue-, and cell-based therapies—illustrate this diversity.[27] While often categorized collectively as "novel" therapies, their evidentiary foundations vary considerably. Certain ATMPs, such as CAR-T therapies, as well as other innovative modalities such as antisense oligonucleotides, are reaching a level of clinical and manufacturing maturity that can support safe and effective use in appropriate clinical contexts, including treatment-focused settings outside traditional clinical trial pathways. Yet, it remains appropriate that more experimental technologies, such as first-in-human gene therapies, be delivered through Health Canada regulated clinical trials while evidence of safety and efficacy is generated.

When considering a novel treatment, clinicians weigh the potential risks of therapy against the risks of disease progression in the absence of treatment. For patients and families who have exhausted all other options, the risks associated with potential disease progression may outweigh the uncertainties of an innovative treatment, particularly when there is credible emerging evidence of benefit. In these cases, treatment-focused special access pathways that support clinician-led decision-making are essential to enable timely and responsible care.

Currently, clinicians seeking access to unauthorized novel therapies that fall outside the scope of the SAP may be required to pursue a Single Patient Study (SPS), also known as an Open-Label Individual Patient (OLIP) study. An SPS is a formally regulated clinical trial under Canada's Food and Drugs Regulations designed for a single participant, in which a specific therapy is administered under a prospective research protocol with defined monitoring, data collection, and reporting requirements.[9]

While SPSs play an important role in Canada's research ecosystem by enabling clinicians and researchers to generate evidence for experimental therapies, especially for rare diseases, they are not an appropriate approach when the primary objective is patient care rather than knowledge generation. In purely clinical scenarios the SPS requirements impose significant, sometimes untenable, burdens on hospitals and clinicians that exceed what is necessary for delivering safe and effective

care to individual patients. This "trial-ization" of care introduces substantial regulatory, administrative, and financial obligations, delays access to urgently needed therapies, and is often disproportionate to the level of clinical risk. In the absence of dedicated funding, requiring an SPS can completely obstruct the provision of novel therapies that could otherwise be safely administered under close clinical supervision.

As currently structured, neither the SAP nor SPS systems provide a sufficiently flexible and proportionate mechanism to access novel therapies with emerging safety and efficacy evidence. SAP approvals often rely on prior authorization or substantial clinical use in other jurisdictions, while SPSs subject treatment-focused care to research-oriented requirements. International experience demonstrates that treatment-focused special access pathways can enable responsible access to therapies supported by emerging evidence, while maintaining appropriate safeguards. Such approaches are particularly important for individuals living with rare disease, who frequently face uniquely complex barriers to access clinical trials and innovative treatments. A risk-based framework that calibrates regulatory requirements to the degree of clinical and product uncertainty would help address this gap.

Case study: Lack of access to novel therapies

Virus-Specific T-Cell (VST) therapy involves the administration of specialized immune cells that have been trained to recognize and eliminate specific viruses. It is used to treat severe or treatment-resistant viral infections in immunocompromised patients. VSTs are supported by a substantial body of clinical evidence demonstrating safety and effectiveness but are not available on the Canadian market.[28] Patients who require VST therapy are often critically ill, and treatment must be delivered within a short timeframe to achieve optimal outcomes.

In Australia, VSTs have been available to patients through the Special Access Scheme (Categories A and B) for more than a decade. A recent analysis of patient outcomes in Australia and New Zealand over the last 15 years found 65% of patients showed definitive clinical improvement with no serious adverse reactions.[29]

In Canada, urgent access to VSTs can only be facilitated under an SPS protocol, even when the primary purpose of administration is clinical care rather than research. Establishing an SPS requires considerable time and administrative effort, creating significant barriers to access for critically ill patients. Multiple clinicians have reported cases in which patients deteriorated—and in some instances died—while efforts were underway to establish an SPS to deliver VST therapy.

International Approaches

Canada is not alone in facing the challenges posed by its small pharmaceutical market. Most similarly sized developed nations have created pathways to facilitate access to non-marketed drugs for patients with serious or life-threatening conditions. Leading global regulators have developed streamlined pathways to effectively and efficiently facilitate safe and appropriate access to non-marketed therapies, without undue administrative burden. These jurisdictions have also developed flexible emergency or "early access" pathways to provide access to innovative treatments while maintaining appropriate regulatory oversight.

Australia

In response to the challenges posed by its relatively small population, the TGA has developed a streamlined Special Access Scheme (SAS) that provides rapid access to essential therapies with modern and efficient regulatory oversight. The SAS also provides for the provision of novel, off-market treatments with risk-sensitive clinical and compliance requirements.

The SAS includes three distinct pathways:[30]

Table 2. TGA's SAS Pathways

Category A: For seriously ill patients in emergency scenarios	A medical practitioner may prescribe a non-marketed product and treat an individual patient without prior approval from the TGA. The TGA must be notified within 28 days.
Category B: For non-emergency clinical scenarios involving a product without an "established history of use"	A licensed medical practitioner with relevant expertise must request approval for a non-marketed therapy from the TGA before treating the patient. The TGA responds to requests within 2 to 5 days.
Category C: For products with an "established history of use"	A medical practitioner may prescribe and treat an individual patient with any therapy included on a dedicated list of special access therapies without prior approval from the TGA. The TGA must be notified within 28 days, and no further notifications are required to continue treatment.

Australia's SAS has distinct advantages over the current Canadian SAP. The "established history of use" (Category C) list drastically reduces the administrative burden on healthcare providers who need access to well-established drugs that are not available on-market in Australia. Medical practitioners can access these products without needing to first "seek permission" or justify use. This provides regulatory flexibility commensurate with clinical risk, recognizing that these products are supported

by longstanding safety and efficacy data. Practitioners notify the TGA of use within 28 days, and the single notification is sufficient should the patient require the same medication for the same indication indefinitely, eliminating unnecessary, repetitive paperwork.

Furthermore, Australia's emergency pathway (Category A) provides a more permissive Special Access system that tolerates a higher level of risk in clinical emergencies. When a patient is critically ill and has exhausted all other options, delaying treatment—or withholding it altogether—may pose an equal, or greater, risk than administering a treatment supported only by emerging evidence. Australia's system explicitly recognizes this balance and places trust in the treating physician. Accordingly, consideration should be given to how a more permissive approach could be translated into the Canadian context.

France

France's ANSM and Haute Autorité de Santé (HAS) have recently implemented a series of reforms to their Compassionate and Early Access programs to streamline administration, accelerate patient access, and leverage real-world evidence in support of patient care.[31,32] The result is an efficient system with distinct pathways to serve patients with different needs.

Table 3. ANSM's Compassionate and Early Access Program Pathways

Compassionate Access Pathway: For non-marketed products[33]	A medical practitioner with relevant expertise must request approval for a non-marketed therapy from the ANSM before treating the patient. Requests are submitted through an electronic system. Requests are approved for one year and are renewable.
Compassionate Access Pathway: For products previously authorized for compassionate access[33]	Products that have previously been authorized for compassionate access are listed on a publicly available reference list.[31] A medical practitioner must submit a request via an electronic system and attest the patient meets the eligibility criteria prior to treatment. Approval is immediate. Requests are approved for one year and are renewable.
Early Access Pathway: For innovative products prior to market authorization and/or finalization of public drug coverage[32]	A manufacturer must apply to the ANSM and HAS to establish an early access program (up to 3 months for approval). Once established, medical practitioners must request access for their patient prior to treatment. Requests are approved for one year, and approvals are renewable. Practitioners must comply with relevant reporting and data collection requirements.

International Approaches

Similar to the Australian system, the ANSM has developed transparent and efficient approval processes for accessing essential unauthorized drugs. The publicly accessible reference list of drugs authorized for compassionate access provides clarity for medical practitioners on which treatments are accessible and the level of clinical justification required for approval. While approval is still required prior to treatment, the use of an electronic system streamlines the process, reducing treatment delays. Innovative therapies can be accessed via the Early Access Pathway.[34,35] Canada should learn from its international peers and develop efficient systems for accessing low-risk unauthorized therapies, and flexible access pathways for innovative therapies.



International Approaches

Case study: Comparison of off-market access pathways for essential medicines

Diazoxide is a well-established medication used to treat low blood sugar (hypoglycemia) when the body produces too much insulin (hyperinsulinism). It is used in the standard care of tumors of the pancreas, certain cancers, rare genetic disorders (e.g., Prader-Willi syndrome), and newborn hyperinsulinemic hypoglycemia. It is available in the United States in both liquid and tablet form, and both forms are listed on the World Health Organization List of Essential Medicines for Children.[36]

Liquid diazoxide was first authorized for sale in Canada in 1984; however, in 2006, it was withdrawn from the Canadian market by the manufacturer for commercial reasons.[37] Liquid diazoxide was also withdrawn from the market in both Australia and France. The table below illustrates the excessive regulatory burden placed on Canadian physicians who need to access liquid diazoxide, as compared to their French and Australian counterparts.

Jurisdiction	Regulatory requirements for access to liquid diazoxide
Canada	1. Complete an 8-page paper form providing information on the patient's condition, medical history, patient-specific justification for liquid diazoxide, document all prior treatments that have been tried (and failed) and/or ruled out 2. Fax the completed form to Health Canada 3. Wait for approval 4. Receive a Letter of Authorization from Health Canada 5. Import drug 6. Treat patient 7. Provide a summary of treatment outcomes to Health Canada 8. Repeat process for further access
Australia	1. Import drug 2. Treat patient 3. Complete a 1-page online notification form including patient demographics, and the name and amount of drug accessed. No additional paperwork is required for additional medication, provided it is for the same patient and indication.
France	1. Complete a 1-page online form and attest that the patient requires liquid diazoxide for hyperinsulinism (tick box) 2. Download automatically-generated letter of authorization 3. Import drug 4. Treat patient 5. Report on treatment outcomes (if required) 6. Repeat process for further access

Policy Recommendations for Health Canada

Canada's SAP, as currently structured, does not provide patient-centred access to important, often life-saving, non-marketed medical therapies. Canada urgently needs a modernized SAP with efficient, streamlined pathways to enable access to therapies with well-established histories of use, as well as flexible pathways to facilitate access to more novel treatments under a regulatory framework based on treatment, rather than clinical trial.

To achieve these objectives, Health Canada should immediately implement the following policy recommendations:

1. Eliminate the requirement for patients to fail available on-market therapies prior to accessing the optimal treatment through the SAP.

The current expectation that patients must first try and fail therapies available on the Canadian market before being considered for access through the SAP can result in clinically inappropriate and ethically problematic treatment decisions. Access determinations should be guided by the therapy most likely to benefit the patient, rather than by whether or not an alternative product is commercially available in Canada.

Requiring patients to try potentially less suitable, less safe, or less effective therapies before accessing the optimal treatment may expose them to avoidable adverse effects, delay effective care, and increase the risk of disease progression. In pediatric populations, this requirement can be particularly problematic when available products are poorly suited to a child's age, developmental stage, or clinical needs. It may also burden patients, families, and the healthcare system by supporting the use of treatments that offer inferior clinical outcomes and lower overall value.

Patients should not be required to fail an inferior treatment simply to satisfy a regulatory process when a more appropriate therapeutic option is already available.

2. Develop streamlined, patient-centred pathways that facilitate access to the full range of therapies essential for providing healthcare in Canada.

- **For well-established therapies not available in Canada: Create a list of therapies with well-established histories of safe and effective use that are pre-approved for importation and use under a modernized SAP.**

  Modelled on Australia's SAS, the creation of a list of non-marketed therapies pre-approved for importation and use would establish an efficient, risk-proportionate pathway for accessing safe, effective medications with long-standing histories of use. By removing the need for case-by-case review, pre-authorization would reduce administrative burden for both healthcare practitioners and bureaucrats while accelerating timely access to

treatment for patients. At a time when the federal government is seeking to reduce red-tape and improve system efficiency, this reform is a low-risk, pragmatic way to create capacity in the healthcare system while optimizing care outcomes.

List Development and Maintenance

To ensure the list of non-marketed therapies pre-approved for special access remains clinically relevant, Health Canada should:

- Establish the list, including the development and disclosure of clear inclusion and exclusion criteria, in consultation with researchers, practitioners, and patients with lived experience. Inclusion criteria may include products that:
  - Have previously been authorized for sale in Canada but have been withdrawn by the manufacturer for commercial reasons (e.g., Cyclomydril eyedrops for neonates);
  - Have been recommended for use by Health Canada in combination with a product available on market (e.g., dinutuximab and GM-CSF);
  - Have an extensive history of use, a well-documented safety profile, and a history of prior approval by the SAP (e.g., albendazole, diazoxide);
  - Are specifically formulated for safe administration in certain populations (e.g., infants) where the adult form has been approved by Health Canada; and
  - Have regulatory approval in trusted foreign jurisdictions.
- Review the list at regular intervals.
- Publish the list, together with a list of trusted manufacturers and licensed establishments, on Health Canada's website.

Oversight and Monitoring

Recognizing the valuable role Health Canada plays in the regulatory oversight of health products in Canada, the following reporting requirements should be implemented:

- Healthcare practitioners must report the amount of product prescribed, the number of patients treated, and the relevant treatment indications within 120 days of accessing a pre-approved product.
- Manufacturers and licensed establishments must report the amount of product(s) they have supplied every 120 days.
- Consistent with current adverse event reporting for all therapeutic products in Canada (including both on-market and off-market therapies), healthcare practitioners must report any severe and unexpected adverse events attributed to products within 30 days of patient outcome.
- Manufacturers and licensed establishments must report any product defects immediately.

Policy Recommendations for Health Canada

- **<u>For case-by-case requests:</u> Develop flexible, patient-centred access pathways that facilitate the delivery of novel treatments with emerging evidence of safety and efficacy under a revised "special access" framework with appropriate regulatory oversight.**

  While the list of therapies pre-approved for importation and use will streamline availability of many routinely accessed therapies, the SAP's case-by-case assessment pathway will remain necessary for more specialized or higher-risk authorizations. Within the case-by-case assessment pathway, the SAP must evolve to facilitate access to more innovative treatments, supported by emerging evidence, through a more flexible, patient-centred approach.

  To support this modernization, Health Canada should consult with practitioners and researchers to develop flexible, patient-centred access pathways that:

  - Accommodate the unique clinical needs of individual patients, especially patients with rare disease;
  - Evolve with both modern clinical practice and advancing medical science;
  - Include consideration of all forms of medical evidence including case reports, observational studies, and evidence derived from clinical practice; and
  - Explicitly consider the risks of disease progression should timely access be denied.

  One practical approach for Health Canada to consider would be the adoption of a structured clinician-led access framework for selected therapies supported by credible emerging evidence where the primary intent is patient care rather than research. Such a framework could be anchored by peer-reviewed treatment protocols and formalized expert consultation processes, reflecting practices that clinicians already routinely employ when evaluating innovative treatment options. With clearly defined eligibility criteria, expert review requirements, and proportionate requirements for consent, monitoring, and safety reporting, this approach could provide Health Canada with appropriate assurance of oversight while reducing unnecessary regulatory complexity. Importantly, it would complement—not replace—the SPS pathway by offering a more suitable mechanism for treatment-focused use of promising therapies when the generation of generalizable research evidence is not the primary goal.

Policy Recommendations for Health Canada

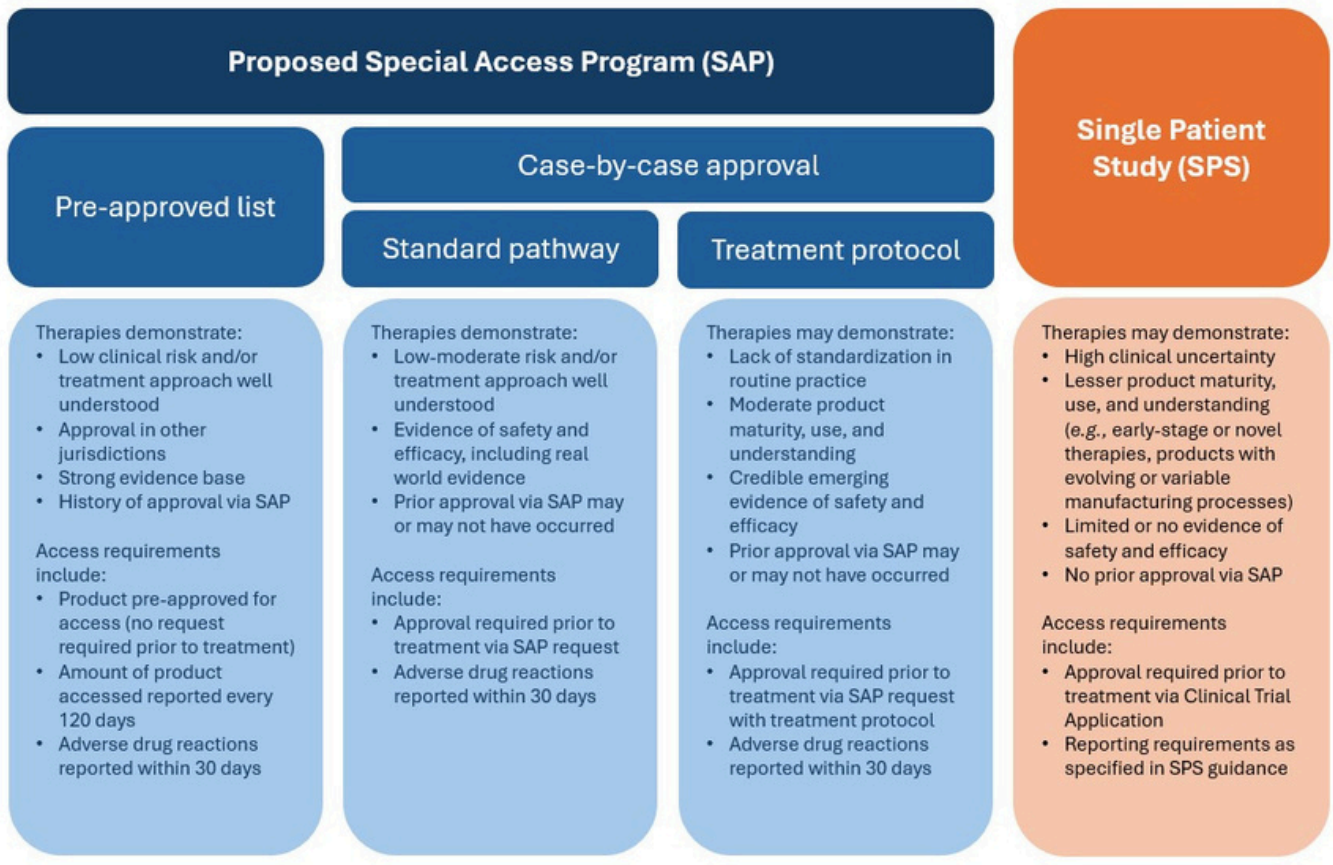
3. For all access pathways: Develop a modern, streamlined, and transparent system that reduces unnecessary administrative burden and increases clarity for practitioners.

Modernizing the administrative infrastructure of the SAP is an urgent priority. Health Canada should replace the current fax-based application system with a secure, centralized online platform to reduce administrative burden for practitioners, streamline application processing, and improve program transparency.

Greater transparency should also extend to case-by-case decision-making. When access to a treatment is denied, Health Canada should clearly communicate the reasons for denial to the practitioner, including any additional information required to warrant successful resubmission and the process for requesting review or appeal.

Finally, Health Canada should commit to publicly reporting, on an annual basis, the number of SAP applications received, approved, and rejected, along with the reasons for non-approval. This public reporting would help practitioners, patients, and policymakers better understand how the program is functioning and identify opportunities for ongoing improvement.

Figure 1. A modernized Special Access System



Conclusion

Rare disease patients in Canada face significant barriers accessing essential therapies that are not commercially available in Canada. While it remains important to incentivize manufacturers to bring necessary products to the Canadian market, our special access systems must also be flexible, patient-centred, and responsive to clinical need. Leading global regulators have demonstrated how streamlined access pathways can optimize timely care without compromising safety. Modernizing the SAP to reduce unnecessary red tape and establish flexible access pathways would provide Canadian patients timely access to therapies and ensure that advances in medical science benefit all Canadians.



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