From Burden to Benefit:

Streamlining Access to Non-Marketed Drugs in Canada

ISSUE BRIEF

Background

As a relatively small market, Canada faces gaps in drug availability. Some medications may never be brought to Canada by manufacturers, others are withdrawn for commercial reasons despite ongoing clinical need, and many novel therapies arrive here only after first being approved in larger jurisdictions. These gaps are particularly problematic for pediatric patients with rare diseases, given that they represent a very small subpopulation within our small market.

Health Canada's Special Access Program (SAP) and single patient studies (SPS) were developed to fill these gaps. The SAP enables access to drugs that are not marketed in Canada when they are proven to be safe and effective in other jurisdictions, while SPS offers access to newer, non-marketed therapies that may have more limited evidence. The SAP receives more than 16,000 applications per year, each for an individual patient. Data on the use of SPS is not publicly available.

While both programs aim to expand access to treatment, they also introduce unnecessary regulatory burdens that compromise timely care. **Canadians deserve access to safe medications with appropriate oversight, but also deserve prompt and effective treatment, free from unnecessary administrative obstacles.** In recent years, regulatory requirements associated with the SAP and SPS have significantly delayed and, in some cases, entirely prevented clinicians from delivering necessary care. These delays have led to disease progression and, in the most tragic cases, preventable loss of life.

Other small market jurisdictions have developed similar programs—and in many cases, have implemented modernized, fit-for-purpose access schemes that minimize red tape while maintaining safety and oversight. Canada can learn from these international examples and meaningfully reform the SAP to better serve patients and the clinicians who care for them.

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Current Situation

Special Access Program (SAP)

The SAP allows Canadians who have exhausted all treatments available in Canada to import medications from foreign jurisdictions. For many Canadians—particularly children living with rare diseases—it is the only viable pathway to access potentially life-saving or life-altering therapies.

Table 1. Current challenges with the SAP

ISSUE	DESCRIPTION
Treatment delays	• Applications must be reviewed and approved before treatment can be accessed, causing delays in access to essential medications. • Newer treatments require additional time for Health Canada review.
Application redundancy	• Applications must be submitted for each individual patient*, even if the same treatment has been approved for the same indication multiple times.
Repeat applications	• Approvals are time-limited. For patients requiring ongoing care, clinicians must re-apply to continue treatment.
Case-by-case evidence requirements	• Applications are required even if drugs have well-established uses, are marketed in trusted foreign jurisdictions, or have previously been licensed in Canada.
Therapeutic history	• Applications require evidence that conventional therapies have failed, are unsuitable, or are unavailable. Patients may be forced to try less effective, or less suitable, on-market options before accessing treatment through SAP.
SAP/SPS intersection confusion	• Specific SAP approval standards are not public. It is often not clear why an SAP application was denied and/or directed to SPS.

*In limited circumstances, a medical professional may request to store a drug accessed through SAP on-site for 'future-use', that is, not linked to an individual patient. However, this option is not feasible for all drugs and still requires repeat SAP applications to replenish stock.

Currently, medical professionals must apply for and receive Health Canada approval to access medical treatments not available in Canada. Clinicians must "ensure that the decision to prescribe the drug is supported by credible evidence available in relevant medical literature or provided by the manufacturer".[1] Once approved, clinicians must account for product use and report any adverse drug events.

The SAP receives more than 16,000 applications per year, many of which involve treatments that are well established, proven safe and effective, and approved for use in other jurisdictions. As a particularly vulnerable population, a disproportionate number of SAP requests are for pediatric patients. **For routinely accessed therapies, the SAP serves no function beyond introducing delays and unnecessary administrative burden.**

For clinicians, the SAP process is both time consuming and frustrating, especially when the requested drug is considered the standard of care. In such cases, the paperwork is an administrative formality that unnecessarily delays treatment. Each request demands coordination among physicians, pharmacists, and other team members, adding to the administrative load on already overstretched healthcare providers.

Single Patient Studies (SPS)

When an application for the SAP is denied, clinicians may be redirected to the SPS program.

An SPS enables access to drugs not marketed in Canada that have limited but emerging evidence of safety and potential efficacy for a specific patient. Like the SAP, an SPS is designed for an individual patient with a serious or life- threatening condition who has exhausted all other treatment options. However, unlike the SAP, an SPS must be run as a fully regulated clinical trial under Division 5 of the *Food and Drug Regulations*.[2]

Table 2: Current Challenges with Single Patient Studies

ISSUE	DESCRIPTION
Treatment delays	• Requires a comprehensive trial application and a team of experts to launch a regulated clinical trial, significantly delays access to care.
Division 5 burden	• Applications require submission of up to 5 modules, good manufacturing practices (GMP) evidence, chemistry & manufacturing controls (CMC) data, and pharmacovigilance and record-retention plans. These require time, significant regulatory expertise, and funding to assemble.
Chemistry-manufacturing-controls (CMC) expectations	• Newer drugs used may lack some CMC data. There is no allowance for risk-based justifications.
Application and amendment reviews	• Although submissions are subject to "expedited review," there are no guaranteed timelines • Treatment protocols are often amended to support patient care. This requires re-review and approval. Reviews only take place during regular business hours, regardless of the impact on patient care.
Post-authorization obligations	• Division 5 requirements continue throughout and after treatment. This includes staff Good Clinical Practice/Division 5 training, study monitoring, bilingual product labelling, 15-year record retention, and a "validated" electronic data capture system. These requirements are not necessary for safe treatment.
Treatment versus research	• The clinician leading the study is often the provider responsible for the treatment of the patient, introducing a potential conflict of interest. • Patients treated under an SPS may need to adhere to non-therapeutic elements of a research protocol when the intent is treatment.

Division 5 imposes strict trial requirements, originally designed for studies involving multiple locations and up to thousands of patients, that do not improve safety or oversight in single-patient treatment settings. In addition to operational burdens, the SPS model also raises ethical concerns: it can significantly delay access to medications and it places patients, families, and clinicians into a research framework when the sole intent is treatment, not investigation.[3]

Both SAP and SPS pathways are inadequate. Under the SAP, structural delays and bureaucratic inefficiencies delay access to safe and effective treatments. **When SAP access is denied, families and clinicians are left with gut-wrenching choices: forgo treatment, enroll the child or patient in a regulated research study, or uproot their lives to seek treatment outside of Canada.**

Learning from Global Leaders

Other small market nations have created efficient pathways to access non-marketed therapies in situations where patients have serious or life-threatening conditions. As a benchmark for best practice, the Australian model provides a proven approach with the potential for effective implementation in Canada.

Australia's Special Access Scheme (SAS)

The SAS is a regulatory framework that allows health practitioners to prescribe non-marketed therapies (i.e., those not included in the Australian Register of Therapeutic Goods) through three distinct programs:[4]

Category A:	For seriously ill patients: A medical practitioner may prescribe and treat an individual patient with notification to the Therapeutic Goods Administration (TGA) within 28 days.
Category B:	For all other clinical scenarios: A registered health practitioner with relevant expertise must request approval for a non-marketed therapy from the TGA before treating the patient. The TGA responds to requests typically 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 with notification to the TGA within 28 days.

In all cases, the prescribing practitioner takes responsibility for the use of an 'unapproved' therapeutic good and must report any associated adverse drug reactions.

Conclusion

Children and youth in Canada continue to face unnecessary barriers to timely, essential off-market therapies. **The federal government should dismantle current obstacles and build a streamlined, patient-centred system that better supports patients with rare, serious, and life-limiting conditions.** Acting now will not only accelerate the availability of vital treatments, but also reduce administrative burden, ease the immense stress on patients and caregivers, and reaffirm Canada's commitment to equitable, future-ready pediatric health care.

References

1 Health Canada. Health Canada's special access programs: Request a drug [Internet]. Ottawa: Health Canada; 2024. Available from https://www.canada.ca/en/health-canada/services/drugs-health-products/special-access/drugs.html.

2 Food and Drug Regulations (CRC, c. 870). (2025). Available from https://laws-lois.justice.gc.ca/eng/regulations/C.R.C., c. 870/.

3 Revon-Riviere G, Young LC, Stephenson EA, Brodeur-Robb K, Cohen-Gogo S, Deyell R, Lacaze-Masmonteil T, Palmer A, Parekh RS, Whitlock JA, Morgenstern DA. Ensuring access to innovative therapies for children, adolescents, and young adults across Canada: The single patient study experience. Pediatric Child Health. 2023 Apr 20;28(7):399-403. doi: 10.1093/pch/pxac122.

4 Therapeutic Goods Administration. Special Access Scheme (SAS): Guidance for health practitioners accessing unapproved therapeutic goods [Internet]. Woden (AUS): Department of Health and Aged Care; 2023 [updated 2024 Oct]. Available from: https://www.tga.gov.au/resources/guidance/special-access-scheme-sas-guidance-health-practitioners-accessing-unapproved-therapeutic-goods.