





# ACCESS TO UNAPPROVED MEDICINE: IS THIS AN OPTION FOR ME? (CANADA)



## Introduction

What if the medication that could improve or possibly save your life was just out of reach—available to others—but not to you? It's a reality that for many patients with rare and difficult to diagnose conditions. They may have exhausted all available commercial therapies for their disease, they may not have access to a clinical trial, or there may not be efforts by the manufacturer to bring the drug to the Canadian market. In some cases, patients find themselves travelling to the United States or Europe to gain access.

It is important to note that **clinical trials** are the preferred and optimal way in which to gain access to an unlicensed medicine. Patients who qualify to enroll in trials can obtain access to investigational medicines through their participation in the trial. However, some patients cannot participate due to geographical limitations, ineligibility based on the patient enrolment criteria, or there simply may not be a trial available in Canada for a particular medicine.

In the event that enrollment in a trial is not possible, there are other pathways through which a patient can access a much needed medicine. The process of obtaining treatments that are not currently available through the normal channels can be confusing for many patients and their families. In this toolkit, we will introduce what access programs are, how they work, and what patients and their families can do to further explore access options, specifically in Canada.

## What is “Named Patient Access” or “Compassionate Use?”

In most countries, regulations exist that allow for access to unlicensed medicines for patients with no alternative treatment options. Broadly referred to as **named patient access** or **compassionate use**, the names of these programs vary by country. For Canada, this narrow, request-based regulatory exemption is known as a **Special Access Program (SAP)**, and it is intended to allow for a legal pathway to obtain access to an unlicensed medicine or medical device outside of the clinical trial or commercial setting.

The Special Access Program addresses individual patient need and does not have any provision to treat groups of patients. Therefore, physicians need to make an individual patient request to the country health authority, known as **Health Canada**, each time they encounter a patient who they would like to treat with an unlicensed medicine. The physician needs to gain individual approval on a per patient basis and further obtain approval for any re-supply of the medicine that is needed over the course of the treatment.

## Introduction (cont'd)

As background, Health Canada is the government department within Canada that is the equivalent to the United States Department of Health and Human Services. The Special Access Program, regulated by Health Canada, was established to provide healthcare practitioners discretionary access to drugs and medical devices that are unavailable in Canada. These could be medicines that:

- Are still in clinical development;
- Are not currently approved or commercially available;
- May never be commercially available, but may still offer benefit for certain populations; or
- Are approved in other countries but not Canada.

In Canada, and under the Special Access Program, an unlicensed medicine is permitted for use only under specific conditions. Generally, access to unapproved medicines is restricted to the treatment, diagnosis or prevention of serious or life-threatening conditions when conventional therapies have failed, are unsuitable, or are unavailable.

Medicines requested through the Special Access Program may not be available anywhere in the world or could be commercially available in one or more countries outside of Canada. It is important to note that in all cases, it is the drug manufacturer or drug wholesaler who must first agree to make the unapproved drug available, and neither is under any obligation to comply with a request from a healthcare provider. Further, Health Canada has the ability to reject a request for access to an unlicensed medicine even if the manufacturer has agreed to supply it to the healthcare practitioner for use with the patient.

To learn more about the **Special Access Program and Health Canada**, go directly to the following website (<http://www.hc-sc.gc.ca/dhp-mps/acces/drugs-drogués/index-eng.php>), email [sapdrugs@hc-sc.gc.ca](mailto:sapdrugs@hc-sc.gc.ca), or call 613-941-2108.

# SECTION 1: FOR PATIENTS IN CANADA



Health Canada has established a Special Access Program (SAP) that permits importation of unlicensed medicines to treat patients with unmet medical needs. The program provides:

- Specific patient criteria to identify exactly which patients are eligible for access;
- Requirements for the manufacturer and the treating physicians; and
- Safeguards to protect patients since these medicines have not been approved for commercial use in Canada.

As previously stated, access to an unlicensed medicine through a clinical trial always is the preferred route. Therefore, the Special Access Program cannot be used in lieu of entering a clinical trial, and is to be utilized where clinical trial participation is not possible for the patient in need.

In all cases, the drug manufacturer or drug wholesaler has the final word on whether the drug will be supplied. Moreover, they have the right to impose certain restrictions or conditions on the release of the drug to ensure that it is used in accordance with the latest information available. For instance, the manufacturer may restrict the amount of product released, request additional patient information, determine charging requirements, and place conditions on shipping arrangements.

In addition, an authorization from Health Canada to import an unapproved drug is limited to a six month supply for chronic treatments. Subsequent access to the same drug for the same patient requires a renewal request submission to the program.

Special access to unlicensed medical devices is also restricted. Patients may access custom-made and unlicensed medical devices for emergency use or when conventional or approved devices have failed, are unavailable, or are unsuitable to provide diagnosis, treatment, or prevention. However, custom-made devices (i.e. devices created for a particular patient or use by a practitioner on a practitioner's orders) are a special case.



## SECTION 2: WHAT ARE THE RISKS?

There are many reasons why a drug may not be available in Canada or was never approved by the health authority and made commercially available for patients such as:

- Clinical trial results may be incomplete, not yet available or found to show no benefit for patients taking the medicine;
- The patient population in Canada may be too small for a drug company to be able to bring the drug to market due to the associated costs; or
- There could have been safety issues when the drug was being studied in animals or humans and development of the drug was discontinued.

Since the drugs obtained through the Special Access Program are exempt from **Canada's Food and Drug Act & Regulations** ([http://www.hc-sc.gc.ca/fn-an/legislation/acts-lois/act-loi\\_reg-eng.php](http://www.hc-sc.gc.ca/fn-an/legislation/acts-lois/act-loi_reg-eng.php)), there may be uncertainty associated with the safety of individual products. Any decision to take unapproved medicines should be directed by and made in close consultation with your physician. It is important to remember that there always is a chance that it may not work or that it may have serious side effects.

### Informed Consent

Before participating in the Special Access Program, individuals (or their guardians) need to review and agree to certain terms outlined in an **informed consent** document. This document is provided by the physician and details the specific information about the product and the associated risks of taking the unapproved medicine. The document must be signed by the participant or his or her legal guardian before gaining access to the medicine.



## SECTION 3: WHAT ARE THE COSTS?

Unlicensed medicines provided by a drug manufacturer or drug wholesaler may be provided either free of charge or at a cost for the drug. Funding for medicines falls under provincial jurisdiction, therefore the cost of the requested drug or device in comparison to the cost of the marketed alternatives in Canada is not part of the decision to provide it through the Special Access Program.

Each province and territory in Canada has a different program for subsidizing drug costs for their residents. These programs have different coverage eligibility criteria, payment criteria, and what drugs are covered.

Each province or territory makes its own decision about which drugs and other treatments it will cover. The decision is based mainly on economic factors, including its drug budget, population base, number of eligible residents with each disease or condition, as well as the cost of treatments and choice of treatments available.

Patients will need to determine if the costs of the unlicensed medicine or medical device will be funded by:

- The province;
- A private insurer; or
- The patient.

Note: Approval through the Special Access Program does not guarantee funding for the product. Dr. Julian Raiman, a Metabolic Physician in Toronto, explains:

“Our experience has been that SAP approval is for the drug itself and a patient can be approved without funding,” says Dr. Raiman. “The funding issue is that private insurance companies may not pay for SAP medications, therefore receiving these products can be cost prohibitive without public or private funding in place.”



## SECTION 4: POTENTIAL EMOTIONAL IMPACT

Pursuing an unapproved medicine will take time. Therefore, it is important to consider how this might affect your family. The process of obtaining unapproved medicines may change the quality of life for your loved ones, but there are some other important things you should consider:

- Am I willing to sacrifice the necessary time it will take to seek access to this medicine? And am I aware that this is quality time that could have been spent with my loved one?
- Will the rest of my family withstand the time out of work and/or loss of attention while this process occurs?
- Am I willing to accept that once I acquire the drug, my loved one's symptoms may have progressed significantly and the drug may no longer be a viable option?
- Am I willing to accept that even if access to the drug becomes available, it is possible that it will be ineffective or even have severe side effects?



## SECTION 5: GETTING STARTED



If you are thinking about exploring access to a treatment prior to its approval or to a drug that is not currently available in Canada, you should first discuss it with your physician. If your physician believes that an unapproved medicine potentially could benefit you, ask your doctor whether he/she is comfortable contacting the drug company or a drug wholesaler on your behalf. If the medicine has not been licensed anywhere in the world yet, it is available only through the drug manufacturer and not drug wholesalers.

However, if the medicine is commercially available in one or more countries, it can possibly be accessed through an international drug wholesaler. **International drug wholesalers** are businesses that are set up specifically to sell medicines, and therefore would offer charged access to drugs that are not currently approved in Canada in this situation.

Keep in mind that the pharmaceutical or biotechnology company that has developed and manufactured the drug may not be willing or able to offer it. Companies are not required by law to provide their drug to patients nor are they required to manufacture drugs for these purposes; it is solely at their discretion. There are many reasons why drug companies may refuse compassionate use / named patient requests, including:

- **Lack of Efficacy:** There may be little to no evidence that the drug will be effective for the specific illness.
- **Safety Issues:** The drug may have toxicity problems, which aren't public knowledge.
- **Production Capacity:** Drugs can be expensive and complex to manufacture. Further, the raw materials may be in short supply and therefore, the feasibility of manufacturing drugs in sufficient quantities to support an access program or to manufacture at all, may be limited.
- **Managing Drug Supply Priorities:** The main priority for the drug supply is to support a clinical trial or commercial supply in countries where the product is approved, and therefore, no drug may be available for patients outside of an ongoing trial.

If the physician has found a source that has agreed to supply the medicine, he or she will then contact Health Canada to request permission to import the drug. A requesting practitioner must explain why a particular unlicensed drug or device is required for diagnosis, treatment, or prevention for the patient. The practitioner must also identify the patient's medical condition and why conventional therapies or licensed devices have failed or are unsuitable. Lastly, he or she must discuss the use of the unlicensed devices or drugs. The application will be refused if the potential risks of the drug or device outweigh its potential benefits, or if a similar option already is licensed for sale in Canada.

## SECTION 5: GETTING STARTED

Health Canada may then contact the source (either the drug manufacturer or drug wholesaler) to confirm that it is willing to supply the medicine. Once confirmed, Health Canada will then issue a **manufacturer's commitment** form. The manufacturer or drug wholesaler must submit this form to Health Canada, which remains on file and serves to meet the requirements for any of the individual requests that come from physicians going forward. The manufacturer may only send the drug to the healthcare practitioner or an inpatient pharmacy, not to a retail pharmacy.

Health Canada's decision to authorize a request is made on a case-by-case basis, and consideration is based on:

- The urgency of the situation;
- The patient's medical condition;
- The availability of commercial drugs to treat the condition; and
- The information provided in support of the request by the physician that details the intended treatment plan for the patient and its safety.

The cost of the requested drug and the cost of the commercial alternatives are not part of the decision. Also, please be advised that Health Canada will only permit a certain amount of medicine to be supplied at any given time. Consequently, for chronic conditions, the physician will be required to periodically submit new requests to obtain more supply for the patient.

In considering the special access request, Health Canada does not conduct a comprehensive evaluation on the validity of the drug or medical device in formulation or assertions from the manufacturer with respect to safety, efficacy, and quality. Therefore, the authorization given for any drug or medical device by no means constitutes an opinion that it is safe or efficacious.

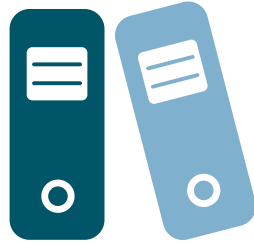
It is incumbent upon healthcare practitioners to ensure their decisions to recommend a drug or medical device are made after an appropriate risk/benefit analysis in the best interests of the patient and are supported by credible evidence found in the medical literature provided by the manufacturer. And, as is the case for any method of treatment, the practitioner must ensure the patients are well informed of the possible risks and benefits of the drug or medical device and have consented to the treatment. The Special Access Program also may request an accounting of the disposition of all quantities of the drug imported, so practitioners must keep accurate and accessible records.

Demand for access to unlicensed medicines in Canada can be highly influenced by its neighboring country, the United States. It is relatively common for a new medicine to be approved in the United States, but take months or years before it is approved in Canada (if at all). It is not uncommon for patients and advocates to be aware of the approved medicines in the United States by virtue of its proximity, and may feel that they too could potentially benefit from them. In some cases, these patients may even consider entering the United States to obtain access to these medicines.

### Other Ways to Learn More

In addition to talking to your doctor, patients and advocates can scan company websites to see if they have information about obtaining access to their medicines. Other helpful resources to learn more about access options are often found by engaging with other patients or patient advocacy organizations. They often provide valuable information through patient forums and discussion boards, and some of these organizations may even serve as an intermediary for the manufacturers of the drugs still under clinical investigation. Additional information about access programs and specific patient advocacy organizations can be found in the **Resource Guide**.

# APPENDIX:



## Q&A WITH ANDREW MCFAYDEN

Executive Director and Founder of The Isaac Foundation

While there is no known cure for MPS VI, a treatment does exist. An enzyme replacement therapy (ERT) that is designed to provide patients with a synthetic version of the enzyme they are lacking is available. Small doses of the therapy are infused into the patient's bloodstream on a weekly basis. The treatment for this orphan disease can range from \$300,000 per year for a small individual to \$1 million per year for a young adult. Due to the lack of an orphan drug policy in Canada, the treatment is only available to Canadian patients through the Federal Government's Special Access Program (SAP).

Prior to starting treatment, Isaac suffered from severe compression of his spinal cord that required the removal of a piece of his skull and a portion of his vertebrae. In addition, Isaac endured numerous other surgeries to treat complications of the advancing disease in his body. Since beginning his weekly infusions, Isaac's liver and spleen have reduced back down to a normal size, his rate of growth has increased, his heart function has improved, and his heart valve disease has stabilized. Furthermore, Isaac has had no further progression of his bone and joint disease, airway disease, and compression of his spinal cord. Andrew McFayden, Isaac's father, took some time to provide some advice to our toolkit readers.

### **When going through the SAP for access to treatment for your son, what were some challenges you faced?**

The challenges we faced initially weren't really with the SAP; it was with the physician. In order to apply to SAP, families need a physician to fill out the paperwork on their behalf, sign, and send it into the SAP team. I've worked with and spoken with many families who have had a difficult time convincing their physician to fill out the paperwork for various reasons. Initially, the hesitation we faced stemmed from a difference in beliefs on whether ERT was the answer or the appropriate course of action for our son; it was early on in the evolution of this treatment and there wasn't much data available to convince our physician that it was what we needed. After much back and forth, he was convinced to apply for us and we were approved in short order.

I've worked with families where physicians tell them they won't apply to SAP on their behalf due to the cost of the treatment, almost as if they are responsible for looking after the public purse themselves. I reiterate (and I will always hold true to this point very strongly), it's the physician's job to do whatever necessary to provide the best care possible for our kids, not to make political statements about the high cost of pharmaceutical drugs. If best practice is to fill out the SAP—whatever the cost of the drug—they should fill out the SAP and allow others to handle the financial aspect.

## APPENDIX: Q&A WITH ANDREW MCFAYDEN

### **What advice do you have for others attempting to go through this process?**

Be resilient. Never take no for an answer. For many of us, it's a question of life and death for our children. There is nothing I wouldn't have done to save my son and whatever barriers that were thrown up, I always believed they could be overcome. We're lucky we persevered. We have our son here with us because of it. Also, know that patient organizations are here to help. Families that have undergone similar struggles can't be dismissed.

### **From what I understand, you were able to secure financial support for your treatment as well, how do you believe you were able to accomplish that (so others may understand how they can increase their chances)?**

To secure reimbursement from provinces requires a concerted effort on many fronts: public lobbying coupled with behind the scenes meetings with Provincial Pharmaceutical Managers, politicians, and decision makers. A plan needs to be put in place and followed in order to achieve success. And you have to believe that things are possible. Stay the course on the plan you've put in place and never waver from your battle.



# APPENDIX: LIFE AFTER RECEIVING TREATMENT

Dane and Laura Sadownyk

Aleena was diagnosed with MPS, which means she lacks the enzyme needed to break down waste in her body, so it builds up in her organs, bones, and muscles. The drug Aleena needs isn't approved in Canada. With the help of The Isaac Foundation, the Sadownyk family secured access to the drug. Dane and Laura Sadownyk, parents of Aleena, speak of life after obtaining access to this treatment.

A few days after her very first treatment, Aleena explained to me how she felt in her own words. While running some errands, I asked Aleena how she was feeling after she had her first "enzymes" (she calls the treatment "enzymes" instead of medicine). I was trying to explain to her what energy was and if she felt she needed to rest as often, while doing my best as possible to explain this to a three and a half year old. I certainly did not engage in a lengthy conversation and continued driving down the road, when a short time later Aleena said, "Dad, I feel like I can dance now!" Needless to say, I almost pulled the car over as I was taken aback by what I had just heard. While trying to maintain my composure and in a broken voice I told her that she definitely can dance and we look forward to it. And many dances!

So where does that leave us now? Essentially, until a cure can be found, our schedule includes bringing Aleena to the Stollery Children's Hospital weekly to receive her treatment. Currently, as a family we are now getting used to this new "normal" that we are settling into and are grateful to see her receive this treatment. We see this as a "celebration of life" each time we bring her in for treatment, especially when she has noticed a difference within herself!

It's important for us to recognize the tremendous support and leadership that Andrew McFadyen from the Isaac Foundation has demonstrated. If it wasn't for the countless hours, sleepless nights, numerous calls, texts, tweets, emails, I'd hate to think where we would be at this point. But I'm pretty sure we wouldn't be here and for that we are forever grateful. His passion for helping children and families affected by MPS is contagious and we now share that passion and anticipate that "many hands will make for lighter work" as we work towards raising funds for a cure.

# RESOURCE GUIDE



## Health Canada's Special Access Program:

- **Health Canada's Special Access Program** (<http://www.hc-sc.gc.ca/dhp-mps/acces/drugs-drogues/index-eng.php>): This website contains information on the program, contact information, and relevant fact sheets and forms.
- **Bureau of Policy, Science and International Programs and the Special Access Program Working Group's "Special Access Program Issue Identification Paper"** ([http://www.phabc.org/files/Special\\_Access\\_Program\\_pas\\_ident\\_english.pdf](http://www.phabc.org/files/Special_Access_Program_pas_ident_english.pdf)): This document provides background information on the Program, regulatory authorities in the Program, the role healthcare professionals play in obtaining access to unapproved medicine, and other important information.
- **The Canadian Special Access Program** (<http://www.dww.com/dww/wp-content/uploads/2007/06/SpecialAccess-Chong.pdf>): Written by Janet Chong in 2007, this document outlines the Special Access Program and the associated procedure and conditions.

## Patient Advocacy Organizations:

- **Canadian Organization for Rare Disorders** (<http://www.raredisorders.ca/>): CORD is Canada's national network for organizations representing all those with rare disorders. It provides a strong common voice to advocate for health policy and a healthcare system that works for those with rare disorders.
- **Rare Disease Foundation** (<http://www.rarediseasefoundation.org/>): This organization focuses on linking basic science and clinical practice to increase the efficiency of rare disease research. Turn to this organization's Parent2Parent Resource Network for cross-disease information sharing and social support.

# RESOURCE GUIDE



## Obtaining Access to Unapproved Medicine:

- **Caligor Rx, Inc** (<http://caligorr.com/global-access-programs-gap/patients/>): Caligor Rx partners with pharmaceutical and biotechnology companies worldwide to develop, implement, and manage ethical and compliant Global Access Programs (GAP) that provide physicians and patients with access to unlicensed medicines. It also acts as an international wholesaler for physicians wishing to obtain unlicensed medicines.
- **Clinicaltrials.gov** (<http://clinicaltrials.gov/>): This website provides a listing of all clinical trials and current U.S. Expanded Access /Compassionate Use Programs. Users can search Expanded Access Programs being offered in the United States by typing “EAP” in the search box. There is also a drop down menu to search for Expanded Access Programs.
- **CheckOrphan Register of Access Programs** (<http://www.checkorphan.org/access-programs>) provides a listing of access programs available from manufacturers in various rare disease indications. It is intended to provide factual information and contact details for physicians wishing to seek access to a specific medicine that is not approved, for their patient in need.
- **Idis Inc.** (<http://www.idispharma.com/who-we-help/patients>, <http://www.idispharma.com/who-we-help/advocacy-groups>): Idis works closely with drug companies and patient communities to support access for patients in need. Its website has links to support both patients and patient advocacy groups as they explore their options to access medicines.
- **myTomorrows** (<http://mytomorrows.com/>): myTomorrows provides physicians and patients who are excluded from clinical trials access to innovative drugs in development. They focus on disease areas with unmet needs: oncology, neurology, psychiatry, and rare diseases. myTomorrows identifies innovative drugs, informs physicians and patients, and facilitates requests for access to these drugs in development.

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