Health Canada Santé Canada

Canada

Update - Special Access Program for Drugs Renewal Presentation to CAPRA

March 5, 2019

YOUR HEALTH AND SAFETY... OUR PRIORITY.

Special Access Program (SAP)

- Program background
- Regulatory requirements and history
- Program function and mandate
- Operational information
- Update on SAP renewal activities
- SAP statistics
- SAP next steps

Special Access Programme (SAP) for Human Drugs

- **Why do we have the SAP?**
  - An access mechanism for individual patients for drugs that are not available in Canada.
  - Not all diseases/illnesses in the world have prevalence in Canada (e.g. malaria, leprosy, snake bites).
  - Canada is a small market and not all approved drugs are available as they would be in larger jurisdictions such as the United States or Europe (e.g. drugs for orphan diseases, some pediatric formulations).

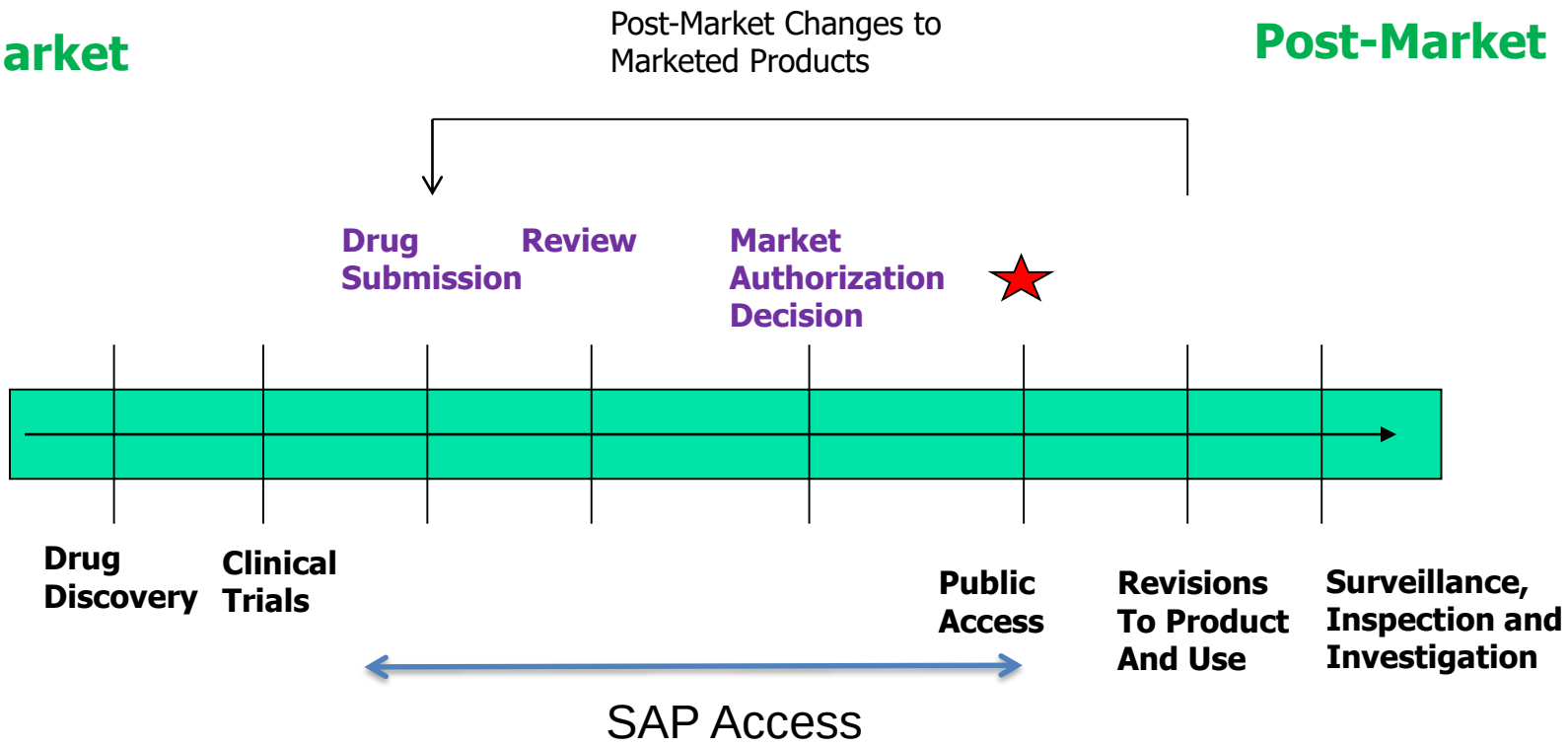
- **SAP operates based on a request-authorization scheme (exempt from Regulations)**
  - Practitioners initiate access requests for drugs that are unavailable in Canada to treat patients with serious or life-threatening conditions when other treatments have failed, are unsuitable or unavailable.
  - Practitioners are responsible for ensuring the decision to prescribe the drug is supported by credible evidence, and monitoring and reporting use.

- **SAP Policy**
  - Emergency access is exceptional and access to an unapproved therapy is optimally done through the context of a clinical trial.
  - Access to any drug through the SAP should be limited in duration and quantity to meet emergency needs only.

Special Access Programme (SAP) for Human Drugs

- **Why are drugs being requested via the SAP?**
  - The drug has never come to the Canadian market
  - The drug in under clinical development
  - The drug was pulled off the market by the manufacturer for economic reasons
  - There is no prevalence of disease in Canada, therefore no economic market
  - The drug was withdrawn from the market for safety reasons (e.g. Cisapride)

Lifecycle of a pharmaceutical product



The authority to regulate the safety, efficacy and quality of therapeutic products is derived from the *Food and Drugs Act*.

- SAP can be used to access drugs that are still under development (i.e., in clinical trials) or marketed in other countries but not in Canada
- Other access mechanisms: Urgent Public Health Need regulations; personal importation; compounding

Food and Drug Regulations

C.08.010. (1) The Director may issue a letter of authorization authorizing the sale of a quantity of a new drug for human or veterinary use to a practitioner named in the letter of authorization for use in the emergency treatment of a patient under the care of that practitioner, if

(*a*) the practitioner has supplied to the Director information concerning

(i) the medical emergency for which the drug is required,

(ii) the data in the possession of the practitioner with respect to the use, safety and efficacy of that drug,

(iii) the names of all institutions in which the drug is to be used, and

(iv) such other data as the Director may require; and

The authority rests with the ADM of the Health Products and Food Branch

Food and Drug Regulations

(*b*) the practitioner has agreed to

(i) report to the manufacturer of the new drug and to the Director on the results of the use of the drug in the medical emergency, including information respecting any adverse reactions encountered, and

(ii) account to the Director on request for all quantities of the drug received by him.

(1.1) The Director shall not issue a letter of authorization under subsection (1) for a new drug that is or that contains a restricted drug as defined in section J.01.001.

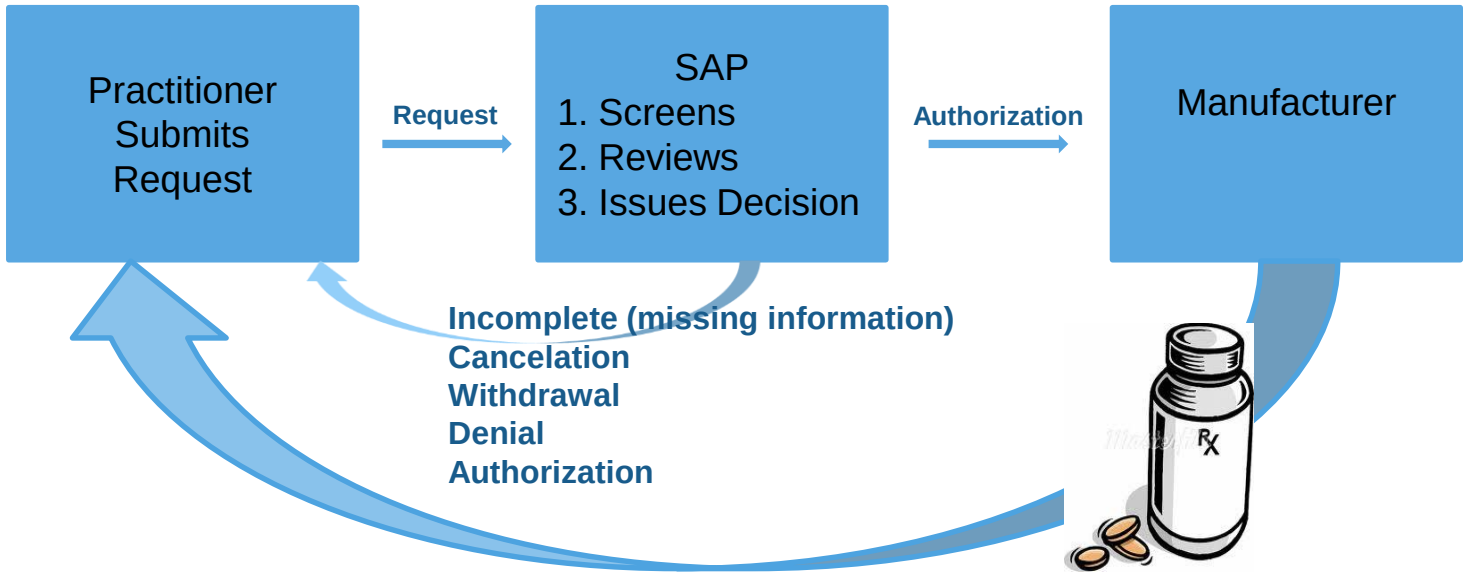
How does SAP work?

- SAP operates based on a request-authorization scheme
- The practitioner initiates an access request, for a specific patient, providing clinical information as to why an unapproved drug is required
- Practitioner is also responsible for ensuring the decision to prescribe the drug is supported by credible evidence, and must present such evidence
- SAP assesses the request (information provided, additional drug information at our disposal, internal consultation when required, etc.)
- For drugs new to the programme, need to contact the manufacturer, explain the SAP, inquire on their willingness to provide the drug (with or without restrictions), request information on the drug, require them to commit to inform us of important new safety issues, etc.
- An authorization allows the manufacturer to sell a specific quantity of an unapproved drug for the treatment of a specific patient.

SAP Requests Decision Making and Processing

Consideration of requests involves:

1. Determining whether the patient suffers from a serious or life-threatening condition;
2. Confirming that other therapies have been tried or at least considered, or unavailable;
3. Reviewing data respecting the use, safety and efficacy of the drug for the condition being treated.



Controlled substances require additional authorizations from the Office of Controlled Substances

Program activity

- 24hr service, 365 days of year
- 14,013 requests processed in 2018

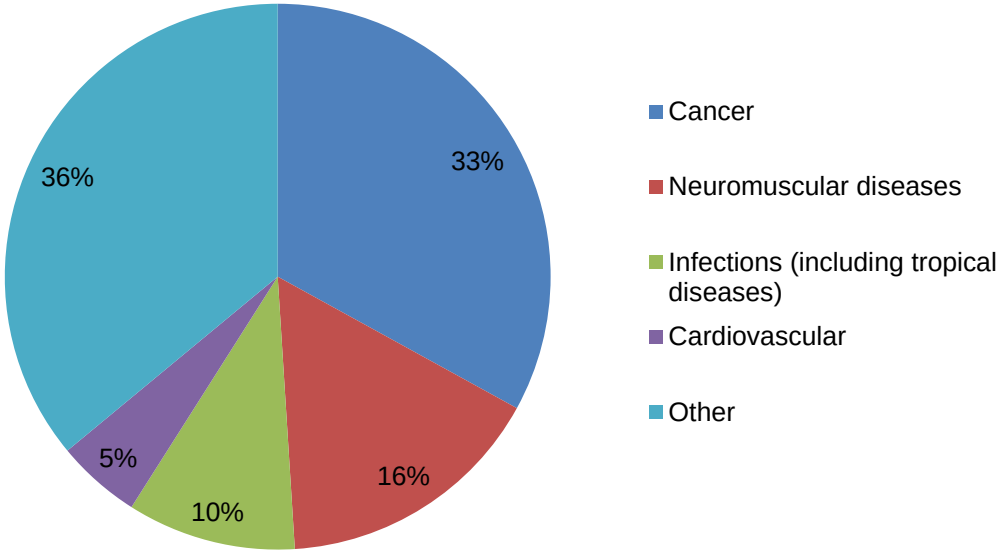
Based on an annual average

- ~100 requests handled each work day
- ~ 5,000 physicians make requests, every year
- Provides drugs for ~75,000 patients/year
- ~ 1,100 telephone calls per month in 2018
- 234 new drug requests

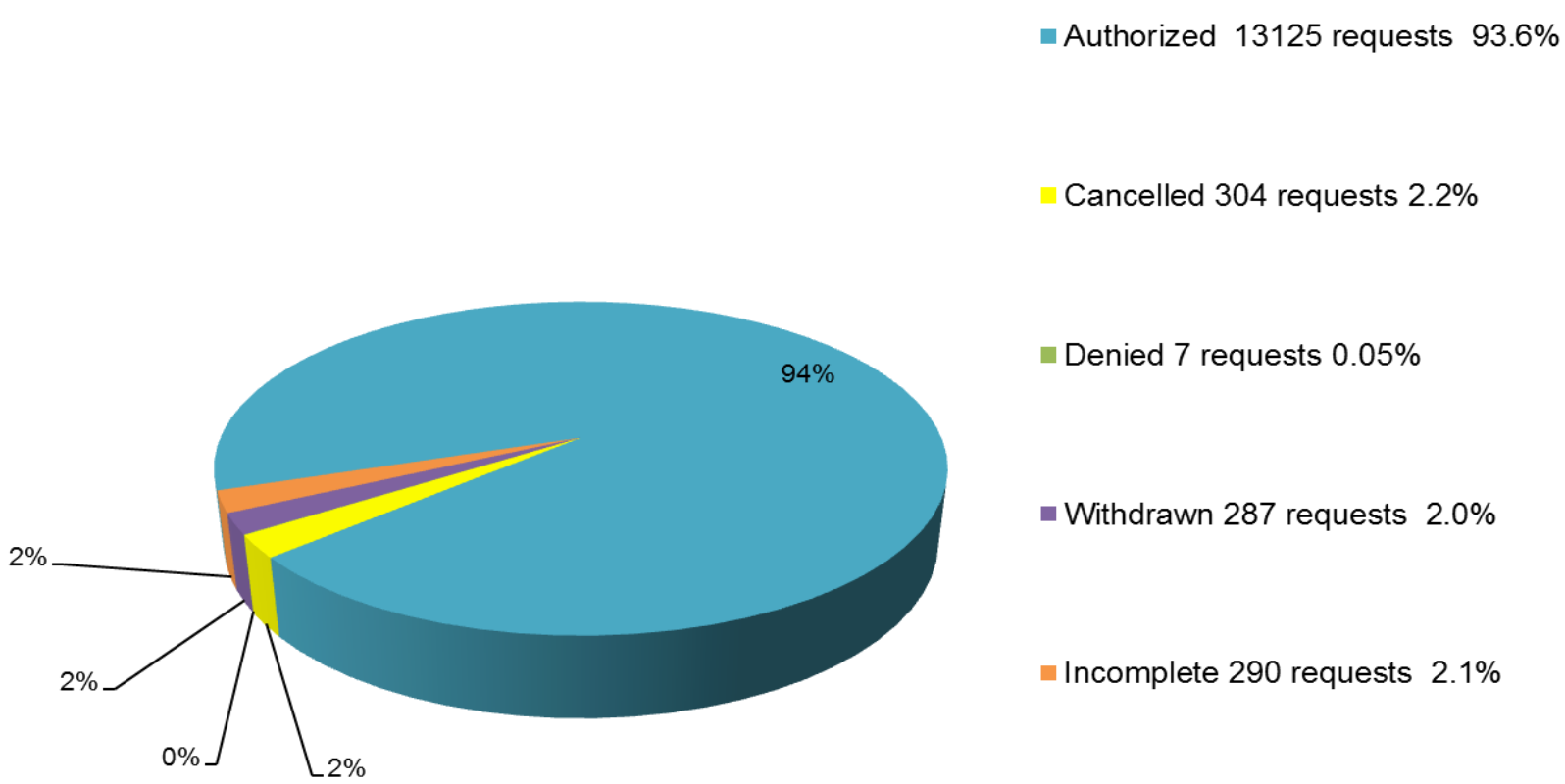
Program activity (cont'd)

Estimated values

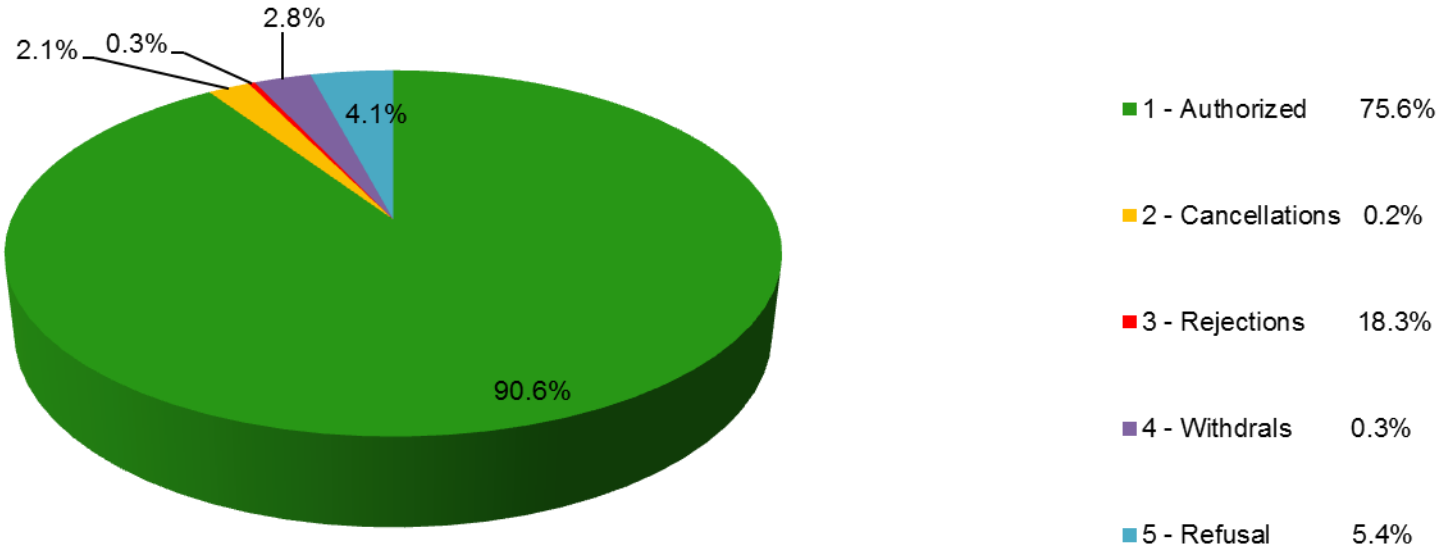
- Requests are received for a broad range of pharmaceuticals, biologics and radiopharmaceuticals
- Almost 80% of SAP drugs are approved in other countries, but not in Canada
- Breakdown of disease areas for which drugs are requested:



Request Activity for drugs: 2018



Request Activity for medical devices: 2018



SAP Renewal: Part of Health Canada's plan for transformation

- **Objective: An agile regulatory system that supports better availability of therapeutic products based on healthcare system needs**

Expanded collaboration with health partners

- ❑ Alignment of the Health Technology Assessment (CADTH) Review with Health Canada Review
- ❑ Implementing a Mechanism for Early (Parallel)Scientific Advice
- ❑ Use of Foreign Reviews/Decisions
- ❑ International Collaboration and Work Sharing in Reviews

More timely availability of drugs and devices

- ❑ Expansion of Priority Review Pathways
- ❑ Improving Availability of Biosimilars and Biologics
- ❑ Improving Availability of Generic Drugs
- ❑ Building Better to Digital Health Technologies
- ❑ Pre-Submission Scientific Advice for Medical Devices
- ❑ Special Access Programme (SAP) Renewal

Enhanced Use of real-world evidence

- ❑ Leveraging Data for Assessing Drug Safety and Effectiveness
- ❑ Strengthening Post-market Surveillance of Medical Devices

Modern and flexible operations

- ❑ Updated System Infrastructure
- ❑ Appropriate cost recovery framework
- ❑ Public Release of Clinical Information

What we have heard from Physicians and Pharmacists

- **SAP can be an administrative burden**:
  - Having to use a different mechanism to prescribe a drug to a patient.
  - Using a fax to submit requests.

- **Concerns about the information required and decision-making process:**
  - Sometimes there is a lack of understanding of the decision rationale.
  - Incomplete notifications: lack of clarity on the type of information needed.
  - Repeat requests must be supported by the same level of documentation.
  - More involved process and greater evidence needed compared to the Medical Devices SAP.
  - Need to provide patient outcome data and adverse reaction reporting.

What we have heard from Patients and Manufacturers

From patients:

- No guaranteed access to the drug they wish to access.
- Not being able to go directly to the manufacturer.
- Not being involved in the process (i.e., not having a say).
- Perception that Health Canada challenges the physician's judgement.

From manufacturers:

- Logistics of making small individual shipments (sometimes from overseas).
- Not being able to preposition product in anticipation of requests.
- Limited supply for drugs early in development.

SAP Renewal
To address from of the SAP challenges

Goal is to:

- Modernize our system (eliminate the use of faxes, duplicate manual data entry)
- Improve client services
- Ensure we have appropriate authority to support our current operations (e.g., requests for future use and pre-positioning)
- Provide new authorities (block release)
- Move longstanding frequently used drugs to a regulated pathway
- Provide a personalized service for urgent life threatening or end of life situations
- Create incentives for manufacturers to transition their drugs to a regulated pathway

Implemented changes as part of SAP renewal

- Increased access to clinical expertise
  - Hired clinical pharmacists
  - Medical practitioners from the Office of Clinical Trials
  - Became an official training site for U. of Waterloo PharmD students and interns

- Increased transparency on rationale for negative decisions since 2017
  - Clinical pharmacist calls physician before issuing a denial
    - Allows for discussion of rationale for decision
    - Provides the physician with an opportunity to be heard
    - Provides an opportunity to discuss other access options
    - Provides an opportunity to change the final decision
  - Led to less denials and increased physicians' satisfaction

- Ongoing work to encourage manufacturers of SAP drugs to bring their drugs to market
  - 2 of SAP's top 10 drugs received a Notice of Compliance in the past year

Reconsideration Process

The main objective of the Reconsideration Process (RP) is a means to provide the practitioner with an opportunity to be heard when the SAP is considering issuing a Denial.

- The requestor has an opportunity to request an independent review following the issuance of a denial recommendation.
- The RP includes two levels of evaluations: 1) the review of denial recommendation and 2) the reconsideration review.
- A requestor always has the option to withdraw their request.
- It should be noted that following a denial, a practitioner can resubmit a new request, should additional information become available.

Improvements due to implemented changes

2016		
Authorizations	Denials	Withdrawals
87.4%	2.0%	1.2%

2017		
Authorizations	Denials	Withdrawals
90.6%	0.3%	2.8%

2018		
Authorizations	Denials	Withdrawals
93.6%	0.05%	2.0%

Current work: SAP Web Site Renewal

- New web layout and revised content, to go live end of February
  - New landing page (SAP)
  - Separate service pages for Drugs, Medical Devices and Veterinary Drugs
  - User testing after the launch of the revised web site

- Revisions to the current SAP Forms and development of Block Release and Pre-positioning Forms
  - User friendly
  - Compatible with the new IT system

- Posting of Draft Guidance Documents and Forms to coincide with Canada Gazette I

- Posting of final Guidance Documents and Forms to coincide with Canada Gazette II publication
  - And any revisions needed following user testing

Current Work: SAP Renewal IT Solution (IP603)

Objectives of IP603:

- Improve the external client experience by providing a common platform with a single interface for both Drugs and Medical Devices Special Access Programs;
- Enable external clients to submit requests and receive decisions electronically as well as follow up electronically on the status of requests (including with mobile devices);
- Build on current data repositories to include automated workflow functionality from receipt of request to generating and sending decision letters;
- Include additional data storage and workflow to support product-related documents, electronic follow-up reports and communications tracking.

SAP Value-added and Comprehensive Services

SAP is an important source of drug information for health professionals in Canada. In addition to receiving and processing thousands of requests each year, SAP staff responds to inquiries from health care professionals, stakeholders, and the general public about the status of drugs in Canada, avenues of access, treatment options and manufacturers and distributors.

In 2018, SAP drugs responded to:

- approximately 3000 telephone inquiries,
- 500 emails, and
- 200 direct interactions with pharmacists, practitioners and provincial and territorial health officials

In 2018, SAP medical devices responded to:

- approximately 1400 telephone inquiries

Top 10 drugs on SAP in 2018

Product name	Therapeutic Use	Number of requests authorized
ETOMIDATE-LIPURO 2mg/mL	anesthetic: emergency intubation in emergency setting	717
**MECTIZAN (IVERMECTIN) 3mg	antiparasitic: variety of tropical parasitic diseases	499
*LEDERLON (TRIAMCINOLONE HEXACETONIDE) 20mg/mL	anti-inflammatory: juvenile arthritis	479
APO-CISAPRIDE (CISAPRIDE MONOHYDRATE) 10mg	prokinetic: severe gastrointestinal motility disorders	474
CALCORT (DEFLAZACORT) 6mg	anti-inflammatory: Duchenne Muscular Dystrophy	410
CAFFEINE CITRATE 20mg/mL	breathing difficulties in pre-mature newborns	333
IKOREL (NICORANDIL) 10mg	anti-anginal: severe uncontrolled angina	346
FOSCAVIR (FOSCARNET TRISODIUM HEXAHYDRATE) 24mg/mL	antiviral: CMV and other viral infections	321
BREVITAL SODIUM (METHOHEXITAL SODIUM) 500mg	anesthetic: anesthesia for procedures	294
DEPAKOTE SPRINKLE CAPSULES (DIVALPROEX SODIUM) 125mg	anti-seizure: pediatric patients who cannot take oral drugs	288

* Lederlon was marketed on October 4, 2018
** Ivermectin was marketed on November 6, 2018

Block Release

- To provide a regulatory mechanism and process that enables access to unauthorized drugs to prepare for (i.e., stockpiling) and/or respond to an imminent public or military health event, incident or emergency for a mass population.

- Public health officials will be those responsible for requesting, monitoring and reporting on the drug's distribution and serious adverse drug reactions (if they occur) of the drugs authorized under this mechanism.

- Public health officials are defined as the following:

(a) the Chief Public Health Officer appointed under subsection 6(1) of the Public Health Agency of Canada Act;

(b) the Chief Medical Officer of Health, or equivalent, of a province;

(c) the Medical Officer of Health, or equivalent, of a municipality;

(d) the Surgeon General of the Canadian Armed Forces; or

(e) the Chief Medical Officer of Public Health for the Department of Indigenous Services Canada.

Continuation of the Top 10 drugs on SAP in 2018

Of the 14,013 requests received in 2018 13, 125 were **authorizations.**

Etomidate-Lipuro was the most requested drug in 2018.

We authorized 717 Etomidate-Lipuro requests, represents 5.46% of those requests authorized in 2018.

If you total all top 10 drugs (4,161 requests) represents 31% of those requests authorized in 2018.

Summary

- It should be clear that drugs shouldn’t be requested by perpetuity

- The question is how to move the drugs on the Program to the Canadian market?

- Who we work with? Who are our stakeholders?
  - Physicians
  - Manufacturers
  - Pharmacists
  - Associations
  - Patient Stakeholder Groups