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Special Access Programme
Issue Identification Paper

November 2007

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Bureau of Policy, Science and International Programs (BPSIP) and the Special Access Programme Working Group

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Special Access Programme:  
Identifying the Issues

This paper is intended to document key issues and concerns related to the regulatory frameworks and defining authorities of Health Canada’s Special Access Programmes (SAP) for Drugs and Medical Devices. The paper reflects the issues noted through consultations within Health Canada and through external consultations with stakeholders, provincial and territorial regulatory partners. All interested Canadians are invited to provide feedback on this document. To do so, please refer to the instructions on how to participate, presented in Appendix B.

BACKGROUND

Health Canada is authorized under the *Food and Drugs Act* to regulate the safety, efficacy and quality of therapeutic products, including drugs (pharmaceuticals, radiopharmaceuticals, biologics and genetic therapies), natural health products and medical devices. This Issue Identification paper focuses specifically on drugs and medical devices that are regulated respectively under the *Food and Drug Regulations* and the *Medical Devices Regulations*.

Prior to market authorization of a drug, access is usually limited to clinical trials sponsored by a manufacturer or research organization, and authorized by Health Canada through a clinical trial application. On those occasions when a drug is not available through enrolment in a clinical trial, Health Canada may allow an exemption from the *Food and Drugs Act* and its *Regulations* to permit the sale\(^1\) of an unauthorized\(^2\) drug for a medical emergency.

This regulatory exemption was initially administered by the Emergency Drug Release Programme (EDRP) within Health Canada’s former Health Protection Branch. The original purpose of the EDRP was to provide access to unauthorized drugs for medical emergencies on a case-by-case basis. In the 1990's, an internal evaluation of the EDRP found that the program was increasingly being used as a means to obtain broad access to drugs that were in the later phases of clinical trials or in the new drug submission review process. Consequently, the Programme's interpretation of the term “medical emergency” was expanded to include serious or life-threatening conditions and the EDRP was renamed as the Special Access Programme (SAP).

It should be noted that the same exemption in the *Food and Drug Regulations* may allow for the sale in Canada of unauthorized drug products to veterinary practitioners. This release would also be administered through a Programme for the treatment of diseases in companion animals, food-

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1. According to the *Food and Drugs Act*, “sell” includes offer for sale, expose for sale, have in possession for sale and distribute, whether or not the distribution is made for consideration.

2. The term “unauthorized” used throughout the document implies that sale of the drug has not commenced, pursuant to C.01.014 or that the product has been discontinued or removed from the market pursuant to C.01.014.6 and C.08.006 of the *Food and Drug Regulations*.
producing animals, and in wildlife species. This Programme continues to use the name Emergency Drug Release Programme (EDRP) and will be referred to throughout this paper as the “EDRP for veterinary drugs”.

Health Canada also administers a Special Access Programme for Medical Devices. Unlike the drug SAP, which administers an exemption from the Food and Drug Regulations, the medical devices SAP administers an authorization set out in the Medical Devices Regulations. These Regulations expressly define special access as “access to a medical device for emergency use or if conventional therapies have failed, are unavailable or are unsuitable.”

In recent years, there has been increasing focus on the timely access to new therapeutic products by Canadians. While Health Canada has been addressing “timely access” through changes to the regulatory review process, it is recognized that the Special Access Programme plays a role by providing access to products that have not yet obtained market authorization.

In March 2006, Health Canada sought advice from the Science Advisory Board on issues associated with the SAP. The Board’s recommendations included:

i. the development of a comprehensive policy framework that encompasses both the ethical and scientific dimensions of regulatory functions;

ii. introduction of measures to prevent abuse of the SAP;

iii. expansion of data provision requirement for manufacturers for products made available by the SAP; and

iv. expansion of the existing information management systems to provide greater capacity for data extraction.

In response to the Science Advisory Board’s recommendations as well as comments from the SAP stakeholders and users, Health Canada has undertaken a comprehensive review of the SAP in order to modernize the regulatory and policy frameworks supporting the Programme.

**Health Canada’s Comprehensive Review of the Special Access Programme**

The comprehensive review consists of three separate sub-projects which include:

i. an operational review to evaluate how the Programme is functioning within its existing framework;

ii. an ethics review to study the ethical context of the mandate and activities of the SAP; and

iii. a broad policy/regulatory review including, the development of a guidance document. Issues identified in the operational and ethics review will feed into this broad policy/regulatory review. This review provides for public involvement activities at key steps in the review process to ensure that all interested and affected Canadians have the opportunity to contribute:

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3. The Science Advisory Board (SAB) was established in 1997 to provide independent advice to the Minister of Health on how to foster the best science in health protection, and to secure and maintain public confidence in our work. It is comprised of individuals from outside the federal government who have scientific knowledge, experience and expertise relevant to the mandate of Health Canada. [http://hc-sc.gc.ca/sr-sr/advice-avis/sab-ecss/index_e.html](http://hc-sc.gc.ca/sr-sr/advice-avis/sab-ecss/index_e.html)
a. Phase 1 (Spring 2007): External consultations to identify the major issues and concerns related to the SAP;
b. Phase 2 (Autumn 2007): Web-posting of the Issue Identification paper to ensure a common understanding of the issues surrounding the SAP;
c. Phase 3 (Spring 2008): Public Forum to obtain feedback from all interested Canadians on the proposed options for the modernization of the SAP.

This issue identification paper reflects the issues brought forward both internally to Health Canada, and externally through the Phase 1 consultations with stakeholders and provincial and territorial regulatory partners. The list of stakeholders met in Phase 1 is provided in Appendix A. The instructions on how to participate in this second phase of consultation are presented in Appendix B.
ISSUE IDENTIFICATION

1.0  Regulatory Authorities for the Special Access Programmes

Established through a regulatory amendment in 1966, sections C.08.010 and C.08.011 of the Food and Drug Regulations grant an exemption from the Food and Drugs Act and its Regulations to allow for the sale of a drug that has not received market authorization for a medical emergency. The SAP administers this exemption, providing practitioners with limited access to new drugs that cannot otherwise be sold or distributed in Canada. These provisions also govern Health Canada’s Emergency Drug Release Programme (EDRP) for veterinary drugs.

The SAP for medical devices administers the regulations for Custom-Made Devices and Medical Devices to be Imported or Sold for Special Access. These provisions, established in 1998, are contained in Part 2 of the Medical Devices Regulations and specifically allow for the sale of Class III or Class IV medical devices through “special access”, which is a defined term in these Regulations.

These emergency provisions are not intended as mechanisms to circumvent drug or medical device access through approved clinical trials or through market authorization pursuant to the review process. Furthermore, the provisions are not intended to allow for the promotion or commercialization of early use of drugs and medical devices before their safety and efficacy are clearly established.

1.1  Definition of a Medical Emergency

The Food and Drug Regulations governing the SAP state that a drug may be sold “for use in the emergency treatment of a patient” and require a practitioner to identify the “medical emergency for which the drug is required.” The Regulations do not expressly define the term “medical emergency.” Consequently, the SAP for drugs has interpreted this term to include “serious or life-threatening conditions when conventional therapies have failed, are unsuitable, unavailable or offer limited options.” Even with this interpretation there has been a broadening of the SAP’s scope to include, for example, drugs used in the treatment of chronic disease or to address drug shortages in Canada. Without a stated definition of “medical emergency”, there is a lack of clarity on the scope of conditions and circumstances for which a drug may be requested under the SAP.

There is a similar lack of a legal definition for “medical emergency” under the EDRP for veterinary drugs, which currently interprets a medical emergency as: a) a life-threatening medical condition/disease where the availability of an early diagnostic or treatment could save the affected patient(s) and, b) a situation when there is no other approved drug available in Canada, or that other drugs available did not produce satisfactory clinical results.
The Medical Devices Regulations also do not define a “medical emergency”. However, the Regulations address the circumstance under which a device could be requested through the definition of “special access” which is “access to a medical device for emergency use or if conventional therapies have failed, are unavailable or are unsuitable.” The Medical Devices Regulations do not address the conditions for which a device may be requested.

1.2 Practitioner or Healthcare Professional

Separate regulatory provisions for drugs and devices have created inconsistencies between two programmes even though they have the same overarching intention, namely to provide emergency use access to products unavailable on the Canadian market. An example of this inconsistency concerns who specifically may request a product for special access.

Section C.08.010 of the Food and Drug Regulations allows a practitioner to request a drug through the SAP whereas under Part 2, section 71 of the Medical Devices Regulations, a device is requested through the SAP for medical devices by a healthcare professional.

Under Part C, Division 1, section C.01.001 of the Food and Drug Regulations, a “practitioner” is defined as “a person authorized by the law of a province of Canada to treat patients with any drug listed or described in Schedule F to the Regulations.” This definition of a practitioner has led to criticism by nurses, naturopaths, midwives and pharmacists who feel they should be able to request a drug through the SAP for drugs, thus raising the question about the appropriate scope of practitioners.

Some provinces have given pharmacists limited prescribing rights. For example, the province of Alberta has recently implemented new authorities allowing pharmacists to prescribe drugs under Schedule 1 of their Pharmaceutical Profession Act, a list that references Schedule F to the Food and Drug Regulations. This raises the operational challenges of verifying whether or not a particular pharmacist has prescribing rights and hence access to the SAP.

The Medical Devices Regulations refer to a “healthcare professional” which is defined as “a person who is entitled under the laws of a province to provide health services in the province” thus allowing for a wider scope of professionals who may request a medical device for special access.

1.3 “Future Use” Requests

The Food and Drug Regulations set out certain requirements to be met before an SAP sale can be authorized. The first of these is that the sale be one “for use in the emergency treatment of a patient” under the requesting practitioner’s care.
There are circumstances where Canadian institutions may require certain unauthorized drugs to be available on-site in anticipation of a life-threatening emergency. Such institutions include emergency rooms in hospital settings, zoos, the Department of National Defence, the Centre for Emergency Preparedness, first responder institutions, such as fire and police departments, and workplaces where hazardous materials are handled. The SAP considers “future use” requests from practitioners who wish to access a supply of a drug in anticipation of an urgent future use need. In such cases, the practitioner must provide justification as to why such products cannot be requested on a patient-by-patient basis. While the Food and Drug Regulations are expressed in terms that permit the sale of a specific quantity of the drug to a specific practitioner for a specific patient, the SAP has accommodated future use requests through a broad interpretation of the Regulations.

In the case of veterinary medicine, drugs are often used to treat a large group of animals at the same time, such as a herd of cattle or swine at a production site. It is impractical to issue, for example, 200 authorizations for a herd of 200 cows to be treated with the same EDR drug. Therefore, the EDRP considers one herd to be one patient under the care of a veterinarian, and the total amount of drug for the herd is released through a single letter of authorization. Again, the Regulations are insufficiently worded to address the specific needs of veterinary medicine without resorting to a broad interpretation.

The SAP for medical devices also considers requests for future use through what the Programme calls a “batch release” of devices. The wording in the Medical Devices Regulations is comparatively more flexible and avoids the suggestion that medical device special access authorizations must only be issued for an individually identified patient. Nevertheless, the Regulations lack explicit language authorizing a “batch release” or future use.

Stakeholders commented that the current “future use” process is unclear. The SAP for drugs and the SAP for medical devices manages this issue differently, and there is no clear guidance on the subject. Stakeholders have requested greater flexibility with regards to stockpiling drugs through future use.

1.4 Informed Consent

Sections C.08.010 and C.08.011 of the Food and Drug Regulations are silent on informed consent by a patient taking a drug released under the SAP.

Since the drugs released through the SAP are exempt from the Food and Drug Regulations, there may be uncertainty associated with the safety of individual products. Some stakeholders have suggested that if safety issues are known, or if there is evidence of an unfavourable risk-benefit profile, it is incumbent on Health Canada to transmit this information to the practitioner and the patient. For its part, the SAP may decline a special access request if there is a known safety issue about a drug and if the practitioner is unable or unwilling to inform the patient of these safety risks.
Some stakeholders believe that written informed consent should be a requirement due to the inherent risk of drugs authorized by the SAP. This measure was deemed important because patients, particularly those for whom a drug accessed through the SAP represents the final option, could de-emphasize risk in favour of benefits and, in the event of subsequent adverse events, could claim a lack of prior knowledge.

In contrast, section 71.2(i) of the Medical Devices Regulations explicitly states the health care professional is to provide, as part of the application, a written undertaking to inform the patient of the risks and benefits associated with the use of the unapproved medical device.

1.5 The Need for a Modern Regulatory Framework

Established in 1996, the current regulatory framework for the SAP has a single patient focus and is silent on potential public health impacts. Furthermore, the framework is challenged in the face of advances in health science and technology, as well as innovations for treatments and disease management. The range of international sources of drugs released under the SAP has also created challenges that did not exist when the framework was created.

For example, some blood products obtained through the SAP are manufactured with plasma obtained from countries that are not Bovine Spongiform Encephalopathy (BSE) free or where blood screening and collection may not be comparable with North American standards. Such requirements are absent from the current regulations. This could present risks not only for the patient but also for public health. It is important in such cases for all known and theoretical risks to be managed appropriately, which would include full disclosure of the known facts to practitioners and their patients.

In cases where the patient is a food-producing animal, the regulatory exemptions under the Food and Drug Regulations that allows for the release of EDR drugs also exempt the legal requirements concerning minimum withdrawal periods or maximum allowable drug residues. With no explicit legal authority over drug residues or blood products, these issues are solely addressed in policy. Although Health Canada has a discretionary authority to authorize or deny a request, a denial could be subsequently challenged due to the lack of legal authority.

1.6 Compliance and Enforcement

Section C.08.010(1)(b) of the Food and Drug Regulations states that a practitioner has agreed “to report to the manufacturer and Health Canada on the results of the use of a drug, as well as information respecting any adverse reactions encountered” with the use of the drug. However, practitioners generally consider this obligation to be voluntary and compliance is poor. In addition, there have been few resources available to the SAP to enforce such reporting.

Lack of compliance and enforcement has also led to concerns in the area of drugs obtained through the EDRP for food-producing animals. There is a duty to protect the safety of the
Canadian food supply, and a lack of enforcement on reporting may impact on both humans and animals when there is the potential for an unanticipated, serious adverse reaction with a drug.

2.0 Expanded Scope of the SAP

The scope of the SAP has gradually expanded to address requests for a broader range of unauthorized drugs. The nature of some of these requests and the intended uses of some of these drugs have led to questions on the appropriateness of such requests under the existing special access framework. The SAP was originally intended to consider requests for drugs that are not yet approved for sale by Health Canada. However the scope has increased to consider the following additional categories of drugs:

- **Drugs in shortage/backorder:** These are drugs authorized by Health Canada and have been notified for sale by the manufacturer (i.e., sold in Canada), but are temporarily unavailable for release in Canada. The SAP will consider authorizing access to an alternative source of an otherwise marketed drug for the duration of the shortage/backorder.

- **Drugs that have received a negative regulatory decision:** During the regulatory review of a drug submission, Health Canada may decide there is insufficient evidence to support the safety, efficacy and/or quality of a drug and, may therefore, deny the drug from the market through the issuance of a negative regulatory decision. The SAP will consider requests for drugs that have received a negative regulatory decision provided that the manufacturer is willing to disclose the reasons for denial of marketing authorization.

- **Discontinued and withdrawn drugs:** Discontinued drugs are those that have been approved for marketing and have been available for sale but are currently not offered for sale on the Canadian market, typically for economic reasons. Withdrawn drugs are similar but were withdrawn from the market either voluntarily by the manufacturer or on order from Health Canada, typically due to safety concerns.

- **Drugs never marketed in Canada:** These drugs were approved for marketing in Canada but for a variety of reasons they have never been notified for sale on the Canadian market.

- **Drugs used by small populations:** These are drugs that are not approved for marketing in Canada and regulatory approval is unlikely to be pursued. They are indicated for rare conditions or for a small population and are, therefore, not commercially viable. These

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4 As per section C.01.014.3 of the *Food and Drug Regulations*, “the manufacturer or importer or person authorized by the manufacturer or importer shall, within 30 days after commencing sale of a drug, date and sign the document issued pursuant to subsection C.01.014.2(1) in respect of the drug and return the document (a) with a confirmation that the information recorded therein is correct; (b) indicating the date on which the drug was first sold in Canada; and (c) accompanied by samples or facsimiles of all labels and package inserts and any further prescribing information stated to be available on request.”
drugs may or may not have been approved in other countries. An example in this category includes drugs used to treat tropical diseases. Overall, these drugs make up a small component of the drugs requested through the SAP.

In some instances, individual drugs have been made available through the Programme for several years, with thousands of requests authorized per year. These examples are considered outside the regulatory intent of the program and their review has created policy, legal and operational challenges. The SAP has no authority to give consideration to other factors that are relevant to a decision on whether to provide access, such as the overall volume of requests received or a manufacturer's unwillingness to set up clinical trials in Canada, during the review of an individual request.

In the case of the EDRP for veterinary drugs, there are no approved drugs available on the market for some “minor” or less common species of animals, such as goats, ostriches, llamas, rabbits, and ferrets. Drugs for these species, for use in both emergency and non-emergency situations, are authorized for release by the EDRP, but have very limited safety and efficacy information and the risks (including both legal and health) are often difficult to assess.

Since Health Canada is responsible for ensuring the safety of foods such as milk, meat, eggs, fish, and honey from animals treated with veterinary drugs, the EDRP will not issue an authorization if a drug has received a negative regulatory decision following a submission review. In contrast the SAP for (human) drugs will consider authorization for those drugs that have received a negative regulatory decision.

### 3.0 SAP as a disincentive for manufacturers to seek market authorization

Health Canada performs a thorough review of a clinical trial application and a new drug submission before a clinical trial may proceed or a new drug is put onto the Canadian market. In contrast, the SAP does not conduct a comprehensive regulatory review. A SAP authorization therefore does not constitute an opinion or statement that a drug is safe, efficacious or of high quality.

As an exemption from the Regulations, the SAP has, upon occasion, been used to circumvent the regulatory review process, including the evaluation of both clinical trial applications and new drug submissions and, at times, subtly promote unauthorized drug products. Health Canada has no authority to compel a manufacturer to conduct a clinical trial or submit a new drug submission.

There is also a similar lack of incentive for those veterinary drugs that are prescribed for minor species. These types of drugs are used infrequently and in low quantities, and manufacturers would not necessarily obtain significant economic benefit from funding studies for drugs that would ultimately have a very limited market.
4.0 **Operational Considerations - SAP for Drugs**

An in-depth operational review of the SAP for drugs has been performed to assess how well the Programme is functioning. During the Phase 1 consultations for the broad policy and regulatory review, operational issues were raised by the stakeholders. The operational issues identified throughout the consultation will be considered by the SAP. Some of the issues identified through both the operational review and the consultations are summarized below.

*Electronic support system:* One major finding of the operational review is the lack of an adequate electronic support system for the SAP, which has contributed to several problems. Approximately 30,000 requests per year are processed by the SAP. The Programme is supported by three separate database systems: one for data entry; a second for tracking incomplete requests; and a third to support drug information needs. There is no link between the separate systems, which impedes the Programme’s ability to obtain the full history of a drug file.

*Centralized information management:* There is a need for a centralized information management system. The SAP currently has paper and electronic files of all drugs released through the Programme. In the absence of a fully electronic environment, the SAP has had to maintain and enhance its capacity to file, store, and archive paper records of all SAP transactions. While the SAP staff has managed the high volume of requests, it is generally agreed that this represents an inefficient process for consideration of requests.

*Lack of an online database:* During consultations, stakeholders repeatedly called for an online database to manage the demand for information and the large volume of requests. Stakeholders suggested that a pre-authorization list of products would expedite the authorization process.

*Review process for the consideration of a request for a new drug is not clear:* Stakeholders expressed concerns that the consideration process for a new drug request was unclear.

*Lack of timely follow-ups on denials of SAP requests:* The lack of timely follow-ups on denials of SAP requests was identified as a significant concern for practitioners awaiting an authorization for a potentially lifesaving drug.

*Requirement to file a request every six months if the patient requires chronic treatment:* The SAP application process is the same regardless of the circumstances.

*Difference in service hours between two programmes:* Stakeholders have questioned why the availability of the SAP for medical devices is only during business hours while the SAP for drugs is available 24 hours, 7 days a week. There were claims that it has taken up to three days to receive a medical devices SAP authorization from Health Canada. It was also suggested that the SAP for medical devices should reduce their targeted processing time to 24 hours, consistent with the SAP for drugs.

*Importation delays for medical devices:* There is also the long-standing concern with importation delays for medical devices. Without an authorization, medical devices from other
countries cannot be imported. The process is impeded when the industry must await as much as three days for an authorization before commencing the importation process. Stakeholders expressed frustration that the SAP for medical devices authorizes a batch release of a device which, if manufactured in Canada, cannot be distributed unless a medical device licence is issued. Medical device stakeholders have expressed the views that both programmes, having the same intent, should operate consistently and in the same manner.

5.0 Economic Considerations

Accessibility to drug products was identified as the primary concern for stakeholders. Prices for drugs accessed through the SAP can vary from being free of charge to prohibitively expensive. The decision to charge for products released through the SAP remains solely with the manufacturer. As a result, a manufacturer’s decision to charge for a product authorized for release through the SAP could limit a patient’s ability to access that product.

Drugs available through special access may or may not be funded by other parties. Provincial formularies include drugs based on evidence of efficacy and safety, as well as cost-effectiveness. The safety and efficacy is demonstrated through a market authorization from Health Canada. Drugs accessed through the SAP are exempt from the Food and Drug Regulations and are not subjected to a regulatory review. Therefore, funding through provincial drug formularies is usually not available. Private insurance plans also generally do not fund drugs accessed through the SAP. Access through a physician or hospital setting could include financial coverage for special access products, although not necessarily.

At present, the SAP does not take the cost of a product into consideration when making an authorization decision. For example, requests for drugs through the SAP as alternatives for expensive marketed products are based solely on the available clinical evidence. There are also situations when a drug has received its Notice of Compliance and is marketed, but its addition to a provincial formulary has been delayed. Even if the manufacturer would make the same drug available through the SAP at a reduced cost, a request would not be authorized at this stage, as the drug is marketed.

Stakeholders have also been critical of those occasions where a drug was initially obtained through the Programme without charge, and a substantial price increase resulted when the drug became available on the Canadian market.
6.0 Ethical Considerations

The mandate of the SAP is often referred to as ‘compassionate access’ although there is debate over what that word means and how it should be explicitly incorporated in the national regulatory framework. SAP activities are often of high profile and there are inherent tensions between stakeholders (e.g. patients, health care professionals, drug companies, institutions) and the regulator, particularly in the context of a patient who is suffering from a serious or life-threatening disease with few if any option for treatment. In these situations, Regulations are rarely precise or broad enough to account for the range of emotions and interests that lay at the centre of these conflicts. Many of these tensions can and have been addressed though open communication, transparent processes, and by applying progressive risk management philosophy but the Programme is often drawn into larger debates over the timing, equity, and extent of access to a particular drug. These issues are less often about strictly scientific and regulatory matters as they are about the ethical dimensions of the work of the SAP.

The ongoing review of the SAP aims to ensure that the regulatory scheme for early access to new therapies is progressive, ethical and in line with basic principles of good care and health protection. Part of that review includes an analysis of the ethical dimensions of the Programme and how to translate good ethics practices into good public policy. Preliminary work has revealed that despite the fact that most western countries have comparable authorities and that much attention is paid to ethical issues related to human research, relatively little attention has been given to the ethical dimensions of early access to new therapies outside of clinical trials. Nevertheless, everyone agrees that the interests of patients are paramount and comprehensive systems must be in place to ensure that patients receive the best possible care within the safest possible environment. The challenge then is in finding consensus on the extent to which the regulator should provide or restrict access to these therapies and the basis upon which these decisions should be made.

Stakeholders hold wide ranging viewpoints on the extent to which the regulator should scrutinize requests for access to unapproved drugs. On the one hand, there are those that support a patient’s right to choose and gain access to any therapy from which they and/or their physician feel they might benefit. Those who hold this viewpoint also say that a decision to deny access represents a violation to Section 7 of the Charter of Rights, which guarantees the right not to be deprived of life, liberty and security of the person except in accordance with the principles of fundamental justice. On the other hand, there are those who feel that such access should be supported by scientific evidence and clinical experience and that access through SAP should not be used as a way to circumvent regulatory review and oversight. Still others argue that greater emphasis should be placed on the potential benefits as opposed to the possible risks associated with a drug.

During consultations, stakeholders felt that safety and efficacy should not be a reason for denial of a SAP request for a drug as long as a qualified practitioner can attest that he/she believes it is the only course of action. Additionally, the lack of clinical expertise within the SAP has led to questions regarding the authority of the SAP to question a practitioner’s medical expertise or to deny access to drugs.
7.0 New Science Considerations

Many new scientific discoveries have been translated into the development of new therapies and innovations for treatments and disease management. In some cases the basis for these new therapies has not been fully validated before they become available to patients either through clinical trials or compassionate access schemes. This is the result of the drive to balance the orderly development of therapies with the need to address unmet or poorly met needs of patients. For instance the use of surrogate markers as endpoints for clinical development of therapies has advanced significantly over the last 10 years, but there is still a need to ensure that these markers predict significant benefit for patients. Surrogate endpoints are measurements that substitute for real measures of health. It is a measure of effect of a certain treatment that may correlate with a real endpoint but doesn't necessarily have a guaranteed relationship. Similarly, receptor based technology is a driving force now in pharmaceutical development. Data on drugs supported by receptor based technology are those drugs which target certain receptors, for example, in treating tumours. However, experience has shown that drugs which target certain receptors may not be effective in treating all tumours which express those receptors.

The SAP has received requests for access to drugs supported only by a receptor based, or surrogate endpoint argument. Theoretical arguments about the possible effectiveness of such drugs are challenging for the SAP which requires, as per subsection C.08.010.1 (a)(ii) of the current regulatory framework, that a request be supported by data respecting the use, safety and efficacy of the drug. This implies that there must be direct human experience with the drug and that SAP should not be the context within which a drug is tested in humans for the first time.

There is need to ensure that any proposed modernization of the SAP regulatory framework keeps pace with significant developments and innovations in the world of drug development.

NEXT STEPS

This paper reflects the issues identified through internal and external consultations in early 2007. It also reflects issues identified during the operational review of the SAP and the ethics review. The paper will be posted on the Health Canada website for approximately 30 days in order for stakeholders to confirm that the issues have been accurately understood and fairly presented. The paper will then serve as the basis for a subsequent analysis of the issues and the development of options that will lead to the modernization of the policy and regulatory framework for the special access programmes for drugs and medical devices, and the EDRP for veterinary drugs.
Appendix A – Stakeholders met in Phase 1 Consultations

- Best Medicines Coalition
- British Columbia Cancer Agency
- British Columbia Centre for Disease Control
- British Columbia Hospital and Health Centre
- British Columbia Persons with AIDS Society
- British Columbia Veterinary Medical Association (BCVMA)
- Calgary Health Region
- Canada’s Research-Based Pharmaceutical Companies (Rx&D)
- Canadian Association for AIDS Research, British Columbia Centre for Health and Excellence in HIV/AIDS
- Canadian Blood Services (CBS)
- Canadian Fabry Association
- Canadian Forces Health Services
- Canadian Generic Pharmaceutical Association (CGPA)
- Canadian Medical Association (CMA)
- Canadian Organization for Rare Disorders
- CancerCare Manitoba
- Groupe de recherche universitaire sur le médicament
- Groupement provincial de l’industrie du medicament (GPIM)
- Hamilton Health Sciences Centre – Henderson General Hospital
- Health Canada’s Provincial and Territorial partners in Health
- Héma-Québec
- Hôpital Maisonneuve-Rosemont, Montréal
- Hôpital Saint-Luc du Centre hospitalier de l’Université de Montréal (CHUM)
- Hospital for Sick Children, Toronto
- MEDEC
- Montreal Children’s Hospital
- PharmaWatch
- Provincial Blood Coordinating Office (PBCO)
- St. Paul’s Hospital, Vancouver
- University of British Columbia – Faculty of Medicine Paediatrics
Appendix B – How to Participate

Health Canada Wants to Hear From You!

You’ve read the Issue-Identification paper on the Special Access Programme, now Health Canada wants to hear you on four key questions:

- Do the issues identified in this document seem accurate to you?
- Have we framed the nature of stakeholder concerns correctly?
- According to you, did we forget any important issue(s)?
- Can you suggest potential solutions or means to solve a specific problem inherent to one or some issue(s)?

Submitting Your Comments

You can send your comments, in English or French, either by e-mail, fax or regular mail by December 17, 2007.

- **Affiliation:** Please identify clearly your stakeholder affiliation (e.g. patient organization, industry, health professional, etc.) when responding.
- **Format:** Acceptable files include: Word, WordPerfect, PDF, Txt or RTF.
- **Comments:** We recommend that you clearly identify the issue on which you have a comment or a suggestion and group all your comments under a question block. General comments can be addressed as a group, separately.

**Example:**

*Issue: Economic Considerations*

1. Accuracy of the issue
2. Issue omitted
3. Solutions

Contact Us: To send us your comments or obtain information, you can do so by:

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