Canadian Blood Services' Plasma Derivative Formulary: A Model to Inform Aspects of National Pharmacare

Report to the Advisory Council on the Implementation of National Pharmacare

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Canadian Blood Services
BLOOD
PLASMA
STEM CELLS
ORGANS
& TISSUES

Canadian Blood Services

Canadian Blood Services is an independent, not-for-profit charitable organization created through a memorandum of understanding between the federal, provincial and territorial governments. We opened our doors in September 1998. Our operational funding comes primarily from provincial and territorial governments.

This paper updates and builds upon our submission to the federal Standing Committee on Health of May 2, 2016, made during the Committee's study of national pharmacare.

What we do

Canadian Blood Services is Canada's biological lifeline. We connect the sincere generosity of donors with the heartfelt appreciation of patients. We undertake a broad range of activities across the country in four main areas:

- **Blood:** We are the blood authority and blood operator in Canada in all provinces and territories except Quebec. We collect, test and manufacture blood and blood products, including red blood cells, platelets and plasma. We distribute these products to hospitals and work with provincial and territorial governments to meet the needs of patients across the country. We also provide diagnostic laboratory testing services in some provinces.

- **Plasma:** We collect plasma from volunteer, unpaid donors in Canada. We retain some of this collected plasma to meet the transfusion needs of Canadian patients but most is shipped to contract manufacturers where it undergoes a process called fractionation to create plasma-derived drugs. We also bulk purchase and distribute Health Canada-approved biological therapies. These drugs, which we refer to as plasma protein products, are either manufactured from human plasma through fractionation or synthetically engineered using recombinant DNA technology.

- **Stem cells:** We operate a stem cell program that supports better outcomes for patients living with any of the more than 80 diseases and disorders that can be treated with stem cell transplants. We collect cord blood and manufacture stem cells through our cord blood bank. We operate a robust national registry of potential adult stem cell donors and participate in an international network of donor registries. We provide human leukocyte antigen typing services to ensure the best possible matches between those willing to donate stem cells and those who need them.

- **Organs and tissues:** We manage a national transplant registry for interprovincial organ sharing and related programs for organ donation and transplantation. Working with partners across the organ and tissue donation and transplantation community, we develop and share leading practices, provide education resources and collaborate on new ways to share data on health system performance in these areas in Canada.

To support these activities, as well as the advancement of transfusion and transplantation science and medicine in Canada and around the world, Canadian Blood Services conducts a wide range of research and development activities and participates in research led by others. Through these efforts, we help to translate new knowledge, processes and technologies into the manufacturing environment. Our research and development work also supports problem-solving in the blood supply chain, contributing to

improvements in quality and efficiency. We support professional education and public awareness activities related to transfusion and transplantation and share our knowledge and expertise with our health-care partners, stakeholders and funders.

We have a unique relationship with Héma-Québec, the provincial blood operator that provides blood products in that province and manages its stem cell donor registry. Our two organizations work closely to share blood products in times of need, and we collaborate regularly to communicate information, insights and data.

National formulary of plasma protein products

Canadian Blood Services is the only national manufacturer of biological products funded by Canada's provincial and territorial governments. We manage a pan-Canadian formulary of about 45 brands of plasma protein products and synthetic alternatives. We also store, ship and deliver these drugs to hospitals and clinics across the country using a distribution network already approved and funded as part of our national blood supply responsibilities.

Plasma protein products are used to treat people with a variety of inherited and acquired disorders, including some who require life-long therapy. They are prescribed for: hemophilia and other bleeding disorders; primary and secondary immune disorders, including some neurological conditions; burns and traumas; disorders related to inherited protein deficiencies and many other conditions. In some cases, they are lifesaving treatments for which there are no alternative therapies. While many of these drugs are administered in hospital, increasingly they are being manufactured in formulations that permit in-home administration through subcutaneous injection.

Our plasma protein products program has an approximate worth of $750 million a year. It leverages the combined buying power of provincial and territorial health budgets to offer publicly funded blood products to those who need them at no direct cost to the patient. While we offer a substantial range of products, ours is a managed formulary, not an open formulary. Selective changes to product listings occur through product selection and public tendering processes.

Blood system principles

A policy framework focused on patient need and sustainability governs how we deliver the blood supply system, including plasma protein products. It is informed by blood system principles outlined in our founding memorandum of understanding, which dates from 1997 and remains in force today. The principles and recommendations in the Krever report, published by the Commission of Inquiry on the Blood System in Canada that same year, also provide Canadian Blood Services with essential guidance.

This framework requires we balance cost, benefit and risk in our decision-making processes. It names Canadian Blood Services the country's national blood authority in all jurisdictions but Quebec and calls for "the gratuity of all blood, components and plasma fractions to recipients within the insured health services of Canada." Similarly, the Krever commission recommended Canadians should have "free and universal access" to blood components and blood products, whether used inside or outside of hospitals, in a publicly administered, national system. Informed by these and other blood system principles, Canadian Blood Services strives to balance equity of access, product choice and innovation, and highly competitive pricing in the management of the formulary.

Product selection

Paths to inclusion in the formulary involve product selection and competitive bid processes. A product under review for potential inclusion as a new category undergoes a thorough medical and scientific assessment by blood system experts as part of the product selection process, as well as a pharmaco-economic analysis by the Canadian Agency for Drugs and Technologies in Health (CADTH). Once this work is complete, Canadian Blood Services provides a recommendation to provincial and territorial ministries of health on whether a new category should be added to the formulary or not. Final approval for addition of a new category must be obtained from provincial and territorial ministries of health before a new category product can be distributed by Canadian Blood Services.

The selection of a new brand of product in an approved category is within the discretion of Canadian Blood Services and is subject to regular public tendering processes. On occasion, the tendering process can result in products being removed from the formulary. However, even when products are discontinued, Canadian Blood Services may procure and distribute them on a case-by-case basis for those patients who have clinical difficulties in tolerating other new or existing products. This mechanism ensures patient access to brand-specific products, based on clearly defined medical need, is not inhibited by choices made during procurement cycles.

As part of our practice, we maintain relationships with key stakeholder groups (patients, physicians, suppliers) to better understand the product pipeline, physician and patient preferences, and to help optimize access, effectiveness and choice. Our procurement processes give patient groups and the prescribing medical community a voice in decision-making, although Canadian Blood Services maintains the final say in public tendering decisions. We strive to offer a reasonable degree of product choice within the formulary. When decisions result in delisting of a product, and therefore the need for patients to switch to a new brand of product, we work with stakeholders to facilitate safe and effective product transition processes.

Ultimately, our goal is to meet the needs of patients and practitioners while working with provincial and territorial funders to maintain the financial sustainability of the formulary.

How we bring value

Because we offer a principles-based, publicly funded, “made-in-Canada model,” we believe aspects of our experience with plasma protein products can contribute to national pharmacare discussions. At the same time, we understand our model isn’t a solution for all of pharmacare’s challenges. In particular, our approaches to equity, access, product choice and cost-effectiveness are worth consideration, as is our ongoing focus on improving patient outcomes.

We believe we bring value in the following areas:

- **Equitable access across the country, at no direct cost to patients, while leveraging existing public investment:** Once a plasma protein product is accepted into our portfolio, it is available to patients and practitioners in all member jurisdictions pending individual provincial and territorial access guidelines. This is a significant outcome of blood system principles and is in line with those informing the Canada Health Act (i.e. universal access.)

Canadian Blood Services distributes plasma products to hospitals in urban, rural and remote centres across the country via the logistics and infrastructure built for the blood system. This includes not just the physical infrastructure but also the process and validation expertise required of a biologics manufacturer regulated by Health Canada. We have robust processes for procurement, quality assurance, warehousing, distribution and monitoring of products.

- **A secure, diverse supply:** In many cases, we carry multiple brands of a product class to encourage security of supply and more competitive pricing. We also buy product in smaller, diverse lots, and we negotiate a guaranteed "safety stock" to mitigate risk of product shortfalls. With few exceptions, our approach has resulted in a competitive market and a secure, diverse supply of plasma protein products and their synthetic alternatives. Canadian Blood Services also independently qualifies new suppliers and audits them periodically, adding an additional layer of vigilance and product safety for patients. Our contractual relationships with suppliers require early and regular reporting of any supply or quality disruptions. This helps with early recognition of supplier issues in bringing products to market or maintaining adequate Canadian supplies, which, in turn, can reduce the risk of product shortages.

- **A collaborative, evolving, evidence-based approach:** As previously noted, CADTH provides a pharmaco-economic analysis when new drug categories are considered within our product selection process. This collaboration is an important part of the procurement program and ensures the appropriateness of drugs being added to the formulary. To encourage optimum product usage, we also collaborate with physicians, patient groups and provincial and territorial governments across the country to develop clinical practice guidelines and to promote optimal utilization practices.

  At the level of individual products, our model also allows provinces and territories to introduce access guidelines as each deems appropriate, to be managed provincially or at the hospital level. National criteria developed by Canadian Blood Services and national physician groups affiliated with the blood system are also possible.

- **Blood system expertise supporting better patient care:** As part of our approach, our hospital liaison specialists maintain close relationships with hospitals, treatment centres and prescribing physicians to manage and work through any technical issues regarding supply, product choice or adverse event monitoring. Our on-staff medical directors also provide expert advice when a physician encounters an issue with a patient that could benefit from the perspective of an additional specialist.

- **Highly competitive global pricing for expensive biologics:** Through a public tendering approach leveraging the combined buying power of provincial and territorial governments, we issue "requests for proposal" to purchase plasma protein products. From 2013–2014 to 2017–2018, we realized cost savings and avoidance of approximately $600 million out of a total spend of about $3.0 billion over the life of those contracts. Between 2018–2019 and 2020–2021, as a result of a new round of procurement, we anticipate a further $455 million in savings and cost avoidance out of a total spend of estimated at approximately $2.0 billion over the life of the contracts. Cumulatively, this provides more than $1 billion in savings and cost avoidance to health system budgets over an eight-year period. In recent years, we have been able to drive pricing for some of the major classes of these drugs below 2009 pricing levels — a testament to our commitment to delivering value for Canadians.

Managing product use in a pan-Canadian context

Although the use of plasma protein products is the purview of prescribing physicians and outside the direct control of Canadian Blood Services, we collaborate with provincial and territorial governments on practices that support patient access but also consider the long-term sustainability of the formulary. We recognize this is a challenging balance to achieve, but we are committed to ongoing discussions that will benefit both patients and health systems.

Within this context, a substantial challenge has been working across provincial and territorial health systems to engage external programs and evolve internal initiatives aimed at understanding and managing product use, while still meeting the needs of patients and practitioners. There are many aspects to this challenge, such as access to consistent data about how products are used, both in home treatment settings, and in hospital or in other treatment centres. Better data brings many benefits, including a greater understanding of the total health-system cost of a drug versus the cost of acquisition alone. Working in tandem with varied provincial approaches to product management can also bring complexity. For instance, some provinces may have more stringent guidelines for the use of certain products, while others may have more developed relationships with prescribing physicians.

We believe a coordinated, effective approach to managing growth in product use under a single payer system may be more easily achieved if:

- clarity of roles and engagement across federal, provincial and territorial jurisdictions is established and embraced by all parties;
- access to consistent data about how products are used is made an essential aspect of the program; and,
- patient and practitioner engagement is appropriately maintained within formulary decision-making.

Finally, as per our funding model, Canadian Blood Services provides plasma protein products to hospitals at no direct cost, meaning we are the entity that is reimbursed by provincial and territorial ministries of health for hospitals' use of these products. While there are both strengths and weaknesses to this approach, there are other reimbursement models that may also be appropriate in a national pharmacare setting, particularly in relation to managing product use. For example, pan-Canadian utilization programs may benefit if the point of reimbursement for bulk purchased medicines is the treating centre — the entity that uses the drugs — rather than the entity that buys the drugs.

Conclusion

While Canadian Blood Services' formulary is focused on plasma protein products — and therefore just one class of drugs — we believe our experience is worth consideration because it provides tangible Canadian examples of some of the key benefits that a pharmacare program could bring. These include: bulk purchasing; national logistics; clinical and patient group involvement in decision making; a forum for balancing choice, access and cost optimization; and, a high degree of patient equity across the country.

Indeed, through careful stewardship and committed partnerships, our supply chain and procurement practices have facilitated long-term value for both health systems and the Canadians who depend on the medicines we deliver. Our approach also raises some relevant challenges, such as how best to manage product use in a pan-Canadian setting, while still meeting the needs of patients and practitioners.

As our principles-based, publicly administered program evolves, we will continue to build on its successes and find innovative, collaborative ways to reduce its challenges. We would be happy to discuss our experience further should the Council have questions.