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Building a National Strategy for Drugs for Rare Diseases

What We Heard from Canadians

Canada

Health Canada is the federal department responsible for helping the people of Canada maintain and improve their health. Health Canada is committed to improving the lives of all of Canada's people and to making this country's population among the healthiest in the world as measured by longevity, lifestyle and effective use of the public health care system.

Également disponible en français sous le titre :
Élaboration d'une stratégie nationale sur les médicaments pour le traitement des maladies rares: Ce que nous avons entendu des Canadiens

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Publication date: July 2021

Cat.: H22-4/24-1-2021E-PDF
ISBN: 978-0-660-39178-6
Pub.: 210130

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Executive Summary

Canadians with rare diseases want access to drugs that could help manage and treat their conditions. Those drugs, however, can be extremely expensive: as of 2019, there were 93 drugs for rare diseases approved in Canada that cost over $100,000 per patient per year, over half of which cost more than $200,000[1].

The issue of how to ensure equitable access to drugs for rare diseases while supporting the sustainability of the health care system was raised during the 2018 consultations of the Advisory Council on the Implementation of National Pharmacare. In its final report, the Council recommended creating a national strategy for providing fair, consistent and evidence-based access for patients to these specialized drugs. In response, the Government of Canada made a commitment to work with provinces, territories and other partners to create a national strategy for drugs for rare diseases.

In January 2021, Health Canada launched a national online engagement to let Canadians — especially patients with rare diseases, their families and other interested stakeholders — share their views and ideas for a national strategy. This report summarizes responses to the national public and stakeholder engagement, which lasted from January 27 to March 26, 2021.

Participants were invited to respond to *Building a National Strategy for High-Cost Drugs for Rare Diseases: A Discussion Paper for Engaging Canadians*, which posed questions on three key issues:

1. How to improve patient access to high-cost drugs for rare diseases and ensure that access is consistent across the country.
2. How to ensure decisions on funding high-cost drugs for rare diseases are informed by the best available evidence.
3. How to ensure spending on high-cost drugs for rare diseases does not put pressure on the sustainability of the Canadian health care system.

The engagement was conducted through five virtual public town halls, 16 virtual stakeholder meetings, an online questionnaire, and email or mail submissions. The engagement garnered diverse perspectives and participation from over 650 individuals and organizations, including patients and family members with lived experience. A brief summary of what we heard follows.

What we heard . . . about improving access to rare-disease treatments and making it consistent across Canada

Where someone lives should not make a difference in their access to treatment. People told us that "postal code lottery" exists because each jurisdiction (i.e., province and territory) decides which drugs it will reimburse (commonly referred to as "formulary listing"). As well, jurisdictions may have different criteria that must be met before drug coverage will be considered. People noted that similar problems are experienced when an individual's drug coverage is part of a private insurance plan (that is, a drug benefit plan offered to employees by their employer). This leads to gaps in treatment based on where someone lives.

Participants said a single national framework for rare disease treatments would make access fairer and was the most important element for a national strategy. However, people cautioned that a national strategy should not remove access from those who already have access through public programs. They also wanted a transparent coordinating body, better alignment across the system and called for patients and clinicians to have a greater say in all aspects of the strategy.

What we heard . . . about getting the best possible evidence for decisions on drugs for rare diseases

Because of the small numbers of people with rare diseases, meeting the usual standard of evidence for drug approvals is rarely possible. Many participants said rare-disease drugs need to be assessed differently than drugs for common diseases. There were calls for patients and their caregivers to be involved in defining what constitutes a "benefit" or "improvement" when treatment outcomes are assessed.

People overwhelmingly ranked innovative approaches to approval and coverage, such as pay for performance, early access and managed access, as the most important option for building a better evidence base. Some of the alternative options suggested included fast-tracking drug approval of drugs already approved in other countries, having expert panels assess evidence using both international and Canadian data, and joining international evidence-sharing networks. Participants emphasized that patients and clinicians must be part of any expert panel looking at a drug's potential benefits.

What we heard . . . about balancing spending on drugs for rare diseases and keeping Canadian health care sustainable overall

Many people felt the emphasis on the high-cost of drugs overlooked their value for patients, the health system and society as a whole. Generally, participants felt there is a need for more transparency in how drug prices are set and suggested governments should work together to lower prices. In considering options, most people felt better cost-sharing and pooling of risks was the best approach. Some felt the federal government could reinsure drug plans, by paying costs for drugs over a specified dollar threshold.

Other ideas raised included pay for performance (where governments fund drugs based on how well the product works), but others warned that this approach does not lead to lower costs or more effective treatments. Some people thought investing in open science and Canadian research, development and drug manufacturing could produce cost-effective treatments over time. We heard overwhelming support for international collaboration and many people wanted to see more done to bring clinical trials to Canada because they are a way for patients to try new and promising treatments.

What we heard . . . from Indigenous partners

Indigenous Canadians experience a significant burden of illness and poorer health outcomes than non-Indigenous Canadians and face a different reality when they access health services. First Nations, Inuit, and Métis peoples' experiences with the system can differ significantly, making it important for the national strategy to acknowledge and consider a distinctions-based approach in order to improve health outcomes.

Building trust and addressing racism in the health system is key. Indigenous participants and many other stakeholder groups want transparency in decisions on an individual patient's eligibility for rare-disease drug funding, including an appeal process where patients denied funding have an opportunity to challenge the decision.

Indigenous peoples are increasingly leading the delivery of their own health services. What impact the national strategy would have on Indigenous health programs in place now was a concern for participants, as there are agreements between Indigenous and other governments on funding and delivering health services. The strategy needs to respect those existing agreements, have continued dialogue with Indigenous partners at decision-making tables, and ensure care is maintained.

Next steps that were important to participants include:

- Working closely with provinces, territories, Indigenous partners and stakeholders to develop a coordinated strategy that get patients the effective treatments they need.
- Establishing common definitions of "rare diseases" and "high-cost drugs" and better guidelines for what constitute "benefits" and "improvements" for assessing rare-disease treatments.
- Researching best practices in other jurisdictions including France, Germany, the U.K. and Australia.
- Ensuring Canada's regulatory approach, drug pricing landscape, and research and innovation capacity are conducive for rare-disease drugs.
- Considering how to support patients more holistically, including screening, testing and diagnostics.

The Government of Canada will closely review the ideas and suggestions we heard throughout the engagement process. Over the summer, as a follow up to the report, Health Canada will continue discussions with patients and caregivers, health care professionals, researchers, private drug plans, the pharmaceutical industry, and other stakeholders. Health Canada will also continue discussions and collaboration with

provinces and territories, pharmaceuticals management system partners, and Indigenous partners to build a strategy that will work for all Canadians. Further public consultation to inform a comprehensive model for the national strategy will take place in the fall. With the support of all those involved, the Government of Canada will work towards launching the national strategy for drugs for rare diseases in 2022.

Introduction

Rare diseases are often chronic, can be seriously debilitating and even life-threatening, and there are few or no treatments available for most of them. Canadians with rare diseases struggle with the reality that conventional therapies and mainstream health care are not sufficient to meet their needs. But they are determined to find treatment that might help their condition, and the pharmaceutical industry is responding by developing a growing number of drugs. When treatments are available, however, they can be extremely expensive. As of 2019, there were 93 drugs for rare diseases approved in Canada that cost over $100,000 per patient per year, over half of which cost more than $200,000[1].

During its work, the Advisory Council on the Implementation of National Pharmacare heard concerns that the cost of rare-disease drugs can threaten the sustainability of both private and public insurance plans—but at the same time, patients rely on them for life-changing, often life-saving, treatment. In its final report, released in 2019, the council recommended the federal government work with the provincial and territorial governments to develop a distinct national process for providing fair, consistent and evidence-based access for these specialized drugs.

How people with rare diseases currently get the drugs they need

Before a drug can be sold in Canada, Health Canada reviews scientific evidence to ensure the drug is safe, of suitable quality, and works as intended. Canada's health technology assessment agencies (the Canadian Agency for Drugs and Technologies in Health and the Institut national d'excellence en santé et en services sociaux) evaluate the clinical benefits and costs of the drug and issue a recommendation for or against public funding. Once authorized by Health Canada, it can be sold and purchased; at that point both public and private drug plans can decide whether to cover the drug for their members. Each public or private drug plan decides for itself whether it will pay for the drug for their members, and under what conditions.

The drugs Canadians with rare diseases need are paid for in a variety of ways: by insurance coverage from a private or government drug plan, through patient-support programs offered by pharmaceutical companies, through Health Canada's Special Access Program or by patients paying out of pocket or enrolling in a clinical trial. There are various steps in deciding how drugs are covered in Canada (see Figure 1 in the discussion paper).

To help Canadians with rare diseases access the drugs they need, Budget 2019 proposed investing up to $1 billion over two years, starting in 2022–23, with up to $500 million per year ongoing[2]. The Government of Canada is committed to working with provinces, territories and other partners to create a national strategy for drugs for rare diseases. In January 2021, Health Canada launched a national online engagement and invited Canadians — especially patients with rare diseases, their families and other interested stakeholders — to share their views and ideas on next steps under a national strategy.

[1] Patented Medicine Prices Review Board, (2020). "Insight into the spending on expensive drugs for rare diseases" (presentation). Retrieved December 30, 2020 from: https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/2020/Research-Webinar1-EDRD-Market-Size-EN.pdf

[2] Government of Canada, (2019). "Moving Forward on Implementing National Pharmacare" Retrieved April 27, 2021 from: https://www.budget.gc.ca/2019/docs/themes/pharmacare-assurance-medicaments-en.html

The online engagement lasted from January 27 to March 26, 2021. A discussion paper (*Building a National Strategy for High-Cost Drugs for Rare Diseases: A Discussion Paper for Engaging Canadians*) was developed to help frame the engagement. It posed questions (see Appendix A) on three key issues:

1. How to improve patient access to high-cost drugs for rare diseases and ensure that access is consistent across the country.
2. How to ensure decisions on funding high-cost drugs for rare diseases are informed by the best available evidence.
3. How to ensure spending on high-cost drugs for rare diseases does not put pressure on the sustainability of the Canadian health care system.

The engagement was conducted through five virtual public town halls, 16 virtual stakeholder meetings, an online questionnaire, and email or mail submissions. The engagement garnered diverse perspectives and participation from over 650 and individuals and organizations, including patients and family members with lived experience. In a parallel process, Health Canada has met with several Indigenous groups since the discussion paper was released and is committed to ongoing dialogue throughout the strategy's design and implementation.

We would like to thank all those who took the time to participate. Many participants shared details of their personal challenges with rare diseases and accessing treatment. We want to express our sincere gratitude for these insights. The ideas we gathered will help ensure the perspective of patients, their families and other stakeholders will inform a national strategy that prioritizes patient access to effective treatments across the country, works in the context of Canada's health system and respects the role of provinces and territories in health-care delivery.

Online engagement at a glance

- Five virtual public town halls, with more than 300 attendees
- 16 virtual stakeholder meetings, with more than 350 participants
- 136 online questionnaire responses
- 100 written submissions
- Who we heard from: Patients and families, patient groups, pharmaceutical industry, clinicians, researchers, private drug plans, benefit brokers, businesses, labour unions, health organizations and more
- Government policy-makers and pharmaceuticals management system partners also attended

What we heard . . . about improving access to rare-disease treatments and making it consistent across Canada

People spoke of many barriers to accessing treatments. They particularly emphasized that the current approach — where each province or territory makes its own decisions on approval and funding for rare-disease drugs — leads to unequal access depending on where a patient lives.

> **"The funding should come from the federal level . . . I know drugs are funded differently according to each province's decision on what and why they choose to or not to fund specific drugs. I understand the cost is a major factor — but life is life."** — *Patient, family or caregiver*[3]

Patients often referred to this as the "postal code lottery," meaning patients with a specific condition in one province may have the drug for it covered, while patients in another province do not. They also cited varying conditions to be met before a drug is paid for as a key factor driving differences in coverage from one payer to another.

Many people said it was important to define both "rare diseases" and "high-cost drugs" in the strategy, and consider how the definition will be used and what it would mean for stakeholders. Failing to do so, it was felt, might lead to a system that left people out or created gaps between public and private payers. Still, a caution was expressed that defining terms must be done carefully because of the risk of excluding drugs or conditions that don't fit the definitions but are unlikely to be funded through standard pathways. People said that the definition needs to go beyond just a numerical definition (i.e. 1 in 2,000 people), and include elements like disease severity, burden, and lack of available treatments. People also said that the strategy's definition should take into account international definitions so that Canada is in line with other countries.

Others raised the need to ensure all jurisdictions or payers participate in the program. It was felt that allowing payers to opt-out and letting jurisdictions decide which drugs to cover would mean continuing uneven access across the country. People were also concerned that basing the deductibles patients must pay on their household income could be unfair if it doesn't take into account the accumulated cost of multiple medications or high non-drug costs many patients face.

People stressed the need to ensure drug coverage was improved with the adoption of the strategy. Some expressed concerns that private drug plans would delay or roll back coverage as a result of the strategy. Overall, participants were focused on ways to reduce fragmentation in drug coverage. Many participants wanted private payers to be integrated in the solution, by continuing coverage of drugs for rare diseases and sharing costs with governments and industry.

> **"While others with my condition are getting coverage through Trillium or ODSP, I am not able to access coverage for these meds and must pay thousands of dollars a month, or refuse the medication . . . there needs to be a national strategy to pick up all of those patients who do not qualify for existing provincial/private plans. Those who fall through the cracks."** — *Patient*

People expressed concern and frustration that a drug can receive regulatory approval to be sold in Canada (following Health Canada review), but provincial, territorial or private drug plans may still choose to not cover it. We heard part of the problem is conflicting reviews and decisions by regulatory authorities and health-technology assessment bodies. Many people said regulators and assessment bodies, along with payers, and price negotiators should work transparently to align their decisions, tailor them to suit drugs for rare diseases and communicate them clearly to the public. In addition, a few participants raised the challenges with the

[3] To protect confidentiality while also indicating the perspective behind a quote, we have used the general descriptors participants were offered to choose from to identify themselves.

Special Access Program. Some mentioned that patient access gaps are created when drugs remain under the program for multiple years instead of receiving authorization. Some clinicians suggested that the Special Access Program request process could be more streamlined, while other clinicians suggested that the assessment and criteria needs to be more stringent.

When we surveyed people on the four options presented in the discussion paper for ensuring consistent access to rare-disease drugs, regardless of where a patient lives, the majority of participants said a single framework for making decisions would be the most important element of a national strategy. They used different terms for the idea: pan-Canadian, national, centralized, or unified approach, but agreed a single framework would make the system fairer, avoid political influence, reduce the burden on smaller provinces and get more consistent information to the public. There were suggestions that Canada could model the strategy on Canadian Blood Services, which has a pan-Canadian approach to procuring and supplying blood products across the country with provincial support and involvement. While a single framework was favoured, participants made it clear that the strategy's approach should not reduce coverage for drugs already available from the provinces — they did not want existing programs taken away.

> **"A single framework for decision making on high-cost drugs . . . would mean consistency in the decision-making approach across the board so that access could be the same in every province."** — *Patient, family or caregiver*

A number of people thought the other three options — a transparent coordinating body, involving patients and clinicians, and coordinating support for research — should all be parts of a single framework (see Appendix B). As one health professional said, "I selected all options because each will assist in a framework that becomes more equitable, transparent, sharing in perspective from patient and clinician engagement, and reflects a national versus provincial network."

While many participants said a transparent coordinating body could be effective for a single framework, others questioned whether it could be truly transparent. There were also doubts a coordinating body would be free from conflicts of interest and wanted its membership and participation of members to be transparent as well. "Key players must not be limited to private and public drug plans — they must include health-care providers, patients, researchers and drug developers," one person said.

There was an overall consensus that the system needs less bureaucracy. Some participants thought creating another decision-making entity to oversee rare-disease drugs might slow down approvals and suggested using organizations Canada already has in place. Participants overwhelmingly agreed the process must be patient-centred, transparent, timely, equitable, predictable and collaborative, regardless of how it's organized. Patients in particular emphasized the importance of timely access because some patients can "age out" of potentially life-changing therapies.

> **"Transparent communication and more information on the deliberation of advisory bodies and their reasons for recommendations, including their rationale, what evidence is given what weight, and process timelines would be welcome because too much of the current system is performed behind closed doors."** — *Researcher*

We heard that involving patients and clinicians is critical. Most discussions patients, caregivers and clinicians participated in, as well as submissions from them, included a call to be treated as partners, not bystanders. Their message, "Nothing About Us Without Us," was clear and recurrent. People felt the opinions of treating physicians are not given enough weight when decisions are made and wanted both clinicians and patients to sit on panels or advisory boards created as part of a national strategy. We heard that while patients and clinicians are engaged to some extent now, the system could create more meaningful and transparent ways to participate. The European Medicines Agency was consistently brought up as an example of meaningful

patient involvement, where patient representatives receive training to be knowledgeable in regulatory and reimbursement processes, and help to design the framework and its guidelines.

It is important to acknowledge that concerns were expressed that patient advocacy groups and non-profit organizations representing rare diseases may not be neutral: some may have conflicts of interest based on their funders or funding models. While industry support is an important resource for many groups, most participants felt those with a conflict of interest should not play active roles on decision-making bodies involved in the strategy, preferring individual patients, their caregivers and doctors take those places. Patients in particular expressed that at times, they felt their opinions were discounted due to perceived or real conflicts of interest and suggested that the government should aim to empower the patient voice and patient organizations.

We were told approval and coverage of rare-disease drugs should be tailored to the unique circumstances of each individual's specific disease and treatment options, because the nature of rare diseases means a "one size fits all" strategy would inevitably leave some patients without effective treatment. Some argued a clinician's request for access or to continue a treatment for a patient should be all that's needed for a drug to be approved and paid for, especially as options for personalized medicine and gene therapies increase.

Concerns over meeting individual needs prompted calls for the strategy to have an appeals mechanism, where an expert panel could be effectively used. Among other examples, people were worried if the strategy allows coverage and approval models such as pay for performance or managed access[4], patients might have a drug that's working for them taken away or have to pay for it themselves if the government decides the evidence doesn't show enough benefit. As one participant said, "I think any process in place should also have a process for appeals. If an individual feels the drug is effective or valuable despite not meeting a pre-determined goal, what's in place to address this?"

Fewer people talked about whether consistent access would be improved by coordinated support for research on rare diseases, although some said research could help sustain the health care system by improving the evidence base and discovering new treatments.

What we heard . . . about getting the best possible evidence for decisions on drugs for rare diseases

Everyone wants drug treatments that work and are safe. However, the usual standards for evidence may not apply because of the small numbers of people affected by rare diseases. Many people said rare-disease drugs need to be assessed and monitored differently so patients are not kept waiting for desperately needed treatments. Patients spoke about the years and effort that can go into raising money for research and clinical trials, only to be followed by disappointment when approval of new therapies is delayed or they are not approved at all.

Some people told us Canada's regulations on assessing and approving rare-disease treatments are too restrictive, take too long and don't encourage industry partners to enter the Canadian market. People were frustrated that many drugs used in other countries are not available here. Sometimes that's because Canada has not approved a drug, other times it's due to companies not bringing drugs here because the approval system is hard to navigate and the market too small to be worth the investment.

Of the options posed in the discussion paper to address this issue, people overwhelmingly ranked innovative approaches to approval and coverage as the most important option for building a better evidence base. These approaches include pay for performance, early access and managed access where real-world data is collected to help inform decisions about coverage. Patients, their caregivers and clinicians said they should

[4] Pay for performance and managed access, where drug coverage is tied to how well a drug works (tracked by long-term studies as patients are taking it with regular reporting on its effectiveness and safety) were the examples given in the discussion paper.

be involved in defining "performance," because outcomes differ among patients as do perceptions of what constitutes a good outcome. Some people warned pay for performance models might not work for all drugs. It would be important to learn from how other countries have used such agreements and consider how they can be best used and managed in a Canadian context.

Some thought "benefit" and "improvement" should be redefined for drugs for rare diseases to help inform decisions in partnership with patients and clinicians. Too often, rare-disease drugs are discontinued or not covered because they don't meet the criteria for benefits. With the involvement of patients and clinicians, establishing start and stop criteria for rare-disease drugs was deemed important. As one participant put it, "It is obvious that traditional models are not working for rare-disease drugs. A national expert panel will be well positioned to consider the evidence . . . international collaboration will result in more evidence collection."

Many people pointed out the processes for approving drugs for common diseases are not suited to assessing rare-disease drugs. They said governments should be more open to accepting different types and amounts of evidence — mentioning anecdotal evidence and patient experience (i.e. patient-reported outcomes) in particular, to supplement available evidence.

People supported several ideas to improve the timeliness of drug approvals in Canada. One was using the decisions of trusted foreign regulators, fast-tracking drug approval for rare-disease drugs already approved in Europe and the United States without requiring additional Canadian evidence. Another was to create national databases or patient registries that would monitor patients' treatments, outcomes, and disease progression over time. Since the evidence is often uncertain in the early stages of market authorization, collecting this information would allow decision-makers, patients and their clinicians to have more information about starting, continuing or stopping treatment. People mentioned using existing Canadian patient registries[5] as foundations for databases. Pediatric clinicians and researchers called for the development of databases to monitor the disease progression and treatment efficacy specifically in children, for whom real-world evidence is even scarcer than for older rare-disease patients. It was noted that children who receive life-changing curative treatments often experience lifelong side effects, and the investigation and treatment of these side effects is an important aspect of treating rare diseases in children that should not be overlooked.

> **"The collection of real-world evidence, coupled with global data-sharing and…expanded access programs to be scrutinized by independent national and international experts (free from any conflict-of-interest) will help to strengthen the evidence base."** — *Clinician*

While 'innovative approval and coverage models' was the single most popular option for strengthening evidence on rare-disease drugs, most participants felt they should be supported by a combination of the other options — a national data system, independent national and international evidence-sharing networks, and a national expert panel (to review the evidence base).

We heard that expert panels should involve the right combination of individuals with expertise in the specific disease and treatment being considered, including patients with lived experience, clinicians and researchers. Some people felt expert panels should include international members and be linked to independent national and international evidence networks. It was mentioned that some countries have open rotating seats for specialists as part of their evaluation process. Some posed concerns that creating a national expert panel to study data and monitor the impact of drugs will add another layer of bureaucracy to an already onerous system.

[5] Existing registries mentioned include Canadian Fabry Disease Initiative, Canadian Inherited Metabolic Diseases Research Network, Canadian Cystic Fibrosis Registry and Canadian Neuromuscular Disease Registry.

"Experts from outside Canada — those who have nothing to gain or lose could share worthwhile knowledge. Follow other examples — don't rebuild the ark! Other nations have a system that works — look to Australia or the U.K. for national health care systems that support rare-disease treatments. Learn from the investment and experience of others." — *Patient advocacy group*

Participants said rare-disease databases should be transparent, publicly funded and run by neutral third-party organizations. We heard that government leadership and support is important for creating and managing a data system that is integrated with other health databases, protects patient privacy, and is accessible only to those who need the information. Patients said it's not always clear who is funding a trial, program or a patient advocacy group, and transparency in who sees and uses patient data, and why, would increase confidence in the system and make patients feel safer participating in research. People were clear that databases must respect the expertise of patients and families and be linked across the country and internationally to help Canadians receive the best care.

A number of people felt the discussion paper overlooked an important option for improving evidence — investing in open science, where researchers share anonymized data freely for anyone to work with and build on. That could allow academic and research institutions to develop new treatments faster, as happened when scientists around the world worked together on COVID-19 vaccines. Treatments developed through open science might also be sold in Canada and abroad for less than established drug companies would charge.

"We need to reimagine drug discovery and development in many areas, including rare diseases, as pre-competitive, publicly financed, mission-oriented, hypothesis-driven scientific discovery, and uncouple it from commercial decision-making around manufacturing, marketing and distribution." — *Non-profit organization*

Participants suggested a relatively small investment in data infrastructure at the outset would go a long way to creating a more sustainable rare-disease strategy by encouraging industry to do research here and to promote open science initiatives as well. People felt such an investment would yield better treatments and outcomes for patients as well as significant savings over the long term.

What we heard . . . about balancing spending on drugs for rare diseases and keeping Canadian health care sustainable overall

Many people objected to the emphasis on the high cost of drugs, which overlooks the value they bring patients, the health system and the country as a whole. Many participants, including patients, caregivers, clinicians, and industry members suggested removing 'high-cost' from the strategy name. Participants across all engagement platforms raised the point that patients who benefit from drug treatments are likely to need fewer operations, hospital visits and follow-up treatments in the future. People also mentioned drugs' potential benefits of reducing the burden on caregivers and increasing productivity.

"We seem overly focused on cost without looking at the benefit to the system. In many cases, these drugs, while costly, prevent an enormous burden and cost to the system. We must stop taking such a myopic view of the price tag by itself and look at the whole picture: The drug has X benefit in terms of patient outcomes, quality of life, etc. and has the potential to save Y amount in future interventions which no longer become necessary. When looked at this way the benefit equation is much better." — *Patient, family or caregiver*

Despite these concerns, participants offered a great deal of valuable feedback on how to improve coverage while maintaining the sustainability of Canadian health care. Pediatric clinicians and other stakeholders called for more investment in screening, testing, and diagnostic technology, because early diagnosis of childhood illnesses leads to faster treatment and ultimately healthier adult lives for many children with rare diseases. In the long run, that could reduce health care costs.

Many questioned why drugs for rare diseases are as expensive as they are. As one participant put it: "While development costs are an important consideration generally, there is no evidence that any relationship exists between the list price of a drug and the actual development cost of that drug. Rather, drugs seem to be priced based on a combination of external reference pricing to maintain artificially high list prices in a global market, along with a willingness to pay by large plans (public and private) . . . commonly among drugs for rare diseases, there may be no comparable product against which to benchmark value."

Generally, participants felt there is a need for more transparency for how drug prices are set and that governments should work together toward lower prices for rare-disease drugs. We heard that Canada's pricing and reimbursement environment should ensure that drugs are affordable and accessible, and encourage industry to bring drugs to market for Canadian patients.

Participants ranked "sharing costs and pooling risk" as the most effective option for ensuring patients get the drugs they need while controlling costs. Generally, people supported the need to establish a long-term and sustainable funding model with involvement and collaboration from payers and industry. However, participants varied in how they thought sharing and pooling should work. Some suggested sharing the risk across the entire Canadian population by allowing the federal government to reinsure drug plans (provincial and territorial public plans or private insurance plans), so costs for drugs over a specified dollar threshold would be paid for by the federal government. As one participant with lived experience said, "If insurance companies and the government made a plan together on how they would jointly cover rare-disease medications, the costs could be spread over many people to lessen the impact on each payer on its own . . . [and] this could create a greater ability to negotiate prices with pharmaceutical companies due to purchasing power."

As we've seen throughout this report, pay for performance sparked considerable discussion on questions of access and improving limited evidence. It came up as a way to help the sustainability of drug plans, but some researchers and academics warned it could do little for sustainability and does not lead either to lower costs or more effective treatments. One researcher said "Ultimately, it's just a scheme that puts essentially more of the cost of drug development [on] the public purse and yet recouping the price per unit of benefit for the firm. So I think we need coverage innovation. I think we need regulatory innovation. But it has to be for the patient and the health systems first and foremost."

We heard overwhelming support for the suggestion that any solution Canada develops must involve international collaboration. There were calls for Health Canada to look at other jurisdictions with early access and pay for performance models, including France, Germany, Australia and the United Kingdom. Input was mixed on whether studying the United States would be useful, but overall most participants thought looking at European jurisdictions would be more beneficial.

There was interest in supporting "made in Canada" innovations and in up-front investments to reduce the risk involved. Researchers in particular spoke about the lost drug manufacturing capacity in Canada over time and the importance of revitalizing this capacity as a long-term solution. Many people throughout the engagement urged Health Canada to narrow the scope of the strategy and focus the money available for rare-diseases drugs, with the opportunity to expand into other important initiatives down the line.

There were also many who felt more needed to be done to encourage clinical trials to take place in Canada because they are a way for patients to try new and promising treatments. However, people acknowledged that sometimes companies run drug trials in Canada as part of a strategy to seek market approval in larger markets, but then don't make them available here when the research is completed. Some people told us that

the federal government should take steps to prevent this from happening without reducing the number of clinical trials available in Canada.

What we heard . . . from Indigenous partners

Health Canada invited representatives of Indigenous governments and organizations to participate in the engagement process in the way that best suited them. These conversations were just the start; Health Canada is committed to having ongoing dialogue throughout the strategy's design and implementation, but what follows are some initial insights.

We heard that Indigenous Canadians (First Nations, Inuit and Métis) experience a significant burden of illness and poorer health outcomes than non-Indigenous Canadians. As a result, we were told, it's important that discussions about Indigenous health care and health benefits need to acknowledge and address the broader social and community challenges Indigenous peoples face. Furthermore, we heard that First Nations, Inuit, and Métis peoples often have unique experiences navigating health benefits and accessing care within the health system — the experience of Inuit people, for example, may not be the same as Métis people. The importance of the national strategy acknowledging and using a distinctions-based approach was emphasized throughout the conversations.

Indigenous peoples access prescription medications in a variety of ways: through private drug plans, provincial and territorial government programs, community programs, or the Non-Insured Health Benefits program administered by the federal government or self-governments. It was reinforced that Health Canada needs to take actions to close the widening gap in health status between Indigenous peoples and non-Indigenous peoples.

Some of the challenges we heard about include:

- First Nations peoples are sometimes caught in a "jurisdictional vacuum," because health care is delivered provincially or regionally, but First Nations also receive federal programs and services. This can result in gaps in care. One participant described provincial borders as "a construct of colonization," and it was emphasized that national consistency is crucial for the health of Indigenous peoples.
- Indigenous peoples face widespread systemic racism in the health care system, both from health-care providers and through policies. This can discourage people from seeking necessary care and affect the quality of care they do get, both of which undermine overall health. In turn, poor health may reinforce stereotypes and perpetuate inequity.
- When patient-support programs provide coverage for medications not covered by provincial drug plans (often as "compassionate care") and the coverage is later discontinued, patients feel abandoned and hopeless because they can't afford to continue treatment. In many cases, the patient was not aware of or did not understand the terms of the compassionate coverage.
- Difficulty getting approval for and access to prescription drugs is not limited to drugs for rare diseases, and any program or framework should enable quick and flexible access to prescription drugs.
- Some Indigenous peoples may have a different understanding of what a rare disease is. There's a need to ensure Indigenous peoples are involved in and understand how the national strategy will define a rare disease. It is also important to look at and compare definitions from global organizations such as the World Health Organization.
- There are often gaps in data collection as well as delays in diagnosis and access to health services in Indigenous communities, so there is no accurate picture of the burden of rare disease among Indigenous peoples.

People told us a national strategy must be built on a foundation of trust. That means incorporating social justice values and cultural safety training, and being clear how equity will be addressed. There should be mechanisms to identify and resolve systemic racism and biases, including awareness training and education for clinicians. Patients should also receive trauma-informed care, which recognizes the connections between trauma, poor health and negative behaviour.

Indigenous participants felt it would be important to ensure transparency in decisions on an individual patient's eligibility for rare-disease drug funding, including a clear process for determining who has a rare disease, a clear explanation why a patient has been denied funding and an appeal process for when that happens. Others said the strategy should provide for continuing coverage for patients who have lost funding for their drugs, and to protect families and communities from having to take on that financial burden. In cases where evidence no longer supports funding, there should be follow-up care to help patients find alternative resources. In a related point, we heard the need for strong social services support is often closely linked to improving the health of Indigenous peoples, but both systems can be hard to navigate. We were told one common access point is very important so Indigenous peoples can get more seamless access to the provincial and community services they need.

What impact the national strategy would have on programs in place now was a concern for participants. There are numerous agreements between Indigenous, federal and provincial governments on funding and delivering health services. Increasingly, Indigenous nations design and deliver their own health services. The strategy needs to respect existing agreements, have continued dialogue with Indigenous partners at decision-making tables, and ensure care is maintained.

Conclusion and Next Steps

It is clear that ensuring rare-disease patients have access to effective treatment is a complex and multi-faceted challenge, requiring creative solutions, coordination, and collaboration. Throughout the engagement, people overwhelmingly felt that all options posed in the discussion paper under the three issues were important and interrelated. However, for the first issue on national consistency, participants said a single framework for rare-disease treatments would make access fairer across the country. A national approach was the most important element for a strategy and should be guided by key principles and values such as patient-centredness, transparency, accountability, and flexibility. For the second issue on building a better evidence base for decision-making, the majority of people supported innovative approaches to approval and coverage. Lastly, for the third issue focused on sustainability, participants ranked "sharing costs and pooling risk" as the most effective option in which payers and industry work together to ensure patients get the drugs they need in a timely manner.

Other important aspects we heard include ensuring that the strategy improves patient outcomes, builds on public programs and existing infrastructure, develops better system coordination and alignment, and involves international collaboration. All stakeholder groups voiced that they want to be involved in guiding, developing, implementing and maintaining a national strategy for drugs for rare diseases. For patients, caregivers and clinicians in particular, this means having places at any table where treatments for rare diseases are discussed, where evidence is assessed or data collected and evaluated, and ultimately, wherever decisions are made that will shape their lives and access to treatments.

Overall, the Canadians we heard from are passionate about creating a strategy for drugs for rare diseases. People told Health Canada we do not have to get it right on the first go — taking small steps is fine. People felt it was better to start as soon as possible with the mindset that trying pilots, being flexible and making improvements along the way is better than spending too much time planning. As one participant put it, we shouldn't let the perfect be the enemy of the good.

Next steps that were important for participants include:

- Working closely with provinces, territories, Indigenous partners and other stakeholders to develop a coordinated strategy that get patients the effective treatments they need;
- Establishing common definitions of "rare diseases" and "high-cost drugs" and better guidelines for what constitute "benefits" and "improvements" for assessing rare-disease treatments;
- Researching best practices in international jurisdictions (including France, Germany, the U.K. and Australia) to learn from them and avoid reinventing the wheel;
- Ensuring Canada's regulatory approach, drug pricing landscape, and Canadian research and development, commercialization and drug manufacturing capacity are conducive for rare-disease drugs; and
- Considering how best to support patients more holistically, including diagnostics, screening and genetic testing.

The Government of Canada would like to thank all of those who participated in this engagement, which has provided us with valuable insights and practical suggestions to better serve patients with rare diseases. We heard clearly that progress requires close collaboration among governments, patients and families, Indigenous peoples, the clinical community, industry, private drug plans and other stakeholder groups. As next steps, Health Canada will closely review the ideas and suggestions we have heard from everyone throughout the engagement process. Over the summer, as a follow up to the report, Health Canada will continue discussions with patients and caregivers, health care professionals, researchers, private drug plans, the pharmaceutical industry, and other stakeholders. In addition, Health Canada will continue discussions and collaboration with provinces and territories, pharmaceuticals management system partners, and Indigenous partners to build a strategy that will work for all Canadians. Further public consultation to inform a comprehensive model for the national strategy will take place in the fall. With the support of all those involved, the Government of Canada will work towards launching the national strategy for drugs for rare diseases in 2022.

Appendix A – What we asked: questions from the discussion paper

These are questions asked in the online questionnaire, public town halls and stakeholder meetings.

1a. How can access to high-cost drugs for rare diseases be made consistent in order to improve patient access to these treatments?

1b. Which of the proposed options, or combination of options, would be the most effective for improving access and improving consistency? [select all that apply]

- A single framework for decision making on high-cost drugs
- A transparent coordinating body
- Patient and clinician engagement
- Co-ordinated support for research on rare diseases in Canada

1c. Please explain the option(s) that you selected above.

2a. How can decisions on covering high-cost drugs for rare diseases be made when the evidence is limited?

2b. Which of the proposed options, or combination of options, would be most effective for strengthening the evidence base? [select all that apply]

- Innovative approval and coverage models
- A national expert panel
- A national data system
- Independent national and international networks

2c. Please explain the option(s) that you selected above.

3a. Which of the proposed options, or combination of options, would be most effective for getting rare-disease treatments to patients? [select all that apply]

- Sharing of costs and pooling of risks
- Investments up front to reduce the risk in early development
- Pay for performance
- Supports for "made-in-Canada" innovation
- International collaboration

3b. Please explain the option(s) that you selected above.

4. Do you have other ideas that might help improve access and lower costs for drugs for rare diseases?

After our first round of stakeholder engagement on the discussion paper questions, we organized multi-stakeholder wrap-up meetings to gather more information on three themes that emerged and provide stakeholders with another opportunity to provide input. These are the questions we asked at the wrap-up meetings.

Theme 1: Strategy scope and definition

1. How should a drug for a rare disease be defined?
2. How can we ensure the strategy includes drugs for multiple therapeutic areas, as well as different types or formats of treatments?

Theme 2: Funding and risk-sharing models

3. What does risk-pooling or cost-sharing mean to you and who would you suggest be involved?
4. What does pay for performance mean to you and what form could it take in Canada?

Theme 3: Infrastructure and supports

5. How could the current pharmaceuticals management system be more streamlined?
6. How could a data system(s) be used? What kinds of decisions could this data help inform?

Overarching question

7. In relation to the themes, are there existing models Canada should explore? Are there any best practices or lessons learned you wish to share?

Appendix B – Poll results

The following table shows the options posed in the discussion paper for each issue, and the total proportion of participants who selected the option as important or effective.

Participants were polled during the virtual public town halls and stakeholder meetings, and were able to 'select all that apply' in the online questionnaire. The total number for virtual meetings reflects the number of people who participated in the poll, not necessarily everyone in attendance. It is important to note participants did not view the options as mutually exclusive and felt most were important and interrelated.

Responses	Online Questionnaire	Virtual Meetings	Total Number	Total Proportion
Q1: single framework for decisions	95/136	184/281	279/417	67%
Q1: transparent coordinating body	83/136	118/281	201/417	48%
Q1: patient and clinician engagement	89/136	123/281	212/417	51%
Q1: support for research	86/136	62/281	148/417	35%
Q2: innovative coverage models	86/136	164/291	250/427	59%
Q2: expert panel	73/136	88/291	161/427	38%
Q2: national data system	87/136	146/291	233/427	55%
Q2: independent national/ international networks	99/136	117/291	216/427	51%
Q3: sharing costs/pooling risk	93/136	184/298	277/434	64%
Q3: pay for performance	61/136	105/298	166/434	38%
Q3: made in Canada solutions	89/136	51/298	140/434	32%
Q3: upfront investments	68/136	56/298	124/434	29%
Q3: international collaboration	92/136	126/298	218/434	50%

Appendix C – Who we heard from

Health Canada would like to thank each individual and every organization who provided their time, advice, and insights on how to design and implement a national strategy for drugs for rare diseases.

More than 650 individuals and organizations participated in the engagement process. Representatives from the following organizations registered for various virtual sessions and/or made a submission to Health Canada. For privacy reasons, the list does not include the names of over 70 individuals, members of the public, and patients who participated in meetings, or made written submissions.

Participants

20Sense
3Sixty Public Affairs
Actra Fraternal benefit Society
Aetion
aHUS Canada
Alberta Blue Cross
Alberta Health Services
Alberta School Employee Benefit Plan
Alexion Canada
Alliance des patients pour la santé
Alnylam Pharmaceuticals
Alpha-1 Canada
ALS Action Canada
ALS Society of Canada
Amicus Therapeutics
Answering TTP Foundation
Aon Health Solutions
Arthritis Society
Assembly of First Nations
Association des pharmaciens des établissements de santé du Québec
Association québécoise des personnes de petite taille
Association québécoise des pharmaciens propriétaires
Astellas Pharma Canada
AstraZeneca
Avir Pharma Inc.
Barbara Jaszewski consulting
Bayer
Bayshore HealthCare
BC Children's Hospital
BC Housing
BC Ministry of Health
BC Transplant
Beneva
Best Medicines Coalition
Bigstone Health Commission
BioAlberta
Biogen Canada
Biosimilars Canada
BIOTECanada
Boehringer Ingelheim Canada
Brain Tumour Foundation of Canada

Bristol Myers Squibb
Business Council of Canada
Canada Health Infoway
Canada Life
Canadian Agency for Drugs and Technologies in Health
Canadian Association for Pharmacy Distribution Management
Canadian Association of PNH Patients
Canadian Blood Services
Canadian Breast Cancer Network
Canadian Cancer Society
Canadian CML Network
Canadian Council of the Blind
Canadian Doctors for Medicare
Canadian Fabry Association
Canadian Federation of Independent Business
Canadian Federation of Nurses Unions
Canadian Generic Pharmaceutical Association
Canadian Health Coalition
Canadian Hemophilia Society
Canadian Immunodeficiencies Patient Organization
Canadian Indigenous Nurses Association
Canadian Institute of Actuaries
Canadian Institutes of Health Research
Canadian Labour Congress
Canadian Life and Health Insurance Association
Canadian Medical Association
Canadian MPN Research Foundation
Canadian Organization for Rare Disorders
Canadian Pediatric Society
Canadian Pharmacists Association
Canadian PKU & Allied Disorders
Canadian Pulmonary Fibrosis Foundation
Canadian Skin Patient Alliance
Canadian MPS Society for Mucopolysaccharide & Related Diseases
Canadian Society of Hospital Pharmacists
Canadian Union of Public Employees
Carleton University
Centre for Health Evaluation and Outcome Sciences
CF Get Loud
Children's Hospital of Eastern Ontario
Children's Hospital of Western Ontario
Children's Healthcare Canada
ClaimSecure Inc.
CML Society of Canada
College and University Retiree Associations of Canada
Colorectal Cancer Canada
Comité des Usagers de la Mauricie-et-du-Centre-du- Québec
Connex Health Consulting
Conseil du patronat du Québec
CSL Behring Canada
Cure SMA Canada
Cystic Fibrosis Canada

Dalhousie University
Denis Garand and Associates
Desjardins Group
Durham District School board
Edmonton Medical Genetics Clinic
Employee Life and Health Trusts in Ontario
Entrepreneurship & Innovation for Leber's Congenital Amaurosis
Equal Access for Rare Disorders
Express Scripts Canada
Fibrose kystique Canada
Fighting Blindness Canada
First Nations Health Authority
FORUS Therapeutics
Foundation Prader-Willi Research Canada
Fraser Health
Garrod Association
Gastrointestinal Society
Gilead Sciences Canada
Green Shield Canada
Grifols Canada
GroupHEALTH
H3 Consulting
HAE Canada
Health Charities Coalition of Canada
Health Coalition of Alberta
Health Sciences Centre Winnipeg
Heart and Stroke
Hoffmann-La Roche Ltd
Horizon Therapeutics
Hospital for Sick Children
Humber College
Huntington Society of Canada
iA Financial Group
IMV Inc.
Independent Voices for Safe and Effective Drugs
Innomar Strategies
Innovative Medicines Canada
Institute of Health Economics
Intercept Pharma Canada Inc.
Inuit Tapiriit Kanatami
Ipsen Biopharmaceuticals Canada
IWK Health Centre
IQVIA Canada
Jazz Pharmaceuticals Canada
J.L. Glennie Consulting Inc.
Janssen
Jesse's Journey Canada
Johnson & Johnson
Kingston Health Sciences Centre
La Societe LMC du Canada
Life Sciences Ontario
Liv A Little Foundation

Manitoba Blue Cross
Manulife Financial
Mark Freedman & Judy Jacobs Gaucher Clinic (Mount Sinai Hospital)
Mastocytosis Society of Canada
McGill University Health Centre
McKesson Canada
McMaster Children's Hospital
McMaster University
Medavie Blue Cross
Médicament novateurs Canada
Mercer
Merck Canada
Métis Nation of British Columbia
Métis Nation of Ontario
MitoCanada Foundation
Mitochondrial Innovation Initiative (University of Toronto)
Mitsubishi Tanabe Pharma Canada
mls Financial Services
Montréal InVivo
Montreal Neurological Institute
Multiple Sclerosis Society of Canada
Muscular Dystrophy Canada
Myeloma Canada
National Association of Pharmacy Regulatory Authorities
National Research Council Canada
Neighbourhood Pharmacy Association of Canada
Network of Rare Blood Disorder Organizations
Newborn Screening Ontario
Nova Scotia Health Authority
Nova Scotia Health Coalition
Novartis Pharmaceuticals Canada
OMG Benefits Consulting Inc.
Ontario Health
Pacific Blue Cross
Parti Républicain Souverainiste
Peter Gilgan Centre for Research and Learning (The Hospital for Sick Children)
Pfizer Canada
Prairie Mines & Royalty ULC
Protect Our Access
Pulmonary Hypertension Association of Canada
Quality HTA
Queen's University
Rare Disease Foundation
RAREi
RBC Insurance
Regroupement québécois des maladies orphelines
Research Canada
Roche
Ryerson University
Sanofi Canada
Save Your Skin Foundation
Shared Health

Shoppers Drug Mart Specialty Health Network
SickKids Research Institute
Sickle Cell Disease Association of Canada
Sobi Canada
Structural Genomics Consortium
Sun Life Financial
Sunnybrook Hospital
Surrey Board of Trade
Takeda Canada
tc Health Consulting Inc.
Thalassemia Foundation of Canada
The Children's Hospital Research Institute of Manitoba
The CML Society of Canada
The Cute Syndrome Foundation
The Hospital for Sick Children
The Isaac Foundation
The Miracle Marnie Foundation
Third Party Administrators Association of Canada
Tuberous Sclerosis Canada
Tumour Foundation of BC
Ultragenyx Canada Inc.
Unifor
Unity Health Toronto
University Health Network
University of British Columbia
University of Alberta
University of Calgary
University of Manitoba
University of Ottawa
University of Saskatchewan
University of Toronto
Vancouver General Hospital
Vertex Pharmaceuticals Canada
Vivre Avec La Fibrose Kystique
weCANreg Consulting Group Inc.
Willis Towers Watson
York University