Nothing About Us Without Us:

Reframing Canadian rare disease policy while amplifying the rare blood disorder voice

Network of Rare Blood Disorder Organizations
Réseau des Associations Vouées aux Troubles Sanguins Rares

immunity canada

A 2024 report and recommendations for policymakers, prepared by Immunity Canada and the NRBDO.

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This report was written on the traditional and unsurrendered territory of the Anishinaabeg people, which include the Odawa, Ojibwe, and Pottawatomi Nations, and the Lkwungen People, also known as the Songhees and Esquimalt First Nations communities. We acknowledge the ongoing injustices and resulting health inequities Indigenous people face on these lands.

We are grateful to Chris, an ITP patient, for sharing her experience to include in this report and to inform our recommendations.

We also wish to acknowledge the people affected by rare blood and bleeding disorders across Canada, represented in this report's statistics and figures. You are not just numbers to us; we stand alongside you on the journey toward timely and equitable access to treatment and care.

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Visit www.nrbdo.ca/drd for the most up-to-date version of this publication and updates on our patient group-led advocacy efforts on this issue.

About This Project

Immunity Canada and the Network of Rare Blood Disorder Organizations (NRBDO) have collaborated to create a report for Health Canada and federal, provincial, and territorial policymakers. The report demonstrates the importance of incorporating the perspectives of individuals with rare blood, bleeding, and immune disorders (hereafter referred to in this report as "rare blood disorders") in the process of creating policies to benefit people living with rare disorders more broadly.

Like most rare diseases, rare blood disorders often pose significant challenges to patients, impacting their quality of life, access to healthcare, and overall well-being. Yet their pathways to treatment access and care can differ significantly from those of other rare disorders and are not necessarily captured in the current dialogue on rare diseases. By including these voices in policy development, governments and healthcare organizations can ensure that policies are more inclusive, effective, and responsive to the needs of these populations.

As sometimes happens, we feel the value of the project lies more in the process than in the final report itself, starting many meaningful conversations which we hope will lead to the creation of greater inclusivity, equity, better understanding, and ultimately, a stronger National Drugs for Rare Diseases Strategy.

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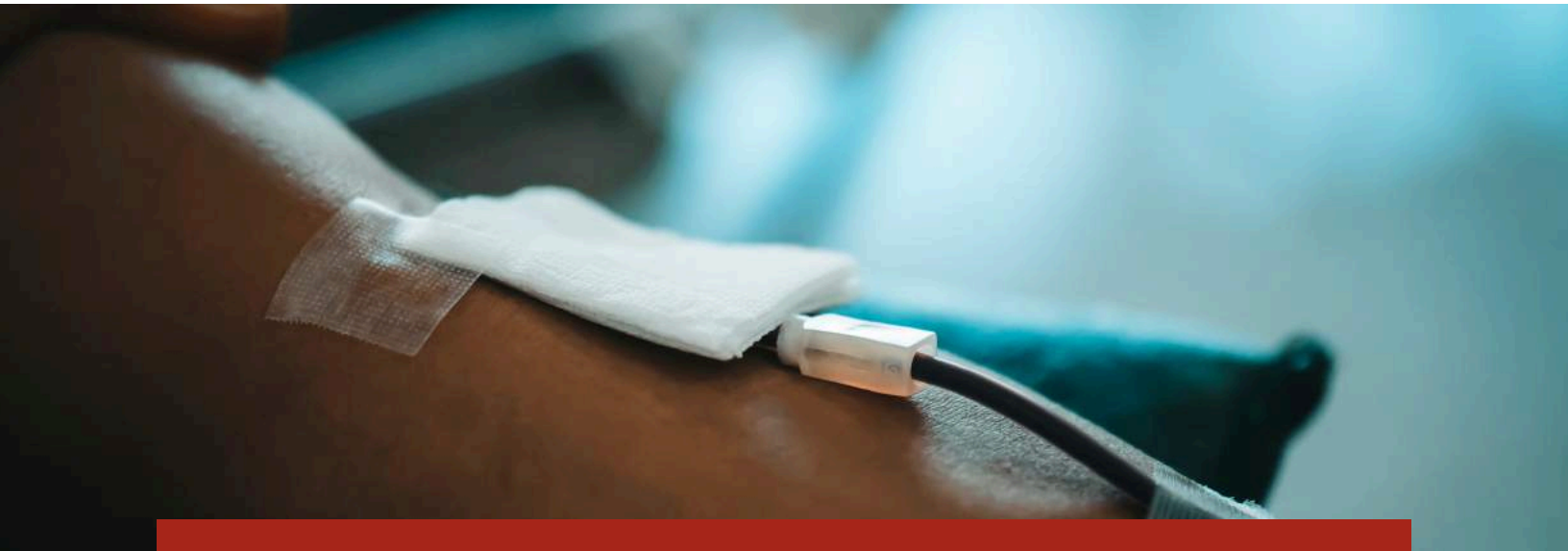


The Other Rare Disease Community

Rare blood disorders encompass diverse conditions, including hemophilia, primary immunodeficiency, sickle cell disease, Guillain-Barré syndrome, and many more. While individually rare, collectively, they impact hundreds of thousands of Canadians and millions of people worldwide.

Despite advancements in treatment, individuals living with these disorders often face barriers to accessing timely and appropriate care.

While Canada is making progress in addressing issues facing the rare disease community, currently, the interests and needs of patients with rare blood disorders are not well represented in the rare disease discourse. These are primarily patients who use blood and plasma-derived therapies and related products, including those used for non-standard or unconventional applications given the rarity of their disease.

The rare blood disorder community and rare disease patients who rely on therapies derived from blood and plasma or their substitutes are in a unique situation—including very limited benefit from a common DRD formulary—and merit recognition as a distinct voice within the rare disease patient community.

Canada's Existing National Formulary

In addition to their role in collecting and distributing whole blood donations, stem cells, and organs, Canadian Blood Services (CBS) has the responsibility for operating a well-evolved pan-Canadian formulary that is evidence-based, transparent, and provides equitable access to safe blood and plasma-derived medicinal products.

CBS has had over 20 years to create and learn from its national formulary, working collaboratively with stakeholders. In this model, Health Canada acts as the regulator, and the provincial and territorial Ministries of Health act as the funders and corporate members.

CBS's formulary represents a budget of $834M in 2023 [1] and provides over fifty products treating several different conditions, almost all of them rare. CBS and the patient communities served by this formulary should be considered key stakeholders in developing a national drugs for rare diseases strategy.

Treatments for Rare Blood, Bleeding and Immune Disorders

This table illustrates the current state of treatment access in Canada and therapies in the pipeline for 22 rare blood disorders, as gathered from the relevant patient organizations. Of note, while only an estimated 5% of rare diseases have effective treatments, over 95% of the disorders represented here have effective treatments available in Canada.

Table 1. Rare blood disorders and their respective therapies by formulary.

Disease	Product on CBS Formulary	Product on P/T Formularies	Product Not Available in Canada	Gene Therapy?	Drugs in Pipeline?
Alpha-1	Augmentation therapy		Other augmentation therapies		Yes
Aplastic Anemia	Supportive care	ATG, cyclosporine			Yes
aHUS	N/A	ecluzimab, ravulizimab			Yes
CIDP	IG	efgartigimod, rituximab (off-label)			Yes
GBS	IG				Yes
HAE	C-1 Esterase inhibitors (mulitple)	berotralstat, icatibant lanadelumab, tranexamic acid,			Yes
Hemophilia A	10 extended life, 1/2 life & factor products	aminocaproic acid, desmopressin, tranexamic acid		Yes	Yes
Hemophilia B	6 extended life, 1/2 life & factor products	aminocaproic acid, tranexamic acid		Under review	Yes
HHT	Supportive care	iron transfusions			Yes
ITP	IVIG (1st-line)	steroids and Anti-D (1st), rituximab (off-label) (2nd)			Yes
Kawasaki Disease	IVIG	anakinra, infliximab			Yes
Mylodysplasia	Supportive care	ATG, cyclosporine, ledelidomine			Yes
MPN	Supportive care	anagrelide, ruxolitinib			
MG	IG	efgartigimod, rituximab			Yes
MMN	IG				Yes
PNH	Supportive care	blood thinners, eculizumab, immunosuppression			Yes
Porphyria	Hemin	gene therapy		Yes	Yes
PI	IG	abatacept, ritumximab, Jak inhibitors	leniolisib, mavorixafor, other IG brands	Yes	Yes
Sickle Cell Disease	Supportive care			Under priority review	Yes
Thalassemia	Supportive care	deferasirox, deferoxamine		Under priority review	Yes
TTP	Plasma exchange	rituximab (off-label)	caplacizumab		Yes
VWD	Factor product	desmopressin			

The National Drugs for Rare Diseases Strategy & the Federal Role: A Question of Framing

Recently, the National Drugs for Rare Disease (DRD) Strategy has been created to address the unmet needs in the rare disease community and the high cost of rare disease drugs to provincial and territorial drug plans.

With the framing as a problem of expensive drugs for rare diseases, the solution that has emerged is that, of the $1.5B pledged for the strategy over 3 years, $1.4B will be used to create and fund a common list of drugs across the provinces and territories through bilateral agreements.[2] This introduces many challenges—including no mechanism for this funding to go toward treatments on the CBS formulary—and as the timeline is currently unknown, the funding of $1.4B is unused and not benefiting patients.

Reframing the objective as moving us toward timely and equitable access to treatment and care for rare disease patients allows the patient community, clinicians, researchers, and policymakers alike to reimagine a role for the federal government to:


support research and data collection, including clinical trial sites and patient registries


create a National Newborn Screening Program


engage with manufacturers to bring novel diagnostic and therapeutic technologies to Canada


improve timelines and lessen uncertainty in our health technology assessment processes


provide clear guidance to the provinces about reimbursement for new indications for approved therapies, including off-label usage

The optimal role of the federal government is to create a **national infrastructure** that supports timely and equitable access to treatment and care for rare disease patients.

> The additional investment is not accompanied by any guidelines on how to spend it, that is to say, how to determine "value for money" for expensive DRDs. In a "pay anything for DRDs" scenario, especially when everyone knows how big the pot is, pharmaceutical companies would be incentivized to set the highest price possible to try and get as large a piece of that pie as possible. Higher prices lead to drugs being unaffordable and not covered by public drug plans. Ironically, throwing money at the problem without specific guidelines on how to spend it could in itself prove to be a barrier to patient access.
>
> *-Aravind Ramanathan, Carelton University, School of Public Policy and Administration*

To date, investments through the National DRD Strategy have aligned with this objective of creating a national infrastructure:

Research and Data Collection

In the rare disease context in Canada, there are often no clinical trials or research sites to gather clinical evidence or patient data. These small patient populations and lack of data make quantifying exact numbers or dollar amounts difficult, creating a large knowledge gap.

Clinical Trial Network

In February 2024, the federal government announced an investment of $20M to create the RareKids-CAN Pediatric Rare Disease Clinical Trials and Treatment Network. The purpose of the Network is to "foster collaboration among researchers, patients, caregivers, health care providers, and policymakers; streamline clinical research; and support national and international clinical trials to advance discoveries, enable better prevention, diagnosis, and treatments, to improve health outcomes for children and adolescents affected by rare diseases." [3]

Patient Registries

The need for patient registries to produce quality real-world data (RWD) has long been recognized. However, the capacity and resources required to create and maintain a patient registry are challenging in the rare disease community. The national DRD Strategy has acknowledged this, and funding has been allocated to assist in creating and maintaining patient registries to inform the HTA process. Unfortunately, the first RFP process was prohibitive for patient organizations exactly because of this limited capacity and resources.

Advances in Newborn Screening

We welcome the creation of a National Advisory Panel on Newborn Screening, as recommended in our 2022 report, "*Timing is Everything: Toward a national newborn screening program for rare disorders,"* [4] to advance equity for all babies in Canada. The advancements in newborn screening and early diagnosis have significant implications that must be considered.

Importance of Different Patient Perspectives in Policy Making

1. **Ensuring Patient-Centered Care:** By involving patients in policy development, governments and healthcare organizations can ensure that services are designed around patients' needs, preferences, and priorities.
2. **Identifying Unmet Needs:** Patients offer unique insights into the challenges they face daily, helping policymakers identify gaps in existing services and areas for improvement.
3. **Promoting Equity:** Including diverse patient perspectives in policymaking helps ensure that policies are equitable and address the needs of all segments of the population, including those from marginalized communities.
4. **Enhancing Policy Effectiveness:** Policies developed with patient input are more likely to be effective and sustainable as they are grounded in the lived experiences of those directly affected.

A Reframed National DRD Strategy

Engage manufacturers to bring novel diagnostic and therapeutic technologies to Canada

CASE STUDY: CD3DELTA SCID GENE THERAPY

The current treatment protocol for infants with Severe Combined Immune Deficiency (SCID) is allogeneic hematopoietic cell transplantation, which requires a matched donor or gene therapy. Gene therapy for ADA SCID, a subtype of SCID, is currently unavailable in Canada. It costs around $3 million, and coverage is at the discretion of the P/T. Inspired by a recent CIHR grant allowing Canada to develop point-of-care CAR-T therapies for blood cancers, Dr. Nicola Wright, Pediatric Hematologist and Immunologist at Alberta Children's Hospital, advocates for a similar Canadian infrastructure for gene therapy.

CD3Delta is a SCID variant predominantly found in the Mexican Mennonite population, with high prevalence in the Canadian prairies. Dr. Wright is piloting a gene therapy trial in Calgary for this variant, a first of its kind. However, without point-of-care gene therapy infrastructure in Canada, the patient's cells will be shipped to the US for gene manipulation and then back to Canada for transplantation back into the patient. All of this increases both time and cost and results in reliance on other countries.

Dr. Wright, along with researchers from Alberta, Manitoba, Ontario, and the city of Los Angeles, California, are paving the way for gene therapy in Canada, using a therapy for the genetic disorder C3Delta SCID as a pilot for a gene therapy framework.

> "Our work highlights the need to explore alternative funding models, including possible public/private partnerships and other potential collaborations. It is not feasible to produce gene therapies for rare blood and immune disorders in the current commercialized model. It is essential for Canada to build a non-commercial pathway to provide these patients access to these therapies close to home.
>
> *-Dr. Nicola Wright, Barb Ibbotson Chair in Pediatric Hematology, Alberta Children's Hospital*

CASE STUDY: MALARIA DEFERRALS FOR BLOOD DONORS

People who have ever had malaria cannot donate blood in Canada. This precaution is warranted because we can only test for malaria antibodies, not active infection. This disproportionately impacts potential Black donors, who are best-positioned to donate blood that is phenotype-matched for Black sickle cell disease patients, a patient community that requires regular blood transfusions.

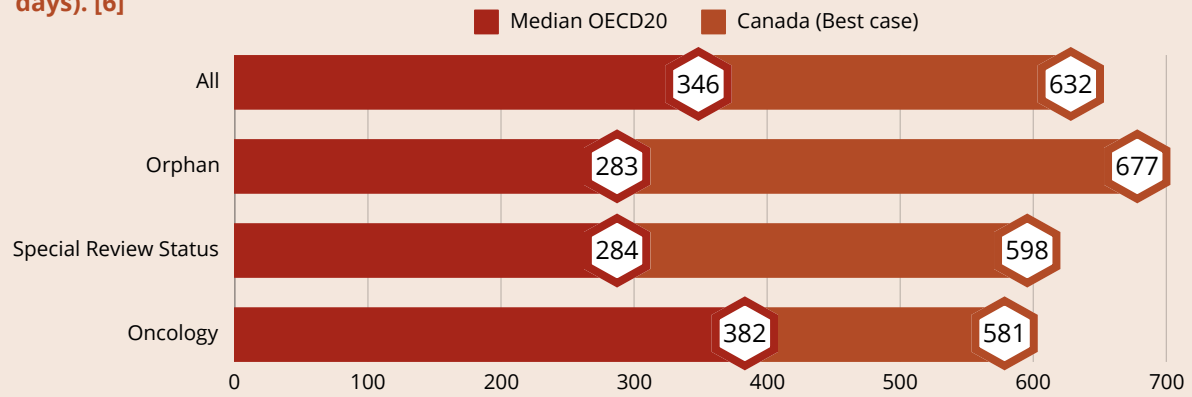
A nucleic acid test that could diagnose a donor with active malaria infection, available elsewhere, would be a game changer for the sickle cell community, but it is not yet available in Canada.

Improve timelines and lessen uncertainty in our health technology assessment processes

Slow Timelines

- With its "fragmented and sequential reimbursement process," Canada is among the slowest of the OECD20 countries to reimburse innovative medicines through public plans, based on the time from the first global launch to public reimbursement. (18th of 20; 926 days vs. median of 519 days). [6]
- Canada's reimbursement process does not appear to prioritize based on patient needs: while other OECD countries are fastest to reimburse orphan and special review drugs, Canada is the slowest in these categories. [6]

Chart 1. Time from Local Marketing Authorization to Public Reimbursement, by Category (in days). [6]



CASE STUDY: QUEBEC

Quebec's 2017-2027 life science strategy includes a commitment to expedited access to promising new drugs and enabled INESSS to take a "promise of therapeutic value" approach.[7]

Since 2018, approximately ten drugs have had a positive recommendation through this novel approach. In June 2024, their reassessments were published for two CAR-T therapies receiving positive recommendations. Both real-world evidence (RWE) and clinical trial data were used in the reassessments.

In 2023, INESSS invited manufacturers to submit "alternative economic scenarios" for consideration by the Minister along with HTA advice. INESSS intends to demonstrate manufacturers' willingness to participate in managing risk and model how to implement innovative market access.

TIME-LIMITED REIMBURSEMENT

In 2023, CADTH (now CDA-AMC) introduced a time-limited reimbursement (TLR) recommendation option in response to treatments "that target smaller patient populations or treat conditions that have limited therapeutic options." Using TLR, regulators may grant earlier approval of these products based on less mature evidence and with certain conditions in place so that patients can benefit from new treatments earlier.

However, the TLR is only for treatments that have received a NoC/c (Notice of Compliance with Conditions) from Health Canada, and unlike the INESSS example above, RWE cannot be used as a data source. Unfortunately, with these conditions attached the opportunity to support timely access through TLR is small.

Provide clear guidance to the provinces and territories about reimbursement for new indications for approved therapies, including off-label usage

CASE STUDY: ITP

Chris lives in British Columbia, and has immune thrombocytopenia (ITP), a rare autoimmune disorder that results in low platelet levels. Different ITP patients respond very differently to the various ITP treatments - what works for some do not work for all. Chris has tried several treatments that have not increased her platelets, including a failed splenectomy. The most effective option for her is eltrombopag. Her private insurance will cover eltrombopag, but the coverage has a lifetime cap, so she is currently on a lower-than-recommended dose to stretch her budget.

BC's pharmacare has denied her request for generic eltrombopag despite the therapy being indicated for ITP and available to ITP patients in Ontario. Generic rituximab, which is not indicated for ITP, is available to BC ITP patients. She also has access to IVIG through CBS, but neither rituximab nor IVIG is an effective treatment for her.

> I've always felt bad getting IVIG, knowing it is really expensive. **But this little pill that helps me more, I can't get**.
>
> *-Chris, ITP patient*

> Access to biologics is a huge obstacle to adult immunodeficiency patients. In Ontario, I personally have had three patients where the processing time took so long that the patients' health had deteriorated by the time it was approved. I know of a patient in Toronto who died waiting for approval.
>
> *-Dr. Jenny Garkarby, Pediatric Immunologist, McMaster University*

CASE STUDY: PRIMARY IMMUNODEFICIENCY

Primary immunodeficiency disease (PI) patients are born with compromised immune systems, leaving them prone to chronic infections. Preventing infections is vital to decreasing morbidity and the risk for certain cancers, lung disease, and other conditions.

While most PI patients (roughly 95%) will receive and depend on IG at some point in their treatment, many will also receive other therapies. For example, many PI patients, both pediatric and adult, would require the use of rituximab, abatacept, or a similar biologic to treat comorbidities associated with their PI. However, these biologics are not indicated for PI in Canada, making access difficult and causing lengthy delays in access decisions.

This results in access issues across Canada for the appropriate treatment of primary immunodeficiencies with biologics and immunomodulators on P/T formularies, greatly impacting patient outcomes.

Additional Challenges & Opportunities

MANUFACTURER-DRIVEN DECISIONS REGARDING INDICATIONS

Introducing novel therapies to the Canadian market is manufacturer-driven and often dependent on market size and profitability. This includes new therapies and considering approved therapies for new or additional indications.

In its newly expanded role, CDA-AMC has the opportunity to provide clear guidance to the provinces on reimbursement for new indications on approved therapies. In the rare disease context especially, this should include off-label usage, therapies where generics and biosimilars are being introduced, and therapies that have been proven effective but that manufacturers have not deemed profitable enough to bring to the Canadian market. In the Canadian rare blood disease space, where the number of patients is relatively low, the return on investment is often found to be lacking, especially when financial disincentives plague the process.

DATA TO INFORM RARE DISEASE RESPONSES

There is a lack of data to support rare disease decision-making in Canada. This includes rare blood disorders.

While measures are being taken to address these issues (for example, funding for rare disease patient registries through the DRD Strategy), the following areas further lend to the data gap in rare disease:

- lack of clinical trial sites in Canada
- insufficient use of experts in Canada (clinical, patient, research)
- patient partners rarely and insufficiently engaged in protocol or policy development
- issues with equity and demographic representation
- lack of adequate tools to determine HRQoL for economic evaluations and connect cost with value.

> If you have to spend $20 million or $30 million to get approval and you have five or 10 patients a year, it's hard to get a return on investment. So we have successful, safe therapies, but it's more the financial, economic elements that are limiting them from becoming approved drugs.
>
> *-Dr. Donald Kohn, Director of the Human Gene Medicine Program at UCLA*

Health Equity in Rare Disease

Indigenous Canadians

It was noted in the Health Canada DRD consultation report that "Indigenous Canadians (First Nations, Inuit, and Métis) experience a significant burden of illness and poorer health outcomes than non-Indigenous Canadians." [8] It is important to recognize that Indigenous communities often have higher rates of certain genetic diseases. For example, a 2024 study has shown that while ADA SCID affects approximately 1 in 200,000–500,000, the reported number in Nunavut in 2020 suggests a significantly higher incidence, in the range of 1 in 600-2000 live births.[9]

Better understanding the prevalence of these conditions in specific communities could help inform public health policies, including newborn screening and immunization programs, and ultimately improve the health of Indigenous children in Canada.

Gender Inequity

Recent studies undertaken by the EURODIS and the National Organisation for Rare Disorders (NORD) showed gender bias in rare disease treatment access and care. [10] Publications from the European Public Health Alliance and the Alliance Maladies Rare indicate that diagnostic delay in women directly impacts health and well-being.[11]

In particular, women with bleeding disorders such as von Willebrand disease (VWD) often face a significant diagnostic odyssey due to stigma related to vaginal bleeding and healthcare provider dismissal of symptoms. The issue is compounded in rural, remote, and northern parts of Canada. As many as 1 in 1000 individuals may be suffering from a bleeding disorder. Treatment is available, but many remain undiagnosed.[12]

Recommendations

1. Include representatives from the rare blood disorder community more inclusively in the DRD process, including on the Implementation Advisory Group (IAG). Leverage patient partners to co-develop plain language summaries and education resources and tools.

2. Expand the scope of CDA-AMC's recommendations to provide clear guidance to the provinces on reimbursement for new indications on approved therapies, including off-label usage, where generics and biosimilars are being introduced, and therapies that have been proven effective but that manufacturers have not deemed profitable enough to bring to the Canadian market.

3. Consider the capacity and resources of small rare disease patient organizations when designing processes for their input, from HTA submissions to RFPs, and where possible, provide assistance, including infrastructure to support participation.

4. Review disease states where IVIG is currently being used as a first-line treatment and where more effective and potentially more cost-efficient treatment options are available.

5. Expand the use of therapies already available in Canada to include off-label or second-line indications, allowing for more equitable and timely access for rare disease patients.

6. Encourage provinces and territories to follow CDA-AMC's reimbursement recommendations without imposing additional restrictions when considering reimbursement for therapies, improving equity across jurisdictions.

7. Establish an expert advisory committee to explore the feasibility of a Canadian infrastructure of publicly-funded point-of-care gene therapy technology to make these therapies more accessible to more Canadians.

We invite policymakers to reframe the federal government's role in Canada's DRD Strategy: not as simply funding a selection of drugs for a chosen few, but as architects of a robust infrastructure to support the provinces and territories in delivering timely and equitable diagnosis, care, and treatment services for all rare disease patients, including those with rare blood disorders. This includes a national newborn screening program, a clinical trial network, efficient HTAs, guidance on reimbursement for existing therapies, and publicly funded technology to provide point-of-care gene therapy.

Future Consideration

Managed Access Frameworks
Many countries, including England, France, Germany, Italy, and Australia, apply adapted HTA and listing pathways to enable patient access while evidence collection is ongoing. This helps them manage the uncertainties inherent in drugs for rare diseases. Canada does not yet have a formalized early access pathway.

> Managed access gives people faster access to promising new treatments. Otherwise, they might not be recommended because of uncertainties about their clinical- or cost-effectiveness.
>
> *-NICE (UK)*

Genomic Screening and Testing
Recent advancements in genetic testing allows for precision healthcare and individual therapy plans which offer renewed hope and better health outcomes for patients and families. However, as technology continues to improve, the number of targeted therapies continue to grow, and sequencing costs continue to decline, how will genome sequencing become the standard in diagnosis and care in Canada without the infrastructure to support it?

Acronyms

ADA - The gene makes an enzyme called adenosine deaminase
aHUS - atypical hemolytic uremic syndrome
ATG - Anti-Thymocyte Globulin
CADTH - Canadian Agency for Drugs and Technologies in Health
CIDP - Chronic inflammatory demyelinating polyneuropathy
CDA-AMC - Canada's Drug Agency (formerly CADTH)
CBS - Canadian Blood Services
DRD - drugs for rare diseases
GBS - Guillain-Barré syndrome
HAE - Hereditary Angioedema
HHT - hereditary hemorrhagic telangiectasia
HRQoL - health-related quality of life
HTA - health technology assessment
IAP - Implementation Advisory Group
IEI - Inborn Errors of Immunity
IG - immunoglobulin/immune globulin
ITP - immune thrombocytopenia
MPN - myeloproliferative neoplasms
MG - Myasthenia gravis
IVIG - intravenous immunoglobulin
NOC/c - Notice of Compliance with conditions
NORD - National Organization for Rare Dsiorders
NRBDO - Network of Rare Blood Disorder Organizations
PI - primary immunodeficiency disease
PNH - Paroxysmal nocturnal hemoglobinuria
P/T - provincial/territorial
SCID - Severe Combined Immune Deficiency
SCIG - subcutaneous immunoglobulin
TLR - time-limited reimbursement
TTP - Thrombotic thrombocytopenic purpura
VWD - von Willebrand disease

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