# Unique approach needed:
## *Addressing barriers to accessing rare disease treatments*

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## 1. Introduction

On behalf of a group of innovators of rare disease treatments in Canada, we thank the committee for undertaking a study addressing the barriers to accessing medicines for rare disorders.

We represent biopharmaceutical companies that are committed to improving the lives of patients around the world living with rare disorders by researching, developing and commercializing rare disease treatments. Our group includes Alexion Pharma Corp., Biogen Canada Inc., BioMarin Pharmaceutical (Canada) Inc., Horizon Therapeutics Canada, Ipsen Biopharmaceuticals Canada Inc., Shire Pharma Canada ULC, Sobi Canada, Inc., and Vertex Pharmaceuticals (Canada) Inc.

As you can already appreciate from the testimony you have heard to date in the context of this study, Canadians experience numerous challenges in trying to obtain access to treatments for rare conditions. Some important recent proposed changes to the Canadian access landscape, coupled with controversial proposed revisions to the *Patented Medicines Regulations*, suggest that access to medicines for rare disease patients will become even more threatened in future.

Of the approximately 7,000 rare diseases, there are only effective treatments for 5% of these conditions. Even with the incentives offered by the US *Orphan Drug Act*, fewer than 15 new treatments for rare diseases are approved in that country per year.[1] And fewer of those are approved in Canada. Even when an effective treatment has been developed, patients must still endure lengthy waits and great uncertainty as to whether they will be able to access these treatments in this country.

[1] Pharmaceutical Research and Manufacturers of America (PhRMA), *Rare Diseases: A Report on Orphan Drugs in the Pipeline*, 2013 Report: http://www.phrma.org/sites/default/files/pdf/Rare_Diseases_2013.pdf.

Many of these conditions are severely debilitating or life-threatening and the affected patients and families face many challenges living with these conditions. We believe that a compassionate health care system should be organized in such a way as to ensure timely and reasonable access to needed therapies. To clarify, even if Canada finds a way to do a better job of extending public coverage for rare disease treatments, these treatments are expected to remain a very small percentage of public drug plan and broader health care budgets. In 2013, rare disease treatments accounted for about 5% of the total national expenditure on pharmaceuticals.[2]

For these reasons, we are very encouraged by your Committee's interest in studying and addressing these issues, and encourage the Committee to develop recommendations that would call for tailored approaches throughout the Canadian regulatory and reimbursement pathway to establish equitable access to rare disease treatments for all Canadians.

We were also very pleased that the provincial-territorial (PT) Expensive Drugs for Rare Diseases (EDRD) Working Group recently issued a proposed new approach for facilitating better consideration of complex and specialized medicines – including rare disease treatments – for potential public reimbursement purposes. However, while we strongly support the initiative and look forward to working with the PT drug plan managers to develop a robust alternative method for considering new rare disease treatments for public funding, we vociferously object to the name of the working group as it immediately prejudices patients with rare diseases.

We believe that the unique characteristics of rare diseases require a different approach and increased incentives to improve the process for continuing to bring these treatments to patients in Canada. In particular, we would like to draw your attention to the following actions we believe the federal government can take to help address the barriers in accessing rare disease treatments:

1. In cases where no approved and marketed alternative exists in Canada for rare disease patients, the federal government's Special Access Programme should remain available as a means of facilitating patients' options for accessing rare disease treatments.
2. The federal government should implement a regulatory framework for rare disease treatments.
3. The federal government should reconsider its proposed pharmaceutical price review reforms.
4. The federal government should encourage the Canadian Agency for Drugs and Technologies in Health to adopt processes, criteria and standards that are more appropriately suited for assessing rare disease treatments.
5. The federal government should collaborate with the provincial/territorial EDRD Working Group to improve the reimbursement pathway for rare disease treatments, including the implementation of measures to help facilitate and provide timely and equitable patient access to rare disease treatments.

This submission outlines these recommendations in greater detail and provides an overview of the unique challenges involved in developing and commercializing rare disease therapies.

[2] Divino V et al, *The Budget Impact of Drugs Treating Rare Diseases in Canada: a 2007-2013 MIDAS Sales Data Analysis*, Orphanet J Rare Dis,. 2016: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4875716/.

## 2. Challenges in developing and commercializing rare disease treatments

Developing and commercializing rare disease treatments is more challenging than for more common diseases for many reasons, including:

- Knowledge of rare diseases is limited. At the beginning of research and development into potential treatments, there is little to no previous scientific or technical knowledge on which to rely given the rarity of the disease. This means that research into rare disease treatments often begins at square one. Further, long-term disease progression data is also difficult to locate or develop. There can also be significant genetic diversity within a single rare disease, further challenging scientists' understanding of the disease itself, which is needed before beginning to develop a potentially successful treatment.
- Rare diseases affect a small number of people. The fact that rare diseases affect few patients presents specific challenges from a research and development perspective. In particular, the low prevalence of rare diseases makes it difficult to locate and recruit patients for clinical trials. Consequently, many clinical trials are global in scope by necessity, and conducted via numerous clinical trial sites.
- Few clinicians are familiar with rare diseases. While also affecting patient care, clinicians' lack of familiarity with rare diseases makes it challenging to recruit knowledgeable physicians to administer clinical trials. This makes it unlikely that the necessary expertise would be available in a given country. If such expertise exists in Canada at all, it is likely that it would be limited to a very small number of clinicians here.
- Gold standard clinical trials are often infeasible or unethical. Evaluations of new treatments, such as health technology assessments (HTAs) conducted by the Canadian Agency for Drugs and Technologies in Health (CADTH), are designed with the expectation that a clinical trial will be a randomized, controlled trial (RCT). However, this type of trial design is often infeasible given the low prevalence or unethical given the disease severity and lack of other treatment options. When innovators cannot implement RCTs, they rely on other suitable methodologies in order to understand whether a potential treatment is effective in treating patients. An example of an alternative methodology is an adaptive clinical trial where participants are monitored to test the effect of the potential treatment on a defined schedule and specific parameters of the study change based on the observations.
- Greater clinical and economic uncertainty. Limited understanding of rare diseases and the small patient populations create greater uncertainty. More specifically, the nascent scientific understanding of many rare diseases results in limited understanding or consensus about clinically validated endpoints for studies of potential treatments. Smaller and shorter studies designed to expedite access to treatments in the face of very severe diseases result in less clinical trial data. In fact, sometimes only unpublished proprietary data is available at the time of submission. As well, the absence of clinician knowledge and lack of expert reference centres on which to rely means that the development of rare disease treatments fails more often than common disease treatments. Alternative clinical trial design methodologies that enable the study of rare disease treatments create greater uncertainty for regulators, HTA evaluators and those involved in the reimbursement process. While regulators have made great strides in understanding this type of data, innovators routinely face challenges when presenting study results in the context of HTAs due to the rigid evaluation framework.

Because of these challenges, the risk and cost involved in developing and commercializing treatments for rare diseases is often much greater compared to more common conditions.

## 3. Recommendations to improve patient access to rare disease therapies

### *a. Special Access Programme*

Contrary to most other developed countries, Canada does not have a distinct regulatory framework for rare disease treatments. Consequently, many of these treatments come to Canada later or do not come to Canada at all. Specifically, submissions to Health Canada for 84% of rare disease treatments approved between 2002 and 2016 were filed after these submissions were filed with American and European regulators. Further, 23 treatments for rare diseases approved by the European and/or the American regulator in that same time period were not approved in Canada by the end of 2016.[3]

At the present time, patients seeking access to treatments that are not approved by Health Canada must rely on their clinicians to help them navigate Health Canada's Special Access Programme (SAP). Although initially designed as an exceptional mechanism, the SAP has become the *de facto* treatment access pathway for many rare disease patients due to the fact that the treatments they need are not available commercially through the standard access pathway. Of the almost 500 medicines approved by the SAP in 2016, 29% were for treatments classified as orphan medications in the jurisdictions where they were already approved. In addition, 50% of the SAP medications were first approved by the programme 10 years previously.[4]

The SAP is not an ideal mechanism for facilitating access to rare disease treatments. It is a time-consuming process that requires patients to rely on their clinician to seek customized approval from Health Canada by providing scientific literature demonstrating that a given treatment that is available elsewhere in the world is the right solution to meet that patient's particular needs. Even when this process is followed successfully, often the initial authorization expires within six months later (or less) and patients must work with their clinicians to undergo the process all over again. Worse still, Health Canada's approval does not come with reimbursement, leaving patients to figure out how to pay for their treatment.

Due to limitations in the Canadian pharmaceutical review and approval process, some manufacturers may rely on the SAP to facilitate access while circumventing the need to pursue a formal Health Canada submission. The SAP must not be used by manufacturers seeking to achieve Canadian market authorization through a reduced regulatory standard. This scenario could put potential competitive products, which are introduced via the formal process, at a disadvantage. So, while it is important to ensure that rare disease patients are not cut-off from accessing products on the SAP when there are no other therapeutic options and little chance that a given manufacturer will submit to Health Canada, we also must be careful not to discourage manufacturers from seeking market authorization through the standard process.

Clearly, gaps in the Canadian medication review and approval process have to be addressed so that patients are not compelled to rely on the cumbersome SAP process to access their medicines. In the

[3] Rawson, N., "Regulatory, Reimbursement, and Pricing barriers to accessing Drugs for Rare Disorders in Canada", Fraser Institute, 2018: https://www.fraserinstitute.org/sites/default/files/barriers-to-accessing-drugs-for-rare-disorders-in-canada.pdf.

[4] Health Canada, "Renewal of the Special Access Programme For Drugs" presentation, Health Canada and BIOTECanada Joint Meeting, September 2017.

meantime, however, while we work on addressing pharmacare gaps, the reform of the SAP undertaken by Health Canada should not reduce access for patients who still need to rely on this program to get their treatments. The SAP often remains the only way for rare disease patients to obtain access to their treatments.

**RECOMMENDATION 1: In cases where no approved and marketed alternative exists in Canada for rare disease patients, the federal government's Special Access Programme should remain available as a means of facilitating patients' options for accessing rare disease treatments.**

While reforms are important to reduce routine reliance on the Special Access Programme, this pathway must remain available for Canadians with rare disorders (where no approved and marketed alternative is available), who may rely on SAP as their only viable option to access the treatments they need to survive or get better.

### *b. Regulatory review of rare disease treatments*

As mentioned above, most developed countries have established distinct regulatory frameworks to help incentivize the development and commercialization of rare disease therapies. These frameworks typically include a definition of rare disease, an orphan drug designation process, strengthened intellectual property or additional data protection, and other financial incentives such as research promotion funds, tax incentives and regulatory submission fee reductions.

While Health Canada developed and consulted with stakeholders on a proposed orphan drug regulatory framework in 2012, no framework was ever implemented. It appears that the federal government no longer intends on moving forward with an orphan drug regulatory framework, as all references to the framework were recently removed from Health Canada's website.

Instead, Health Canada published an orphan drug regulatory approach, which explains innovators' options for seeking regulatory approval and bringing their treatments to patients in Canada. While this is a positive step forward, it does not replace the need for a regulatory framework for rare disease treatments that includes the key elements adopted by other jurisdictions, including a definition of rare disease, an orphan drug designation process, additional market exclusivity, research promotion funds, tax incentives and Health Canada submission fee reductions. This would help encourage the development and launch of rare disease treatments in Canada. To clarify, we are not supportive of any reforms that would compromise Health Canada's current high review standards, rather we are seeking modifications that would support and incentivize the development and introduction of effective rare disease treatments in Canada.

**RECOMMENDATION 2: The federal government should implement a regulatory framework for rare disease treatments.**

The federal government should implement a regulatory framework for rare disease treatments that includes incentives to develop and commercialize rare disease therapies in Canada, including a definition of rare disease, an orphan drug designation process, additional market exclusivity, research promotion funds, tax incentives and regulatory submission fee reductions.

### *c. Pricing of rare disease treatments*

Developing treatments for rare disorders is a risky and costly enterprise for the reasons previously mentioned in this submission. As a result, rare disease treatments tend to be priced higher than medicines for common diseases. In particular, the price difference can be explained in large part by the fact that research and development investments for rare disease treatments have to be recouped from a smaller market worldwide.

That said, while rare disease treatments generally result in higher per-patient costs, their overall budget impact is comparatively low given their small patient populations. In particular, based on a 2016 analysis, expenditures for rare disease medicines represented 3.3 to 5.6 % of total Canadian pharmaceutical expenditure in 2007–2013, and were expected to remain less than 6 % of total expenditure for the period 2014–18. [5]

Despite this, the federal government has proposed to amend the *Patented Medicines Regulations* to change how the federal Patented Medicine Prices Review Board (PMPRB) assesses the prices of patented medicines. This proposed reform will have a disproportionate effect on rare disease treatments. As discussed further in this submission, it is already very difficult under the current system to commercialize rare disease treatments in Canada. The proposed reform will introduce an additional barrier by establishing a restrictive and uncertain new price assessment process, which will result in price reductions of 70-90% for rare disease medicines.[6] A recently published study clearly shows the negative implications of the reform on rare disease treatments.[7]

This will make it very challenging for innovative companies to bring rare disease medicines to the Canadian market, which means the reforms would end up further slowing down or limiting patient access to new rare disease treatments. As proposed, the reforms could also jeopardize access to these treatments through the SAP, as medicines provided through this program also have to meet the PMPRB's pricing thresholds.

In particular, if the currently proposed reform moves forward, the PMPRB will use cost-effectiveness studies produced by CADTH to set pricing thresholds. It is not appropriate for a quasi-judicial regulatory body, such as the PMPRB, with no connection to reimbursement, to be applying cost-effectiveness studies to establish pharmaceutical prices. This is highly problematic, especially for rare disease treatments, as the current cost-effectiveness methods used in these studies are not adapted to evaluating rare disease treatments (more details are provided on this issue in the next section of the submission). The health economic studies, which are designed to inform payers for reimbursement decisions, are not suited to regulating prices because they are based on a set of assumptions and results that can vary widely.[8] Finally, the use of cost-effectiveness studies as part of

[5] Divino V et al, *The Budget Impact of Drugs Treating Rare Diseases in Canada: a 2007-2013 MIDAS Sales Data Analysis*, Orphanet J Rare Dis., 2016: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4875716/.

[6] Rawson, N., *Regulatory, Reimbursement, and Pricing barriers to accessing Drugs for Rare Disorders in Canada*", Fraser Institute, 2018: https://www.fraserinstitute.org/sites/default/files/barriers-to-accessing-drugs-for-rare-disorders-in-canada.pdf.

[7] See also Rawson N., *New Patented Medicine Regulations in Canada: Case Study of a Manufacturer's Decision-Making about Regulatory Submission for a Rare Disorder Treatment*, Canadian Health Policy Institute, October 2018: https://www.canadianhealthpolicy.com/products/new-patented-medicine-regulations-in-canada--case-study-of-a-manufacturer---s-decision-making.html.

[8] Rawson N., *New Patented Medicine Regulations in Canada: Case Study of a Manufacturer's Decision-Making about Regulatory Submission for a Rare Disorder Treatment*, Canadian Health Policy Institute, October 2018:

the PMPRB's price assessment framework will lead to a highly uncertain, complex and multi-layered price control system for patented medicines in Canada. No other country has implemented a health economic approach to regulating prices of medications.

**RECOMMENDATION 3: The federal government should reconsider its proposed pharmaceutical price review reforms.**

The high level of uncertainty and the substantial price reductions resulting from the proposed federal reforms would create a significant additional barrier to timely patient access to rare disease treatments. We encourage the federal government to work instead on speeding up access to breakthrough medicines for rare disorder patients.

### *d. Health technology assessment of rare disease treatments*

Following Health Canada's regulatory review, CADTH – an agency funded by the federal and provincial governments (except Québec) – evaluates the comparative clinical and cost-effectiveness of a medicine (also called health technology assessment or HTA) to help governments decide whether and how to reimburse the medicine. However, as previously mentioned, the evaluation methods currently used by CADTH are not well suited for assessing rare disease therapies.

The current HTA review process fails to account for the unique characteristics of rare disease treatments, including the small size of clinical trials and the fact that RCTs are often not possible for life-threatening conditions. In addition, CADTH reviews are not flexible enough to accommodate informed therapeutic and/or current patient management knowledge in a specific therapeutic area or for a given condition.

Further, many experts have recognized the limitations of current cost-effectiveness methods to evaluate rare disease treatments.[9] These methods were designed to be used in broad population-based assessments and they do not work well when considering treatments developed to meet the needs of small populations and that have higher per-patient price. Also, these methods are intended to compare the value of a new medicine with the value of an older medicine, which is often impossible to do with rare disease breakthrough treatments, as many do not have appropriate comparators.

Since CADTH's current methods are ill-fitted for rare disease treatments, these medicines often fail to meet the cost-effectiveness thresholds set by CADTH. Further, CADTH's recommendations for these treatments often include the suggestion that substantive price reductions (e.g., up to 97%) are required to meet the cost-effectiveness thresholds.[10] Despite therapeutic value being recognized by

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https://www.canadianhealthpolicy.com/products/new-patented-medicine-regulations-in-canada--case-study-of-a-manufacturer---s-decision-making.html. See also Law M. and Critchley W., *Ottawa's plan to change drug price regulations is not good policy*, Policy Options, Institute of Research on Public Policy, October 2018: http://policyoptions.irpp.org/magazines/october-2018/ottawas-plan-to-change-drug-price-regulations-is-not-good-policy/.

[9] Drummond M. et al., *Orphan drugs policies: a suitable case for treatment*, Eur J Health Econ, May 2014, 15(4): 335-340: http://www.ncbi.nlm.nih.gov/pubmed/24435513; and Hughes-Wilson W. et al., *Paying for the Orphan Drug System: break of bend? Is it time for a new evaluation system for payers in Europe to take account of new rare disease treatments?*, Orphanet Journal of Rare Diseases, 7:74, 2012: http://www.ncbi.nlm.nih.gov/pubmed/23013790.

[10] Rawson, N., *Regulatory, Reimbursement, and Pricing barriers to accessing Drugs for Rare Disorders in Canada*, Fraser Institute, 2018: https://www.fraserinstitute.org/sites/default/files/barriers-to-accessing-drugs-for-rare-disorders-in-canada.pdf.

CADTH, the associated recommendations make it very hard for rare disease treatments to be successfully negotiated and reimbursed by public drug plans.

In fact, in a recent article, Trevor Richter, the current director of CADTH's Common Drug Review – the program that assesses non-oncology medicines – acknowledged the higher rate of negative HTA recommendations for ultra-rare disorders and suggested that it may be warranted to apply different standards when evaluating these therapies.[11]

While flexibilities for assessing rare disease treatments were introduced in CADTH's recommendation framework in 2016, these changes do not go far enough to help account for the particularities of rare disease treatments and facilitate their reimbursement. Building on these initial measures, CADTH should go a step further and adopt processes, criteria and standards that are better adapted to the characteristics of rare disease treatments. The development of the pan-Canadian Oncology Drug Review (pCODR) process, for example, is a best practice in this regard, in large part because it was and continues to be a multi-stakeholder and patient-centred initiative that involves developers, clinicians and patients throughout the process. pCODR's expert review body is mandated to consider alignment with patient values and health system adoption criteria. INESSS is another example where broader criteria is applied in its deliberative process, which requires its review committee to consider societal values.

**RECOMMENDATION 4: The federal government should encourage the Canadian Agency for Drugs and Technologies in Health to adopt processes, criteria and standards that are more appropriately suited for assessing rare disease treatments.**

The Canadian Agency for Drugs and Technologies in Health should adopt specific processes, criteria and standards for rare disease treatments that help address the unique characteristics of these therapies, including specialized clinician expertise, patient values, smaller study sizes, more frequent reliance on trials other than randomized, controlled trials and the higher cost of treatment per patient.

### *e. Reimbursement of rare disease treatments*

Following CADTH's HTA evaluation, the next step in the public reimbursement process is negotiations between the manufacturer and the pan-Canadian Pharmaceutical Alliance (pCPA), which represents the federal and provincial public drug plans. Before a medicine can be reimbursed by public drug plans, the reimbursement terms of a new medicine have to be negotiated through the pCPA process.

There are many challenges with the pCPA process that negatively affect patient access to all new medicines. The process can take anywhere between a few months to several years to complete (the average timeline in 2017-18 has been approximately a year following the finalization of a CADTH recommendation[12]). Further, even once an agreement has been reached with the pCPA, there is no

[11] Richter T. et al., *Characteristics of drugs for ultra-rare diseases versus drugs for other rare diseases in HTA submissions made to the CADTH CDR*, Orphanet J Rare Dis. 2018: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5793441/.

[12] Morse Consulting, *pCPA Reducing Time to Initiate Negotiations*, October 10, 2018: http://morseconsulting.ca/pcpa-reducing-time-to-initiate-negotiations/.

guarantee that public plans will add the medicine to their formularies or that they will do so in timely manner. This is because there is no requirement for public plans to reimburse the medicine and no set timeline for reimbursement following the successful conclusion of a pCPA agreement.

As a result, Canada ranks 15th out of 20 OECD countries when it comes to wait times between regulatory approval of a medicine and its reimbursement by public plans.[13] As well, there is a significant difference in reimbursement timelines between public and private drug plans in Canada. According to a recent study, private plans take on average 142 days to cover new drugs following Health Canada's approval compared with 449 days for public drug plans.[14]

We know that challenges relating to public reimbursement are even more complex when it comes to medicines for rare diseases given the greater uncertainty relating to the clinical data and the higher per-patient cost. This means that vulnerable patients, such as those with a rare condition, may not be able to access the treatments they need or may have to wait for a much longer period of time to access life-saving treatments.

For instance, a recent study indicated that a successful pCPA agreement could only be reached in slightly more than half of rare disease treatments that had received a positive CADTH recommendation.[15] This shows how challenging negotiations at the pCPA can be despite having received a positive reimbursement recommendation from CADTH. This has devastating effects on patient access, as public plans will not reimburse a medicine that has not been successfully negotiated with the pCPA. Further, it should also be noted that even when negotiations result in the conclusion of an agreement, the reimbursement criteria is often much more restrictive than the criteria approved by Health Canada, leaving many patients who do not meet the more limited criteria without access to needed medicines.

Many countries have adopted specific pathways or measures to help facilitate more timely reimbursement of medicines, especially for breakthrough and/or rare disease medicines. For instance, in Germany, medicines are reimbursed as soon as they receive regulatory approval while negotiation on reimbursement terms unfold between the government and pharmaceutical companies. France has implemented a system that allows for patient access to rare disease therapies even prior to regulatory approval.

We suggest that the pCPA be enhanced to provide more timely access to rare disease treatments. Specific measures to be implemented include:

- Reimbursement at regulatory approval: Measures allowing the reimbursement of rare disease therapies at the time they receive their regulatory approval should be implemented. We need to expedite access to these therapies, which are often for life-threatening conditions that have no other alternative treatment. At a minimum, the timeliness of the pCPA process should be

[13] Innovative Medicines Canada, *Access to New Medicines in Public Drug Plans: Canada and Comparable Countries*, 2016 Annual report: http://innovativemedicines.ca/wp-content/uploads/2016/05/20160524_Access_to_Medicines_Report_EN_Web.pdf.

[14] This for new drugs across the period of 2008-2017. See Canadian Health Policy Institute, *Coverage of new medicines in public versus private drug plans in Canada 2008-2017*, 2018: https://www.canadianhealthpolicy.com/products/coverage-of-new-medicines-in-public-versus-private-drug-plans-in-canada-2008-2017.html.

[15] Rawson N, *Regulatory, Reimbursement, and Pricing Barriers to Accessing Drugs for Rare Diseases in Canada*, Fraser Institute, August 28, 2018: https://www.fraserinstitute.org/sites/default/files/barriers-to-accessing-drugs-for-rare-disorders-in-canada.pdf.

improved by implementing specific reasonable timelines at every stage of the pCPA process, including between the conclusion of a pCPA agreement and the reimbursement of the medicine by individual public drug plans.

- Increased use of Managed Access Programs: The pCPA could better leverage the use of managed access programs. These programs provide patients with access to a medicine while evidence continues to be gathered. This can help address uncertainties resulting from clinical trial evidence and payers' related concerns of reimbursing treatments with more limited evidence.

As previously mentioned, we are very encouraged that the EDRD Working Group has moved forward on developing a new draft approach that should help improve access to rare disease therapies. Specifically, the proposal envisions concurrent Health Canada, CADTH, PMPRB and pCPA reviews, an enhanced health technology assessment review process, consideration of managed access agreements, enhanced use of real-world evidence and an adjudication process to address individual patient coverage requests by expert panels. If implemented with a patient-centric and multi-stakeholder approach, this new process would go a long way in addressing the many challenges outlined above. As such, we recommend that the federal government support the EDRD Working Group's efforts and actively collaborate on this initiative.

**RECOMMENDATION 5: The federal government should work with the EDRD Working Group to improve the reimbursement pathway for rare disease treatments, including the implementation of measures to help facilitate and provide timely and equitable patient access to rare disease treatments.**

The reimbursement pathway needs to be enhanced to provide better and timelier patient access to rare disease therapies. This includes implementing measures allowing reimbursement at the time of regulatory approval and increased use of managed access programs and real world evidence that can help address uncertainties related to rare disease medicines while facilitating timely patient access.

## 4. Conclusion

While this is an extremely exciting time for the rare disease community given the incredible scientific and technological advances in recent years, there is still a lot of work to be done to ensure the benefits of these new technologies reach the Canadian patients who need them. This underscores the importance of the Committee's study and indicates that it is a vital step towards improving the system for the most vulnerable patients in Canada.

By implementing the recommendations outlined in this submission, the federal government can lead the way in helping overcome the challenges in accessing rare disease therapies. In fact, the current discussions on national pharmacare provide a unique opportunity for the federal government to work with the provinces to address these challenges and close outstanding access gaps.

We thank you again for the opportunity to provide our input and look forward to reviewing your report and recommendations on this important health issue. Ultimately, we hope to work with the Government of Canada, the provinces and territories, patients and clinicians to help ensure that Canadians with rare disorders receive timely access to the health care that they need and deserve.