Submission: 2023 Federal Budget
Pre-Budget Consultations

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Summary of Recommendations

1) Implement the National Strategy for Drugs for Rare Diseases promised in Budget 2019.

2) Reduce regulatory roadblocks to drug innovation and access.

3) Quickly pass Bill C-22 and expeditiously establish the Canada Disability Benefit with eligibility criteria that are inclusive of chronic, intermittent, and progressive impairments.

4) Direct the Patented Medicine Prices Review Board (PMPRB) to conduct price reviews under the current regulatory and guideline framework until new guidelines are adopted which create more certainty in the market.

Introduction

Cystic fibrosis steals many things from people and families. Restful sleep is broken up by coughing fits. Time with friends and loved ones is second to daily physiotherapy and treatment routines. School and work are interrupted by frequent infections and subsequent hospital stays. Family dynamics may be strained by the stress and anxiety of managing a chronic illness. Dreams about the future are clouded by heavy realities of a fatal, rare disease.

But there is hope. There are highly effective medicines that treat the basic defect of cystic fibrosis (CF) rather than just the symptoms and that significantly improve the health outcomes and quality of life for many people with cystic fibrosis. These therapies offer a changed reality for people living with the disease.

However, more affordable and faster drug access and more inclusive disability supports are needed to ensure all Canadians with cystic fibrosis can access the therapies their doctors prescribe. To those ends, we call on Parliamentarians and the Government of Canada to implement the following recommendations.

Recommendation 1: Implement the National Strategy for Drugs for Rare Diseases promised in Budget 2019.

Cystic fibrosis is a rare disease. Canadians with rare diseases need a shared strategy to ensure they get the medicines they need in timely and affordable ways. Canada's patchwork of drug access programs is broken and leaves Canadians with rare diseases, including cystic fibrosis, behind. There is vast inconsistency in coverage across jurisdictions, unaffordable deductibles and co-pays, and, in some jurisdictions, long wait times for access.

Our country is an outlier when compared to international peers, which implemented rare disease strategies years ago. On January 4th, 2023 the U.S. Food and Drug Agency's *Orphan Drug Act* turned 40. This legislation "incentivized drug companies to put more resources toward the research, development, and distribution of therapeutics for people with rare diseases, who until then had been 'orphaned' by the medical and scientific community".[i] It has been instrumental in driving both innovation and access. Meanwhile, Canadians with rare diseases remain orphaned. This must change now.

People who live with cystic fibrosis – a fatal, progressive disease – and the broader CF community had to aggressively advocate every step of the way to access new important therapies, including life-changing treatment Trikafta.

Trikafta can dramatically improve a patient's life. For example, Trikafta can help approximately 90% of the CF population in Canada, reducing exacerbations and hospitalizations. A 2020 Canadian study projected that early introduction of Trikafta would result in life-changing health improvements by 2030 and that the estimated median age of survival for a child born with CF would increase by 9.2 years.[ii]

Despite having great efficacy, it took over two years of momentous advocacy efforts at the federal, provincial, and territorial levels to get public access to Trikafta for those 12 years of age and older, followed by access for those 6-11 years olds. While far too long to wait when you have a fatal disease, this is considered fast when compared to other drugs for rare diseases.

Many who tried to access the drug encountered access issues with public and private drug plans, including processing and time-to-list delays, high insurance deductibles and/or premiums, and complex coordination of benefits. Some still cannot access the drug through public or private drug plans. Canadians with rare diseases should not have to pay for drugs that save their lives or offer them a future they never thought they would have.

An international comparative study[iii] of public reimbursement of orphan drugs in Canadian provinces compared to European countries showed that:

> *Sixty-three approved therapies with an orphan drug designation from the European Medicines Agency (EMA) were identified. Fifty-three (84%) of these drugs had also been submitted to the Food and Drug Agency (FDA) for approval, and 41 (65%) were submitted to Health Canada for approval. In Europe, Germany, Denmark, and the U.K. had the highest percentage of publicly reimbursed orphan drugs (84%, 70%, 68%, respectively).*
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> *In comparison, Ontario (32%), Quebec (25%), and Alberta (25%) had the highest percentage of drugs reimbursed among the Canadian provinces. The shortest median duration (in months) from EMA approval to jurisdictional decision on reimbursement was in Austria (3.2), followed by Germany (4.1), and Finland (6.0). In Canada, the shortest median duration (in months) from regulatory approval to reimbursement was in British Columbia (17.3), Quebec (19.6) and Manitoba (19.6), while the longest duration was in P.E.I (38.5), followed by Nova Scotia (25.9), and Newfoundland (25.1).*

Canada must do better. The Government of Canada must implement the *National Strategy for Drugs for Rare Diseases* promised in 2019 federal budget to:

> *"… help Canadians with rare diseases access the drugs they need, Budget 2019 proposes to invest up to $1 billion over two years, starting in 2022–23, with up to $500 million per year ongoing."*

In February 2019 the Standing Committee on Health (HESA) released a report on the barriers that Canadians with rare diseases face in accessing treatments and how the federal, provincial and territorial governments could work together to remove these barriers. This report recommended that:

- The Government of Canada, in collaboration with the provinces and territories, develop a coordinated process for the market authorization and reimbursement of drugs for rare diseases.

- Health Canada and the Canadian Agency for Drugs and Technologies in Health (CADTH) undertake their respective scientific evidence review processes of drugs for rare diseases in tandem as a standard practice.

- The reimbursement of drugs for rare diseases be included as part of a national pharmacare program established by the Government of Canada, in collaboration with the provinces and territories, through amendments to the *Canada Health Act,* as recommended by the House of Commons Standing Committee on Health in its report entitled *Pharmacare Now: Prescription Medicine Coverage For All Canadians*.

It is now February 2023 and we are still without a framework, funding and a way forward. The federal government must honour its commitment to implement a drugs for rare diseases strategy now. Most importantly, this strategy must improve access to drugs for rare diseases. It must be collaborative and sustainable, with defined roles for both public and private payers, while streamlining drug review processes and reducing duplication, to expedite access to drugs for rare diseases. Finally, it should drive research and innovation and encourage pharmaceutical companies to bring their rare disease medicines to Canada, much like our comparator countries have done, including the United States' *Orphan Drug Act*.

Recommendation 2: Reduce regulatory roadblocks to drug innovation and access.

Canada's drug regulatory system is antiquated and not designed for assessing drugs for rare diseases. As part of its regulatory innovation agenda, Health Canada is consulting on proposed agile regulations and guidance for licensing drugs and medical devices.

The proposed amendments to the *Food and Drug Regulations* and the *Medical Devices Regulations* are meant to:

- Reduce regulatory issues and roadblocks to innovation.

- Make Canada's science-based regulatory system more agile and internationally aligned.

The proposed amendments and guidance documents aim to:

- Improve risk management practices, including continual assessment of risks, benefits and uncertainties.

- Facilitate earlier access in the Canadian market through rolling drug reviews, allowing manufacturers to provide specified information after a drug submission has been filed.

- Recruit representative clinical trial participants to ensure that participants are representative of the population who will be using the drug. This will ensure that the data from clinical trials is used to assess efficacy and safety in diverse populations.

- Continued and timely access to safe and effective public health emergency drugs.

This is not an exhaustive list. Some objectives may create new barriers or exacerbate existing barriers to access. For example, ensuring diversity of clinical trials is paramount to improving access to medicines for underserved populations, but these populations are often hard to reach, especially in rare disease communities. The Government of Canada must work with stakeholders to develop clinical trial capacity and to improve inclusion of rare, diverse populations.

On the other hand, some objectives are needed to support implementation of a drugs for rare disease strategy. Of note is that new risks or uncertainties about a drug's safety, effectiveness or quality for the authorized indication(s) may be identified through:

- Post-market assessments of real-world evidence or
- New evidence from studies or reports

Use of real-world evidence to measure a drug's impact is crucial to driving access for small, rare disease populations in which large-scale clinical trials are not possible. In its agile regulations consultation documents, Health Canada acknowledges that:

> *The market has evolved to include more complex and personalized therapies. Our regulatory system also needs to evolve, to ensure Canadians have access to the drugs they need.*

The draft regulations will also allow for "*reviews of certain drugs that address unmet medical needs to rely on the authorization of a trusted foreign regulator*" and will align with international regulators. Both of these measures may help expedite access to drugs for rare diseases.

However, the use of laboratory evidence to inform decision-making at the regulatory level has been excluded. This leaves Canadians behind at a time when other countries are using ground-breaking technologies to provide access to CF modulators to those with rarer and ultra-rare mutations where no clinical trials have taken place. The UK's National Health Services (NHS), the Federal Drug Agency (FDA) in the US, and France's L'Agence nationale de sécurité du médicament et des produits de santé (L'ANSM) have all used laboratory evidence to expand access for people with rare mutations that can be treated with Trikafta.

A partnership of Cystic Fibrosis Canada and The Hospital for Sick Children, the Program for Individualized Cystic Fibrosis Therapy (CFIT) is well positioned to improve access for people with rare mutations now. To allow for the broadest access to precision medicines possible, Health Canada must create a path in its agile regulations and relevant guidance documents for high quality laboratory evidence to inform decision-making.

The Israeli Ministry of Health used CFIT data and organoid data to grant off-label short-term access to Trikafta for those with a rare mutation for which clinical trials did not take place. That Canadian laboratory data has been used to inform decision-making by a foreign ministry but is not accepted in Canada shows how absurd our current evidence practices are.

Recommendation 3: Direct the Patented Medicine Prices Review Board (PMPRB) to conduct price reviews under the current regulatory and guideline framework.

In 2019 the Patented Medicine Prices Review Board (PMPRB) proposed drastic changes to the way it determines excessive prices of medicines. Many stakeholders objected and expressed serious concerns about some changes. After multiple court rulings, some of the proposed changes were ruled unconstitutional. As a result, the scope of the proposed changes was significantly reduced.

In June 2022, the Parliamentary Budget Officer (PBO) published a report that underscored what patient communities have been saying for years:

> *…lower prices for new innovative drugs may reduce timely access to those drugs for Canadians…Canada must inevitably balance the interest of consumers who ultimately pay for pharmaceuticals with obligations to help fund R&D and incentivize the development of future products*.[iv]

In August 2022, the PMPRB implemented interim guidance that included not conducting a price review of new patented medicines until new guidelines are implemented. New guidelines were to be implemented on January 1, 2023, but they were delayed once again, until further notice.

All of this creates uncertainty of the regulatory and reimbursement environment in Canada, which impacts access to innovative drugs. Since manufacturers are less likely to launch innovative products in markets of uncertainty, the PMRPB should conduct price reviews under the current regulatory and guideline framework until new guidelines are adopted. We know firsthand that uncertainty costs Canadians their health and, in some instances, their lives.

The PMPRB held many consultations with stakeholders, including patient communities, yet we have seen little of the feedback given reflected in the newer iterations of the guidelines. The PMPRB must listen to stakeholders and work with us to find a new way forward, and this must happen quickly.

Recommendation 4: Expeditiously establish the Canada Disability Benefit with eligibility criteria that is inclusive of chronic, intermittent, and progressive impairments.

Nearly one in five Canadians with a disability live in poverty, with more at risk of falling below the poverty line.[v] Canada's myriad of disability support programs does not provide adequate nor equitable levels of financial support. To help address this issue, Bill C-22, *Canada Disability Benefit Act,* Canada Disability Benefit (CDB) for eligible working-age Canadians who are living with a disability.

We support implementation of the CDB, but it alone won't lift Canadians with disabilities out of poverty. The extent to which the CDB will – or won't – help Canadians with disabilities depends on where they live, whether they also receive provincial and territorial public disability supports, and how these supports all work together.

A recent report showed how dire the current disability support landscape is in Canada:

> *There are no unattached single with a disability households that have welfare incomes that reach the Official Poverty Line in any province. Furthermore, the households in eight of the ten provinces are below the deep income poverty threshold.* ***The income gap to reach the Official Poverty Line ranges from $4,700 to $14,700, depending on the province****.*[vi]

The CDB is designed to address equity issues across jurisdictions, but the amount of support it will provide falls short of what Canadians with disabilities need to live above the poverty line, nor is it reflective of the additional costs associated with living with a disability. Our governments must work together to address these challenges.

Overall, the Canada Disability Benefit is a positive move forward that will impact many lives. To provide the broadest access to the CDB, the Government of Canada must ensure that it adopts an inclusive definition of disability, such as the one legislated in the *Accessible Canada Act*:

> *…any impairment, including a physical, mental, intellectual, cognitive, learning, communication or sensory impairment—or a functional limitation—whether permanent, temporary or episodic in nature, or evident or not, that, in interaction with a barrier, hinders a person's full and equal participation in society.*

This definition is more inclusive than that of the Disability Tax Credit (DTC), a "gatekeeper" of access to most federal, provincial and territorial disability support programs, where they exist. The DTC definition fails to recognize the harm from the episodic severe disability of cystic fibrosis and other conditions that may affect people in intermittent ways. Universally applying the Accessible Canada Act definition will help to align all of our disability support programs and better serve Canadians with disabilities.

About Cystic Fibrosis and Cystic Fibrosis Canada

Cystic fibrosis is the most common fatal genetic disease affecting 4,332 Canadian children and young adults. There is no cure. Of the Canadians with cystic fibrosis who died in the past five years, half were under the age of 37. Cystic fibrosis is a progressive, degenerative multi-system disease that affects mainly the lungs and digestive system. In addition to the physical effects of the disease anxiety and depression are rampant in this population. Double lung transplants are the final option for patients with end-stage disease; most fatalities of people with CF are due to lung disease.

Cystic Fibrosis Canada has dramatically changed the cystic fibrosis story. We have advanced research and care that has more than doubled life expectancy. Since being founded by parents in 1960, Cystic Fibrosis Canada has grown into a leading organization with a central role engaging people living with cystic fibrosis, parents and caregivers, volunteers, researchers and healthcare professionals, government and donors. We work together to change lives for the 4,332 Canadian children and adults living with cystic fibrosis through treatments, research, information and support. Despite our remarkable progress together, we are not yet done. We will keep pushing, keep going further until all people with cystic fibrosis can and do experience everything life has to offer – and enjoy everything life has to offer. Learn more at www.cysticfibrosis.ca.

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[i] *The Orphan Drug Act Turns 40: NORD Celebrates Its Impact on Rare Diseases*. National Organization for Rare Diseases (NORD). January 4, 2023.

[ii] *Projecting the impact of delayed access to elexacaftor/tezacaftor/ivacaftor for people with Cystic Fibrosis.* Journal of Cystic Fibrosis. August 24, 2020. Stanojevic S, Vukovojac K, Sykes J, Ratjen F, Tullis E, Stephenson *et al*.

[iii] *An international comparative analysis of public reimbursement of orphan drugs in Canadian provinces compared to European countries.* Orphanet J Rare Dis 17. March 4, 2022. Ward, L.M., Chambers, A., Mechichi, E. et al.

[iv] *Canadian patented drug prices: gauging the change in reference countries*. Office of the Parliamentary Budget Officer. June 14, 2022.

[v] Canada. Statistics Canada. "The Daily — Canadian Survey on Disability, 2017," November 28, 2018.

[vi] *Advancing the Canada disability benefit quickly to meaningfully support working age adults with disabilities.* Maytree Foundation. November 15, 2022.