Health Canada

Building a National Strategy for High-Cost Drugs for Rare Diseases

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Submitted By:

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PULMONARY HYPERTENSION ASSOCIATION OF CANADA

L'ASSOCIATION D'HYPERTENSION PULMONAIRE DU CANADA

A better life for all Canadians affected by pulmonary hypertension

Introduction

Pulmonary hypertension (PH) is a rare but very complex and serious lung disease that is progressive and potentially fatal. PH is defined by high blood pressure in the lungs resulting from inflammation or scarring in the pulmonary arteries. If left untreated, PH can lead to enlargement and weakness of the right-side of the heart, a serious type of heart failure. PH can strike people of all backgrounds, ages, and sexes and can develop without any known cause.

Of the five identified types of PH, pulmonary arterial hypertension (PAH) is the rarest, affecting less than 10,000 Canadians. PAH shares a number of its symptoms with other conditions, resulting in many people being misdiagnosed. Without treatment, the average life expectancy of someone with PAH is less than three years. Alarmingly, many patients spend two to three years seeking an accurate diagnosis. Relative to many "rare diseases", we are fortunate that *because of access to available treatments*, patients are living longer, healthier lives. Yet, the closest thing to a cure remains double lung (and sometimes also heart) transplantation. And despite the 10 medical treatments currently approved for PAH in Canada, most patients remain seriously ill, markedly limited in everyday life, and potentially facing a death sentence within a few short years.

The problems we are being asked to solve in this consultation—consistency of drug access across the country, availability of evidence-based decision making, and sustainability of the Canadian health care system—seem to require much greater solutions than can reasonably be provided by a rare disease dug plan, let alone one fixated on "high-cost" drugs. What we need is a *rare disease strategy*. We don't only need mechanisms to manage the financial impacts of small patient populations and medical innovation, we also need a commitment (backed by resources) from government to take a holistic approach to rare diseases that includes early detection and prevention, timely and equitable access to evidence-based care, enhanced community support (including for community-based patient organizations), and the promotion of innovative research. How fortunate that such a strategy already exists (see: Canadian Organization for Rare Disorders).

The lack of a comprehensive rare disease strategy alone ensures that our European counterparts will remain years ahead of us. We do not need to reinvent the wheel or find the perfect Canadian solution. We have an opportunity to look at what has come before us in terms of best practices, apply some forward thinking, and design a made-in-Canada Rare Disease Strategy that includes a National Rare Disease Drug Plan. Since we do not see how we can do one without the other, our submission is intended to reflect the need for a Rare Disease Drug Plan that is informed, supported, and resourced by a Canadian Rare Disease Strategy.

Question #1 – Consistent Patient Access

A single national framework for decision making concerning rare disease drugs is necessary if we are to eliminate the existing barriers to not only equal access to new therapies, but also timely and affordable access. Such a framework must operate transparently, be centred on patient and clinician engagement, and help to coordinate rare disease research nationally. Which is to say, the options provided in the discussion paper are not mutually exclusive and should not be treated as such.

A national framework could include, but may not necessarily require, a separate health technology assessment (HTA) body. The recently proposed "supplemental process for rare disease drugs" aims to reduce approval times through its concurrent review with Health Canada, but it needs to be flexible enough to approve drugs based on less robust clinical evidence and higher degrees of uncertainty. Getting patients earlier access to potentially life-altering therapies will require the HTA body to support changes to clinical trial design, such as reducing the need for randomized, placebo-controlled clinical trials, increasing the use of surrogate endpoints in the decision making—including patient-reported outcomes, and reassessing approved indications, therapeutic value, and coverage at several points along the clinical development and regulatory review path.

Question #2 – Evidence-Based Decisions

By working collaboratively with international researchers and regulatory agencies to discuss and exchange scientific information, we will generate a larger body of evidence from which to base final approval decisions. Meanwhile, independent patient registries need to be developed such that they can be used to provide real world evidence—including patient reported outcomes—for after-market reassessments and review by regulatory agencies. This information can be used both at the time of initial upfront access and throughout any reassessment process in order to mitigate deficiencies in clinical evidence.

National expert panels (both pre- and post-access) need to include experts from the specific disease in question. This includes clinical experts, as well as patients with diverse lived experiences. We live in a time of extraordinary technological connectedness. There is no reason not to include more voices at the table, including those that can bring the data to life.

Like all who generate and use health information and data, patients must be properly resourced to be effective in their role. Therefore, any effective rare disease strategy would require investments in building the capacity of patients and their advocates, including the very patient organizations regulators depend on to produce the patient evidence they say is so crucial to their decision-making processes. A truly patient-centred approach would ensure that patients are able to lead in the creation and translation of their own health data.

Question #3 – Sustainable Health Care

In order to provide earlier access to drugs that meet the criteria for expedited review, more innovative approval and coverage models will also be necessary. We see how it is possible (such as in Germany and France) to make innovative therapies immediately available to patients upon approval, when the unmet need is high and the budget impact below a certain threshold. Managed Access Plans, Pay for Performance, and structured amortization payments are also possible ways to improve time to access and should be utilized depending on the disease, unmet need, and potential cost of treatment. There will never be a one-size-fits-all approach. We must be willing to be creative and flexible, assessing and utilizing whichever strategies are suitable in each particular circumstance.

A national drug plan for rare diseases is also required. The model of collective negotiation through the panCanadian Pharmaceutical Alliance (pCPA) will not work. While the pCPA has done a reasonable job of reducing the price of new drugs for both common and rare diseases, that success has not translated into earlier or equal access for rare disease

patients. Just as the models above suggest a sharing of costs/risks between manufacturers and payers, in Canada some form of cost sharing and pooling of risks between the provinces and territories also seems necessary.

Finally, up-front investments to reduce the risk in early development and supports for Canadian innovation would also be part of a complete rare disease strategy. We have world-class hospitals, clinicians, researchers, and universities in this country. By not investing in this impressive human capital and the potential breakthroughs they could generate, we risk losing them to countries and governments more willing to measure the value (reward) rather than the cost (risk) of the investment.

Conclusion

Drugs for rare diseases can and will continue to be expensive due to small patient size and the innovative nature of new therapies. That hasn't stopped other countries from being able to provide access to expensive therapies within weeks or months of drug approval, while we wait months or even years for initial drug approval and then more months and years for public funding (if we get it at all), even for drugs that are relatively inexpensive.

In May 2008, Don Bell, Member of Parliament for North Vancouver and father of PHA Canada's first chairperson, Darren Bell, introduced a private member's motion (M426) in the House of Commons in support of PH and other rare disorders calling on the government to "respond specifically to the challenges faced by Canadians with rare diseases and disorders". It's been nearly 13 years since that motion was introduced and rare disease patients are still waiting for a response from the federal government. They deserve a full response, not one limited to a cost-saving exercise. It's time that rare disease patients were valued as equal citizens and not simply seen as a burden on the system. It's time for a Canadian Rare Disease Strategy.