Delivering on Canada's Rare Disease Investment

Rare Disease Day Conference

March 28 – 29, 2023

Ottawa Marriott

100 Kent Street, Ottawa

*Virtual option is also available

Canadian Organization for Rare Disorders

How to Optimize $1 Billion Investment in Rare Drug Strategy

In 2019, the federal government committed $1 billion to invest in a rare disease drug strategy with on-going investment of $500 million annually. Following three years of nation-wide multi-stakeholder consultations, the Canadian Organization for Rare Disorders (CORD) on behalf of the rare disease community is proposing a 3-pillar Rare Disease Strategy to deliver optimal value to patients, the healthcare system, and society.

Recommendation 1: Invest in Infrastructure for early accurate diagnosis, access to specialists, and patient-centred management

Recommendation 2: Invest in alternative Managed Access Pathways to deliver drug therapies as soon as possible to achieve optimal patient outcomes

Recommendation 3: Invest in Canadian research in rare drug development, clinical trials, and outcomes monitoring

Session 1: Canada's Rare Disease Expert Network

Why Needed: Orphan drugs are, by definition, either the first or a significantly improved therapy for a rare disease which is also severe, progressive, or life-threatening. About 80% of rare diseases are genetic and many "transformative" therapies are based on understanding the gene and reducing the impact. But to achieve maximum benefits, we need to diagnose and treat patients as early as possible, preferably before they are symptomatic. Therefore, to optimize value from a Rare Disease Drug Strategy, we need to invest in infrastructure, namely, a Canadian Rare Disease Expert Network that includes Centres of Expertise, clinicians and researchers, and community providers. The goals of this network are to provide patients with rare diseases access to early, accurate diagnosis and referral to a specialist regardless of where they live in Canada.

Current status: Lack timely accurate diagnosis, expert rare disease care, and societal support.

- From symptoms to accurate diagnosis: average of 13 years, nearly six healthcare professionals and more than 3 wrong diagnoses
- HCPs experienced as lacking rare disease knowledge: 80% GPs, 62% paediatricians and 47% specialists not aware or knowledgeable
- Lack information and support: 42% did not get needed

information, 72% not counseled at diagnosis; 70% no psychosocial support

- Lack societal support: 90% experience educational, disability, and employment services uninformed about rare disease

Proposal: Canada's Rare Disease Network will be comprised of Centres of Expertise, healthcare providers and researchers and will function as an informal multi-provincial/territorial virtual network to:

- Promote awareness and support
- Establish provincial/ regional rare disease hubs to provide support on diagnosis and patient management
- Develop disease-specific clusters to collaborate on guidelines, training, research, care, and awareness
- Collaborate on Patient registries and data platform that can serve multiple purposes including collection of treatment-related patient outcomes

Session 2: Alternative Managed Access Pathways for Timely Access to Rare Disease Therapies

Why Needed: Rare disease medicines and other therapeutic interventions can dramatically change the progression or impact of disease. As the first effective therapies for previously untreated diseases or significant advances over existing treatments, there is an urgency to bring these out of clinical trials and into the broader patient community as soon as possible. In Canada, the pathway to timely and appropriate access becomes hopelessly mired in antiquated assessment and reimbursement processes that were never designed to appraise appropriate use and value of small population rare drugs or advanced therapies.

Canada stands alone among the "top-tier" high-income OECD countries in having no process for immediate access to approved drugs even those that can save a patient's life, prevent or reduce crises, or slow or halt irreversible damage and no separate pathway for access to rare disease drugs. "Managed access programs" (MAPs) or "access with evidence development" (AED) programs allow for immediate post-approval access with real-world data collection. There are established pathways for MAPs in some countries but not in Canada. In rare cases when used, MAPs are negotiated on a province-by-province

basis, with no national criteria for access, no national registry, and no national data platform for collecting and analyzing patient outcomes to enable learning and updating the initial access protocol.

Current status: Canadians living with rare disease do not have timely access to essential medicines

- More than 40% (2 out of 5) are not getting access to medicines that are appropriate, approved, and/or prescribed to meet their needs
- More than 60% cannot access “off-label” medicines
- More than one-third cannot access prescribed medicines due to cost (lack of coverage or high co-pays)

Proposal: Alternative Managed Pathways are needed for timely access to approved (innovative) orphan drugs and rare disease therapies, including immediate access through managed access programs (a.k.a. coverage with evidence development) for patients with urgent, unmet, or underserved needs.

The success of Managed Access Pathways requires capacity to collect safety data and outcomes in real-world usage. To that end, Canada’s Rare Disease Network of rare disease centres shall encompass a real-world patient data platform. We propose an experimental approach whereby on a case-by-case basis, we can custom design a “fit-for-purposed” managed access pathway based on the disorder, therapy, and Canadian healthcare context.

Session 3: Canadian Research in Drug Development, Trials, and Outcomes

Why Needed: By all counts, the 1983 USA Orphan Drug Act has been wildly successful. In the prior decade, there had been only 10 new drugs approved for rare diseases; four decades later, there have about 800 approved orphan drugs benefitting over 50 million patients worldwide. Other countries adopting orphan drug legislation include the European Union (27 countries), Japan, Australia, Taiwan, South Korea, and the Philippines. In contrast, in 1996, Health Canada declared Orphan Drug legislation was unnecessary since the USA was developing drugs and a majority (63%) were also approved in Canada. Finally, in 2012, Health Canada announced the intention to develop an ”orphan drug framework” to “harmonize” its regulatory policies with those of other jurisdictions. The OD framework was completed but, inexplicably, was never brought to the legislature for vote.

Current status: Despite the lack of facilitating incentives, academic and private sector researchers have discovered and developed a number of rare disease drugs, including gene therapies, although most were transferred (sold) to

ex-Canada firms for late-stage clinical trials and marketing. Today, in the wake of the pandemic, the federal and provincial governments have announced increased investments in biomanufacturing and life sciences to drive innovation, manufacturing, and training, other institutions and organizations, including MICYRN, CIHR, Genome Canada, Life Sciences Ontario, Invivo Montreal, Ontario Genomics and Genome BC, it may be the right time to revitalize Canada's investment in rare disease research, including drug development and clinical trials

Proposal: Enable and facilitate collaboration on multi-site rare disease research, including:

- Clinical trials
- Understanding etiology and natural history of disease
- Discovering or enhancing or repurposing drug treatments
- Designing or applying technology from devices to AI that improve delivery of healthcare and support patient engagement and self-management
- Participation in global rare disease research

Session 4: Critical Success Factors
(to be developed)

- Patient Registries and Data Platform
- Returning Value for Investment
- Demonstrating Individual Impact