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THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

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RAREi Recommendations

1 – Optimize and accelerate the implementation of the National Strategy for Drugs for Rare Diseases (DRDs), working collaboratively with the rare diseases community

2 – Ensure that the promised funding for DRDs leads to patients receiving coverage for needed treatments as soon as possible

3 – Expand the focus of the federal Biomanufacturing and Life Sciences Strategy to improve the policy, research and commercial environment for developers of medicines for rare diseases

About RAREi

RAREi is a network of 17 Canadian biopharmaceutical companies committed to improving the lives of rare disease patients by researching, developing and commercializing rare disease treatments.

Recommendations in Context

Recommendation 1 **– Optimize and accelerate the implementation of the National Strategy for Drugs for Rare Diseases (DRDs), working collaboratively with the rare diseases community.**

The Canadian rare disease community welcomed the recent launch of Health Canada's national strategy for drugs for rare diseases (DRDs) and the creation of a dedicated directorate within the department to address rare disease policy. The announcement, along with a major funding commitment of $1.5 billion during the next three years, is a significant development at the federal level. RAREi has invested in a number of forecasts addressing the cost of investing in non-cancer rare disease medicines – last updated in 2022 – and the planned spending would help fund a very significant portion of provincial health system spending on rare disease medicines, allowing them to provide patient access much more quickly and comprehensively than in the past.

At the same time, a number of concerns remain, including a continuing lack of direct patient and community involvement in building the strategy, inadequate underlying infrastructure to ensure that patients in need can receive the treatments they require and the absence of clear objectives or targets against which the strategy can be monitored, evaluated and recalibrated.

A March 2023 announcement launched the strategy (RDDS), but it was limited to addressing DRDs only and there was no accompanying strategy document beyond a press release and a backgrounder. The details remain to be developed and there is limited recognition in the materials provided that effective management of rare diseases requires much more than just access to medicines.

Good rare disease care would ensure that patients can be identified, screened, tested, diagnosed, referred to knowledgeable clinicians, treated actively with appropriate interventions and supported throughout their journey.

Canada remains one of the only developed nations in the world without a dedicated, government-endorsed national rare disease strategy. This has contributed to patients' ongoing and uphill battle when trying to obtaining effective care and treatment.

The good news is that under the leadership of the Canadian Organization for Rare Disorders (CORD), a broad-ranging multi-stakeholder rare disease community coalition is working collaboratively to develop and establish a national network of rare disease expertise that aims to support the diagnosis, care and treatment of rare disease patients wherever they live in Canada. The group is also providing substantive input on accelerated pathways for the evaluation and funding of rare disease medicines. The initiative involves patients, a wide range of clinical experts (physicians and other health care

professionals), policy experts and innovators to envision and create the infrastructure necessary to ensure rare disease patients will be well-served by the Canadian health care system.

RAREi strongly endorses that activity and encourages the federal government and its provincial counterparts to join the effort. It would be ideal if Health Canada would participate and integrate its current DRDs strategy work into the broader initiative. To that end, one concrete step that Health Canada could take immediately would be to recognize the work that CORD has underway, and ensure the RDDS is aligned with and supports those efforts.

Recommendation 2 **– Ensure that the promised funding for DRDs leads to patients receiving coverage for needed treatments as soon as possible**

Please note that it took four years following the 2019 federal budget commitment for rare disease treatments before the funding was announced, and while some of the investments have been announced with respect to research, the vast majority of the committed money is not yet flowing to provinces and territories. Canadian rare disease patients are waiting anxiously for funds to start flowing sooner than later.

The snail's pace at which the inter-governmental negotiations are proceeding is disappointing, as is the lack of involvement by the rare disease community in framing and facilitating access in a way that meets patients' needs effectively.

One of the key stumbling blocks is the fact that the current public reimbursement review and approval process simply does not meet rare disease patients' needs. The process is explicitly focused on identifying evidentiary uncertainty, which is inevitable with medications intended for use in small patient populations. Therefore, unless the process is reformed, new orphan treatments will continue to be challenged in getting through it successfully and thereby, achieving public reimbursement. RAREi urges all public payers to be innovative in the way that they evaluate new rare disease treatments and to customize all aspects of the review process in a manner that reflects the reality of orphan treatment development. In the meantime, there are a number of potential ways to accelerate access to needed medicines that should be considered.

Specifically, early access opportunities must be created which permit patients to benefit from promising treatments and innovators to demonstrate their products' value through real-world experience, with requirements for data collection and reassessments after sufficient evidence is obtained and analyzed.

One key concern (which is shared by the broader rare disease community in Canada) is the strategy's reliance on a small subset of medicines that would be expected to be reimbursed equally across the country. RAREi contends that reliance on a defined list of covered medicines will not keep up with the evolving science and will fail to cover patients equitably. The complex nature of treating rare diseases indicates that one-size-fits-all models create problems for clinicians and make it challenging to meet patient needs.

There is another way to improve consistency across public drug programs in Canada. Canada already has a *de facto* national list of medicines for public reimbursement. All the major public drug plans in Canada are participating members in the pan-Canadian Pharmaceutical Alliance (pCPA), which negotiates favourable terms with innovators to facilitate public reimbursement. Successful negotiations lead to mutually agreed commercial terms outlined in a letters of intent (LoI) that apply nationally. However, it is often true that signed LoIs do not lead to listings on public plan formularies for a variety of reasons. Simply by specifying that the LoI terms related to any successfully negotiated rare disease medicine by the pCPA represent a ***commitment*** by participating payers to reimburse the product (rather than an ***option to list*** as is the case now) would ensure more consistent coverage would be achieved nationwide without the need to create any new processes or lists. The new federal funding could be used to support those commitments.

Another reform at the pCPA level which should be considered is the elimination of the participating plans' insistence that product negotiations be based exclusively on recommendations developed by the health technology assessment (HTA) agencies on which it relies. Given that products designed for small populations are not well-served by the current HTA review process, that limitation often stops negotiations from going anywhere from the outset. If negotiations could proceed based on best available evidence and a pragmatic understanding of what can be provided, it would give much more scope for fruitful negotiations.

In addition, consideration should be given to allowing for HTA reviews to be waived or elements omitted for some rare disease treatments that represent a small budget impact or when it is not practical to obtain the level of evidence typically required for a comprehensive HTA review. Some rare disease medicines are for such a small number of patients in Canada that it makes little commercial sense to develop elaborate patient registries and evidence-development programs. Targeted exemptions to address circumstances like these are in place in Europe, and are often a critical incentive for developers to research and market innovations in those jurisdictions. There should be an objective triage system implemented that would determine the eligibility for a new innovation for such waivers or exemptions.

Finally, exceptional access programs (like Quebec's *Patient d'exception* mechanism) should be created in all jurisdictions to allow for case-by-case reviews of access requests when patients' needs are outside the HTA recommended indications or when an HTA body has recommended against reimbursement, but the product remains the best available option for an individual patient.

Implementation of some or all these suggested approaches could be achieved relatively rapidly and would facilitate almost immediate improvements in the current access environment. RAREi urges Health Canada to explore these concepts with the provinces and territories in the context of its planned bilateral negotiations related to $1.4 billion set aside to promote improved patient access to DRDs on a priority basis.

Recommendation 3 **– Expand the focus of the federal Biomanufacturing and Life Sciences Strategy to improve the policy, research and commercial environment for developers of medicines for rare diseases**

As medical research evolves into an era of increasingly personalized treatments that rely on a genomics-based understanding of disease and illness, rare disease treatment development represents something of a vanguard on which many developed countries are increasingly taking advantage to drive economic growth.

The dramatic increase in the number of new treatments for orphan conditions can be traced directly to ground-breaking policy choices made by governments in the US, Europe and Asia that are designed to encourage research and innovation that targets rare diseases.

Canada has a critical opportunity to build out its capacity in this area and catch up with our counterparts to the south and overseas. Canada has a unique opportunity to capitalize on the momentum currently driven by federal, national, provincial and stakeholder efforts in rare disorders policy and life sciences.

The federal biomanufacturing and life sciences strategy (BLSS) announced in the summer of 2021 contains some elements that can help improve Canada's competitiveness on the global stage, including an emphasis on enabling innovation by ensuring world class regulation. Still the government should go further, by linking the BLSS to the RDDS in a way that facilitates access and drives innovation.

In this context, RAREi recommends that the federal government consider adding the following rare disease research elements to its BLSS.

- *Formal rare disease treatment designation* – Based on agreed upon eligibility criteria, Health Canada would designate new medicines as rare disease treatments. The designation would guarantee that the product would be reviewed and considered as a priority throughout the Canadian medication review and approval process. In addition, the sponsor would be encouraged to take advantage of any regulatory measures designed to permit adaptive approaches to review.
- *Potential for waiver or reduction in review fees* – In cases when the anticipated number of patients to be served is small and/or the expect budget impact of a product is likely to be minimal, sponsors should be encouraged to submit products as part of the medication review and approval process by offering them reduced or no fees for any reviews required.
- *Additional intellectual property protection* – When innovators undertake clinical research in orphan diseases, the opportunity for extended patent life and/or additional data protection should be available.
- *Regulatory sandboxes* – As part of the federal regulatory modernization framework, the government has proposed the use of regulatory sandboxes as a means of road-testing new and innovative approaches to review and assessments. Opportunities should encourage government, researcher and industry innovators to propose and pursue new approaches with regulators, reviewers and payers to assessing and making products available for patients.

Adding any or all of these to the Canadian system would demonstrate that Canada wishes to compete for the economic development opportunities that arise from rare disease development. They should be considered as core elements of both the BLSS and the DRD strategies.