Addendum to IMC Submission on Drugs for Rare Diseases

September 15, 2021

Background

On August 11, 2021, Health Canada requested additional details regarding Innovative Medicines Canada's March 2021 consultation submission on Drugs for Rare Diseases. In particular, Health Canada is seeking input on the government's immediate focus on the use of budgeted funds of $500 million per year to be invested in drugs for rare diseases, beginning in 2022.

Health Canada indicated that it is open to receiving further input on possible use of these funds by mid-September 2021. As an addendum to IMC's original submission (attached for reference), this document identifies specific ideas for further dialogue with Health Canada and rare disease stakeholders.

Key Considerations for 2022 Investments

IMC has previously identified key principles and international best practices for a made-in-Canada rare disease policy.[i] There is a clear ongoing need for a comprehensive pan-Canadian policy to advance rare disease innovation and enhance timely patient access. IMC also notes that the funding allocated for this initiative, while material, will be insufficient to address many of the needs and solutions arising from Drugs for Rare Diseases. A holistic and long-term commitment beyond 2022 will be required for an effective pan-Canadian rare disease policy. Notwithstanding these concerns, regarding the immediate 2022 investments, some additional key parameters are set out below to help guide the federal government's decision making:

- **Patients must receive tangible access benefits:** A majority of the funds (~80%) should be used to directly support provincial governments in providing patients with timely access to treatments and to support provincial screening/diagnostic infrastructure.
- **Future focus:** Funds should be used to provide access to future innovations which address unmet patient needs, rather than revisiting coverage agreements for technologies already adopted within the care pathway.
- **Prudent use of limited resources:** Given that federal investments are significant but cannot address the entire rare disease market, the funds should be directed to supporting public drug plans and not to offsetting private drug plan costs. Additionally, administrative costs must be minimized to maximize patient and health system benefits.
- **Balance federal initiatives with provincial needs and delivery:** The approach should balance the practical benefits of fiscal transfers to provinces with pan-Canadian policy initiatives and investments.
    - Given provincial responsibilities for medicine coverage and the need for efficient deployment of funds, Federal-Provincial transfers will necessarily be a central element. Transfer payments would allow provinces to address local needs, for example, to direct funding towards increased capacity for screening and diagnosis (e.g., prenatal / genomic).
    - Additionally, an incremental approach to pan-Canadian policy can provide specific federal areas of focus. The federal government can make a meaningful contribution in areas of federal jurisdiction, for example, by streamlining the product review pathway for rare diseases and through pan-Canadian data infrastructure investments in support of Real-World Evidence (RWE).

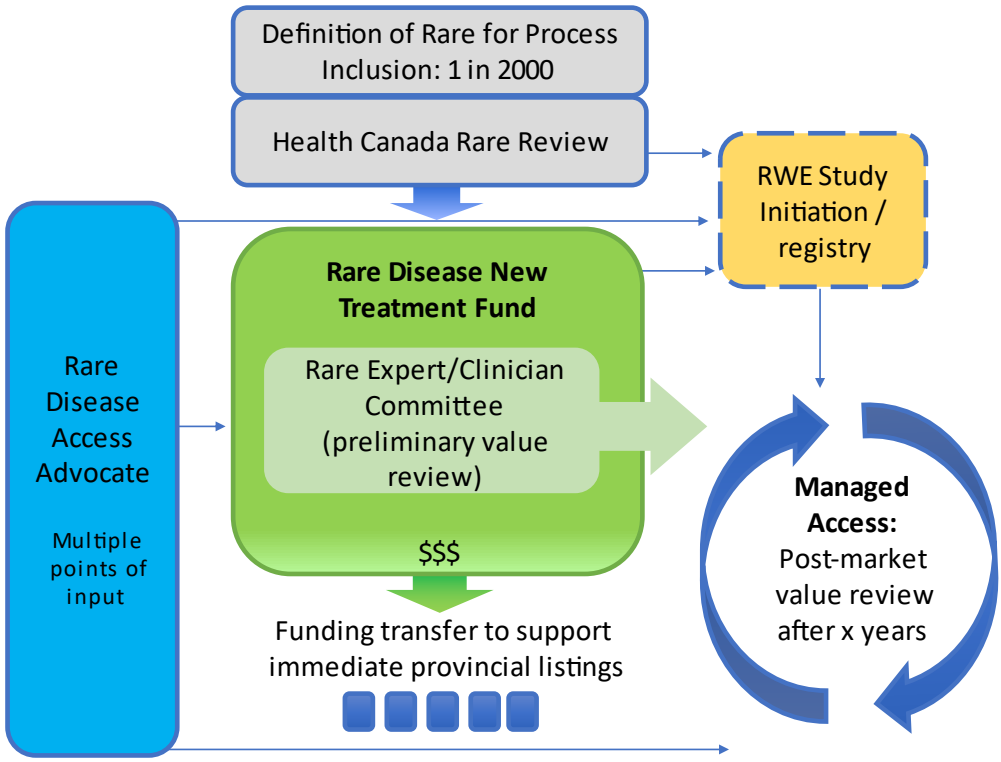
- A more detailed breakdown of possible funding distributions to achieve such a balance is provided in the table below.

A Value-Based Rare Disease Review Pathway to Fast-Track Access

As articulated in IMC's previous submission, the federal government should create a dedicated and accelerated pathway for those medicines meeting a prevalence definition of rare (e.g., no more than 1 in 2000 population). Medicines meeting this definition could enter into a streamlined Health Canada regulatory review. In parallel, value assessments could be conducted by a recommendation committee (separate from CADTH) comprised of rare disease experts. This committee would directly engage with patients and manufacturers, and de-emphasize the role of traditional pharmacoeconomics, which is poorly suited to assess drugs for rare conditions. Please see Figure 1 for a process overview.

In accordance with the discussion of the transfer model set out above, a Rare Disease New Treatment fund could guide transfer payments to the provinces and house the committee of rare disease expert clinicians. This committee could, in consultation with patients, conduct initial assessments, advise on RWE development projects, and where needed, review the value of rare disease products after some period of time on the market as part of a value-based or managed access framework (e.g., to support early and conditional uptake of treatments on the basis of Phase II trials with ongoing evidence generation). As discussed in IMC's March 2021 submission, there are several international best practice models that Canada could consider as part of a made-in-Canada approach to managed access.

Figure 1: Dedicated Rare Disease Pathway Schematic



To facilitate this work, the federal government could make pan-Canadian data infrastructure investments to support RWE generation and fund patient disease registries. Allocations would necessarily be larger in the initial years to provide seed funding to build infrastructure. Decision making regarding the use of funds could be informed by a committee of rare disease patients, the expert clinician committee, as well as specific RWE proposals from manufacturers and vendors.

An office of an independent Rare Disease Access Advocate, selected with patient input, could be engaged to help establish the pathway and have a mandate to facilitate timely access. The advocate role could be fashioned in an ombudsperson office model and include a governance body comprised of rare disease stakeholders. The patient advocate could have multiple points of input into the process including working with regulators, providing input into patient registries and RWE study initiation, input into expert committee discussions, post-market value reviews, and working with patients directly to help navigate and secure access.

Possible Funding Scenario

The current funding could be allocated according to the following breakdown. It should be noted that federal discussions with the provinces would be required to determine specific transfer allocations. A starting point for these discussions might be allocations on a per capita basis or provincial disease prevalence basis.

	Years One and Two (set up)	**Year three (ongoing)**
Pan-Canadian Data Infrastructure for RWE / Patient Registries	$100 million	$25 million ongoing/new projects
Rare disease "access advocate" office	$5 million	$3 million ongoing
Rare disease access fund admin / secretariat	$2 million	$2 million ongoing
Independent Expert Review Group (separate from CADTH)	$5 million	$3 million ongoing
Funds available for provincial transfers for medicines and screening	$388 million	$462 million
TOTAL	**$500 Million**	**$500 Million**

Conclusion and Next Steps

IMC wishes to thank Health Canada for the opportunity to provide additional detail on IMC's submission and more specific input into Health Canada's 2022 priorities regarding drugs for rare diseases. As discussed, this addendum offers a starting point for further stakeholder discussion and may provide a first step to a broader and more comprehensive rare disease policy that could include elements such as regulatory improvements and incentives for medicine research and development. We note that Health Canada indicated at our August meeting that it would continue its consultations with stakeholders regarding Drugs for Rare Diseases in the Fall of 2021, and IMC welcomes the opportunity for further dialogue at that time.

[i] **Rare disease policy should:** be sensitive to provincial and patient needs; provide a predictable definition of rarity; enhance a predicable pathway for timely access with fair value for patients, payers and companies; leverage high quality real world evidence generation and enhance data infrastructure; provide incentives for rare disease innovation; maintain a viable private insurance market and the avoidance of cost-shifting from private plans to public plans; and recognize that a rare disease policy cannot be successful in the context of pending PMPRB regulatory changes, especially new economic factors.