Canadian Organization for Rare Disorders
Initial Response to:
Canadian Government's Discussion Guide for National Strategy on Drugs for Rare Diseases

CORD High-Level Critique

Our reactions to What We Heard: We feel dismayed, bewildered, and betrayed.

- Know you heard us because your recapitulation of your WWH summary was mostly what we said back then
- Potential Draft Framework: sets up the slippery slope toward no meaningful action
- Language throughout is highly speculative, theoretical, and deliberately vague with no commitment to actions, goals or outcomes, and timelines

1. Potential common vision is underwhelming, not worthy of the boldacious 2019 government commitment backed with $1 billion initial funding. We cannot galvanize the Canadian rare disease community to collective action with a vision of "improved access" and "better health outcomes."
2. These eight principles are meaningless. Principles are "fundamental truths" that underlie actions, whereas these are presented as a list of words with no explanation as to how these will be fundamental to the design, implementation and evaluation of the Rare Disease Drug Framework.
3. Invest in 4 Strategic Pillars

- These are not strategic nor pillars nor even "four" different sects of activities but necessarily highly related toward one (unstated) goal
- Many of the activities are couched in the form of "advisory" activities, such as "explore feasibility", "engage with", "create a plan for future", "support", "invest in", "build relationships with" with no reference as to "the entity" that is doing the advising (government, advisory committee, new drug agency) and to whom the advice is provide for execution (designated or accountable body or coalitions).
- There are no linkages of activities to the guiding principles or the desired outcomes of these activities.

Pillar 1:

- Improved and consistent access could be a "death spiral" unless there is a clear commitment to an overarching principle: All rare disease patients shall get the fasted access possible to the best drug for their specific condition and personal profile in the shortest time possible (through clinical trials, special access, and other early access paths) comparable to the best countries). Otherwise:

- Improved access without requirement of best could mean any therapy that is better than nothing or access for all rare disease patients to the lowest common denominator of willingness to fund
- Without commitment to rapid access, coordination across decision makers could lead to intolerable delays anchored by the least willing funder

Pillar 2: Optimize, collect and use data along drug system continuum and across lifecycle must be based on:

- Principle of "adaptive learning", that is, recognize the realities of our non-existent national data platform and system (have we learned nothing from InfoWay)?
- Learn from discrete programs (existing and new) which rely on existing data systems and platforms that are already collecting health data and using them to monitor treatment and outcomes, in specialty clinical networks, in cancer networks, in private services using and service public systems.

Pillar 3: Support optimal patient outcomes and sustainability of health system by spending on drugs that bring "value for money."

- This pillar is just big giveaway or back door to health technology assessment (HTA), that is, cost-effectiveness and cost-utility assessments (cost per Quality-Adjusted Life Year calculations) with comparative and competitive ICERS (incremental cost-effectiveness ratios). The premise of "value for money" can ONLY work in achieving the best outcomes for patients and society are the driving principles and IF the failure of tradition HTA methodologies (timing, evidence requirements, and benchmark ICERs) to achieve optimal and even reasonable assessments for rare disease therapies.
- Additional concern for Pillars 1, 2, and 3: these are all the same domain and all directed toward shared or consensual HTA for DRDs and providing access to those that meet whatever criteria or threshold would be deemed "value for money." These do not represent an innovative step forward.

Pillar 4: Strengthen alignment of research and innovative systems with DRD access objectives

- This in and of itself is not very illuminating. However, the proposal to build rare disease research capacity to create data collection and sharing systems that can contribute to clinical trials (and other goals) is worthwhile, even if vague as to implementation.

Iterative Implementation

Drug Coverage

- We are truly baffled by the proposal that the first phase of an "iterative implementation" of drug coverage should be federal support, with partners and payers, of coverage for "select drugs of common concern."
- First of all, there is absolutely nothing in any of the What We Heard, what CORD submitted, or what any pay has submitted to suggest that this is the most pressing need for a national Rare Disease Drug Strategy.

- While there are gaps in access for patients with neither private or public drug coverage, this approach duplicates much of what already exists, a strategy that runs counter to the importance of not duplicating what is in place.
- A more reasonable first step provide coverage to all families, similar to the Quebec model with mandatory drug coverage.
- The greatest obstacle to consensus is which drugs are to selected by whom and serving whose common concern? By definition, rare disease drugs are not common concerns.
- As the testimonials, case studies, and advocacy appeals have demonstrated, the top priority for a new Rare Disease Drug Strategy is access to those (often new and advanced) therapies that have the greatest impact on saving lives, improving disease outcomes, or transforming lives. We have proposed accelerated access pathways that would provide access at the very earliest possibility, that is, during clinical trials, pre-NOC, immediately upon NOC through managed access programs that would provide patient access with provisions for monitoring and real-world data collection, with reimbursement potentially conditional on outcomes.
- An initial "common concerns" drug list does not address the patient needs, the system gaps and definitely not long-term "value for money" and sustainability of the Rare Drug program and the health system.

Governance

- A governance structure, ideally an independent Rare Disease Drug Program, should include all stakeholders at all levels in multiple roles, including patients who serve not only on advisory committees but assessment, monitoring, and decision-making bodies at every level, including the top governing board.
- Committees for specific therapies should include experts from the affected rare disease community, including patients and families, clinicians, and other care providers.
- All stakeholders should receive the necessary training and support to share expertise in a collaborative environment.
- Transparency should include open access to all materials and to the assessment, deliberation, and decision-making processes.

Optimal patient outcomes

- Optimal patient outcomes through "value for money" is just another way of describing health technology assessment, which has not worked with its traditional methodologies for rare disease drugs, not in Canada and indeed not anywhere in the world.
- Streamlining the process and efforts for DRD will NOT resolve the fact that the traditional model is not "fit for purpose", which is true worldwide.
- Risk-sharing and equitable sharing of costs among payers can only be achieved by created a single platform for assessment and development of "managed access" agreements based on providing the drugs to the patients on an appropriate "risk

assessment" that will take into consideration the evidence from clinical trials, the uncertainties in terms of extension to non-trial patients, and a process for collecting outcomes in real-world to monitor safety and efficacy.

- Sharing of costs is a very different process than managing or sharing the risks of access with the patients and the clinical community. Risk-sharing of the costs and innovative payment models are on-going processes where the costs paid (over time) may vary as evidence on outcomes and number of patients evolve over time in real-world usage.

Summary Outline of Key Points

What We Heard Summary

Improving access and consistent decisions for RDD

- Value drugs bring to patients; enable sustainable access
- Build consistent evidence
- Leverage or build infrastructure
- Expand coverage while not comprising existing coverage
- Opportunities for access to promising treatments, despite clinical uncertainties (no other treatments or lifesaving or transformative)
- National coordinating mechanism, advisory structures, common decision making-tools for centralized and evidence-informed decision making
- Transparency, clear communication in DM: how to access drugs, eligibility criteria, rationale for coverage, timelines, appeals
- Sustainability: involve multiple payers in sharing risk and costs for drugs to maximize inclusion across Canada

Coordination and collection data

- Coordination, avoid duplication throughout pharmaceutic management system
- Leverage and strengthen current PMS
- Continuous review with RW safety and efficacy evidence, modification of treatment initiation and discontinuous criteria
- Independent national data system or patient registries to monitor treatment outcomes and disease progression; improve value and clinical effectiveness

Evidence generation and capacity building

- Promote research, clinical trials and open science approach to improve knowledge and develop RD treatments
- Mindful of financial impact of COVID
- Partnerships, engage patients, caregivers, indigenous, clinicians, others in future DM, including meaningful outcome measures
- Invest in infrastructure to address gaps for reimbursement and formulary decisions
- Support clinicians and researchers in building knowledge for evidence-informed care decisions

Proposed Draft Framework

Potential common vision

- Patients with rare diseases have improved access to effective drugs and better health outcomes

Preliminary underlying principles

- Patient-centered
- Transparent and accountable
- System alignment and sustainability
- Ethical
- Efficient and effective
- Evidence-informed
- Collaborative and inclusive
- Adaptive

Invest in activities across 4 strategic pillars

1. Improve access and make it consistent across Canada
   - Common vision and commitment for national strategy Common vision and commitment for national strategy
   - Coordination and shared DM around reimbursement decisions
   - Optimize, collect, and use evidence to meet needs of DM along management continuum and lifecycle of drug
2. Develop data standards to collect, build knowledge
   - Leverage and analyze data, including RWE generation to improve on-going DM process
3. Support optimal patient outcomes and sustainability by ensuring spending on DRD brings "value for money"
   - Streamline process and efforts for DRD
   - Promote risk-sharing and equitable cost sharing among payers
   - Explore innovative drug reimbursement models
4. Align research and innovation systems with DRD access objectives
   - Build on RD research capacity
   - Support access to data to spur clinical trials

Iterative implementation approach

Overview

- Continuous learning and agility to evaluate, adapt and improve
- Foundational phase and corresponding set of activities to yield outputs to shape future decisions and adaptations
- Potential activities include action across drug coverage, governance, and evidence generation

Drug coverage

- Work with partners and payers to improve accessibility of RDD by supporting coverage for select drugs of common concern.
- Initial drug list form basis to work toward formulary for DRD
- Example activities
    - Initial set of RDD
    - Adopt common principle and develop DM framework to assess and manage formulary of DRD
    - Horizon scanning and planning for pipeline of RDD

Governance

- To manage list of select drugs and evidence generation activities
- Structure should leverage existing structures as much as possible
- Example activities
    - Fit-for-purpose advisory committees and WGs including grange of partners and stakeholder groups
    - Explore feasibility of sustainable and innovative cost-sharing/risk-sharing models with multiple players
    - Relationships with international partners and networks; foster information sharing among health system partners

Evidence generation infrastructure

- Invest in real-world data and evidence activities (patient registries), support data framework/data standards, data-sharing agreements,
- Investments for improved knowledge of RD and drugs
- Example activities
    - Draft governance framework
    - Plan for future national data system, including assessing, piloting, and enhancing existing databases and registries
    - Engage with indigenous peoples to collaborate on governance and infrastructure, including data governance

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payment models are on-going processes where the costs paid (over time) may vary as evidence on outcomes and number of patients evolve over time in real-world usage.

- To assure effectiveness of access to rare disease drugs with high uncertainty based on initial clinical trial evidence, it is essential to establish a Canadian Network of Rare Disease Centres of Excellence, with specialty networks embedded across the Centres. These Centres of Excellence are also the vehicles for data collection and analysis, which will allow for aligning research and therapy management.