Bumpy Road to Access: Navigating the Speed Bumps, Potholes, and Roadblocks

February 28-29, 2024

Canadian Organization for Rare Disorders

Amazing news to announce to celebrate Rare Disease Day 2024

RÉSEAU CANADIEN DES MALADIES RARES

CANADIAN RARE DISEASE NETWORK

The challenges continue with the Fight For Our Lives

FIGHT FOR OUR LIVES

1. Visit www.fightforourlives.ca to make our voices heard!
2. Tomorrow, join us again on Parliament Hill (dress warmly!)

Drug Access: From Slow Multi-Step Stairway …

...designed for pre-90s, pre-biologics, orphan drugs, and gene therapies

…involving several distinct review agencies with unique but overlapping mandates

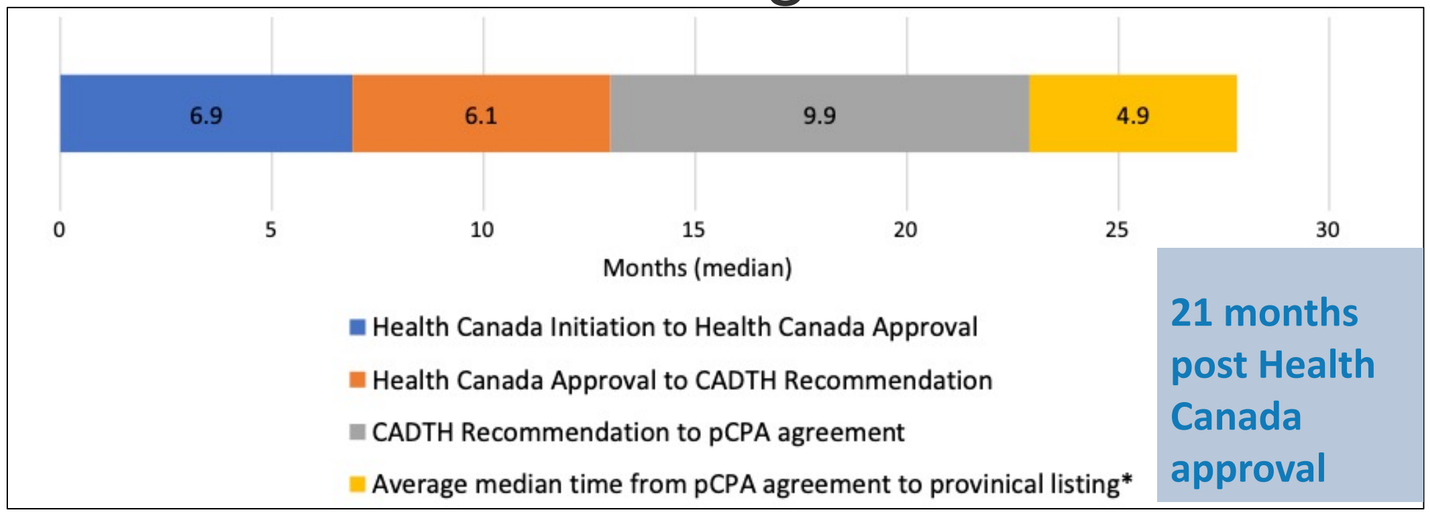
…making “non-binding” recommendations or negotiating non-binding agreements leading to delayed or no implementation by payers and limited patient access

…private drug plans delaying or deferring some reimbursement decisions to mimic public payers

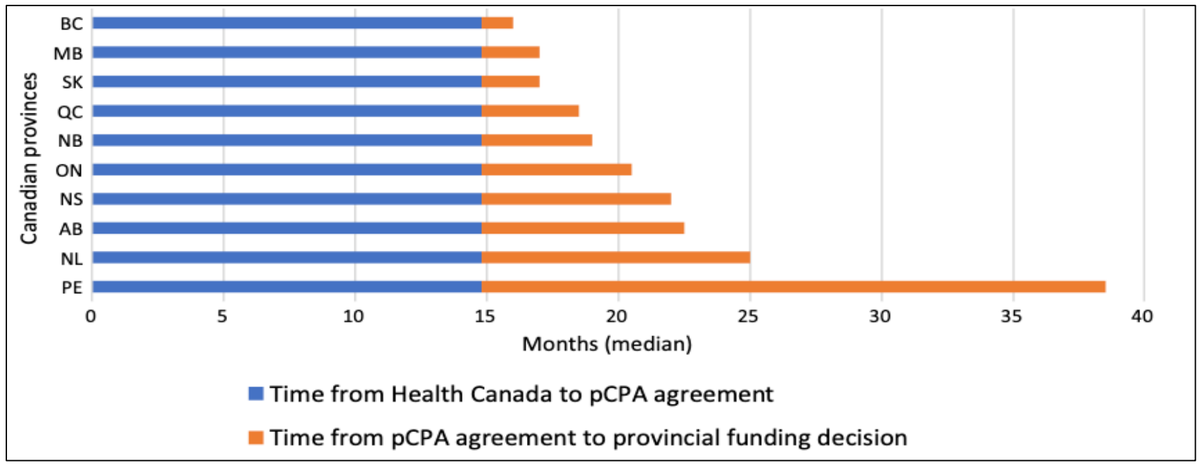
IMPACT for Patients: Delayed or no access

To express elevator...!

Canada has long review & reimbursement timelines for rare disease medicines cf. other high and middle income countries

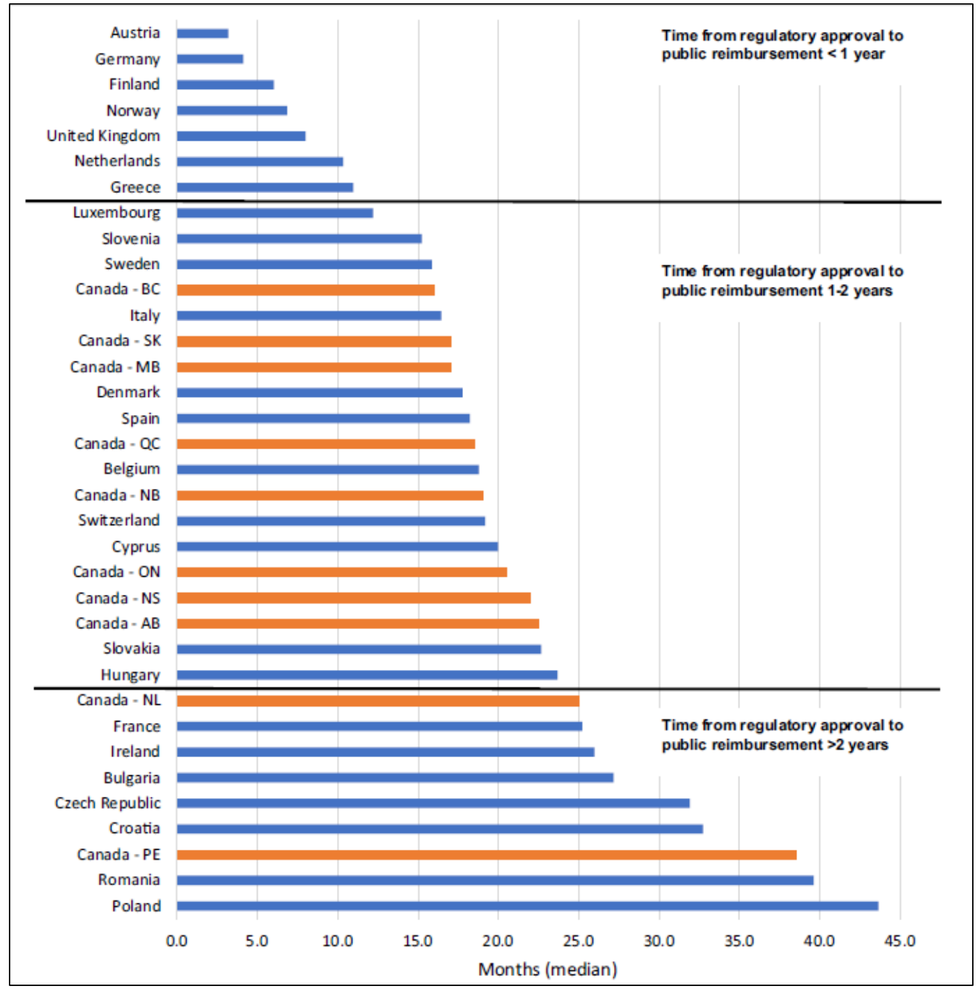


21 months post Health Canada approval



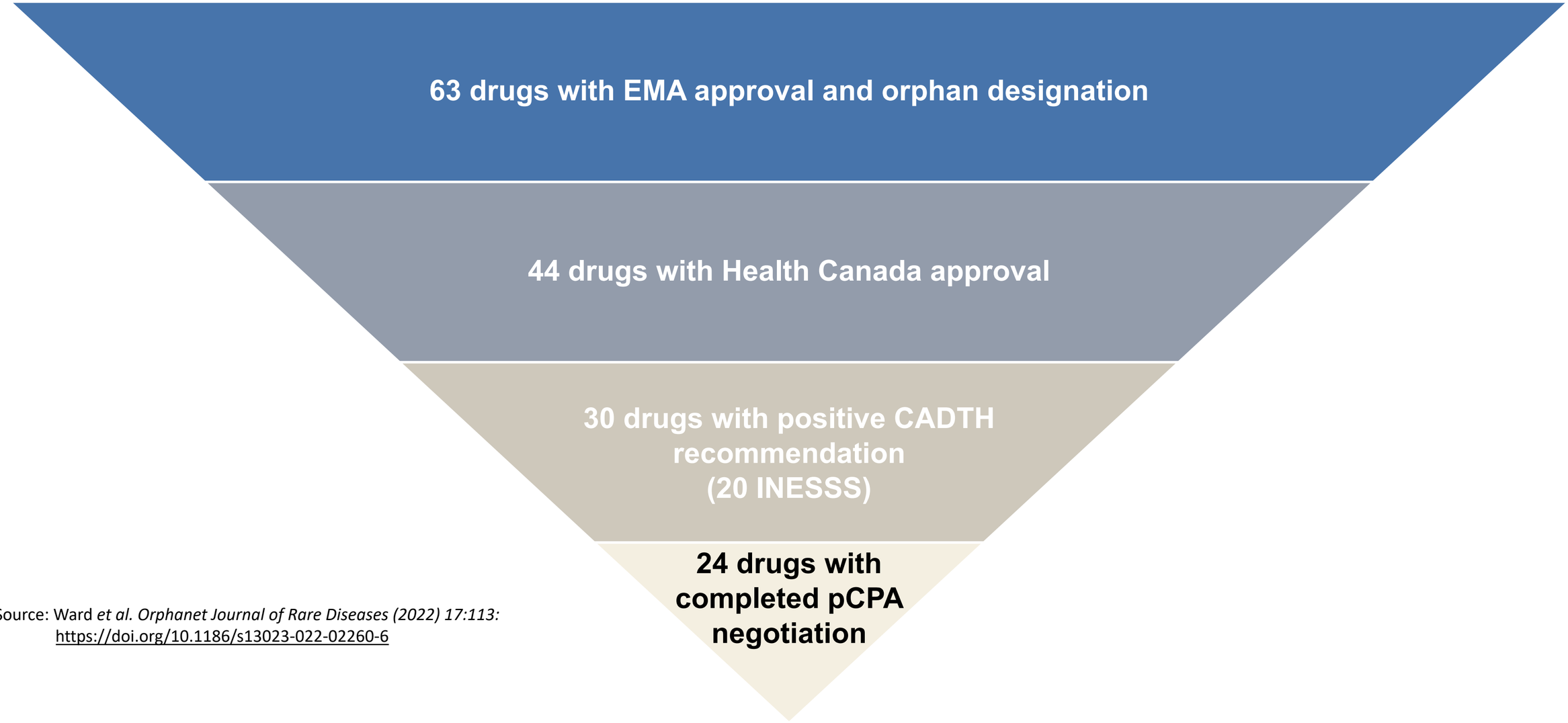
*PEI excluded from average provincial median timeline calculation because PEI timeline only 2 drugs reimbursed

Source: Ward *et al. Orphanet Journal of Rare Diseases (2022) 17:113:* https://doi.org/10.1186/s13023-022-02260-6



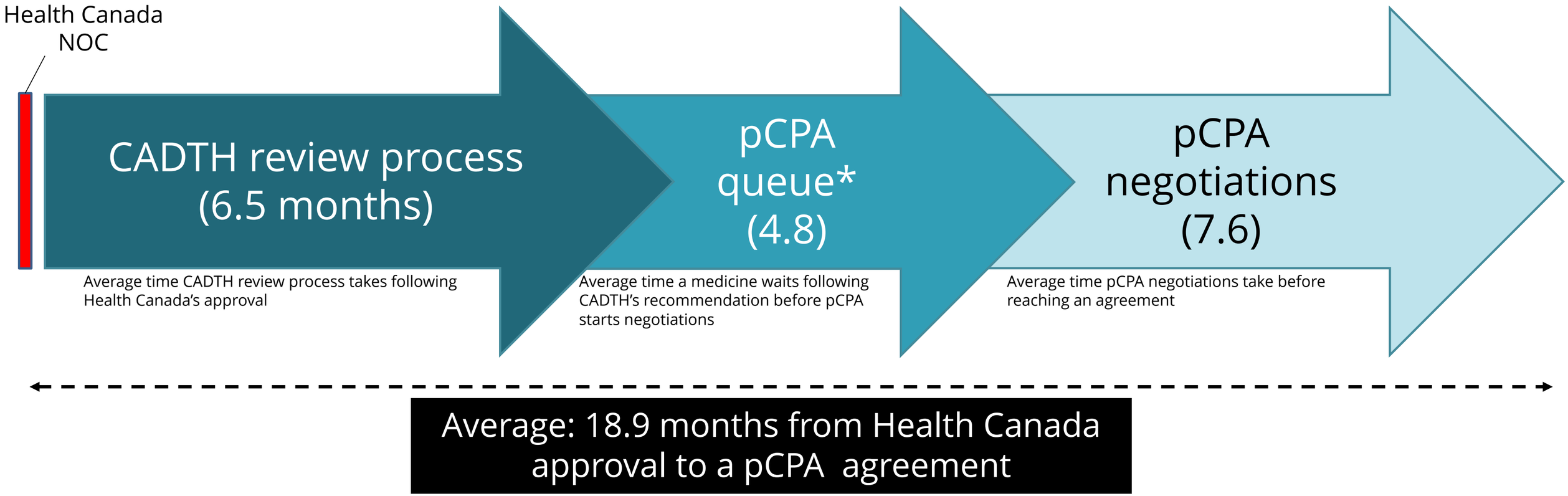
Time from Regulatory Approval to Reimbursement
Canadian Provinces cf. Other Countries

Far less access to publicly-funded medicines than other jurisdictions, for example, Europe



Source: Ward *et al. Orphanet Journal of Rare Diseases (2022) 17:113:* https://doi.org/10.1186/s13023-022-02260-6

Current Timelines are long



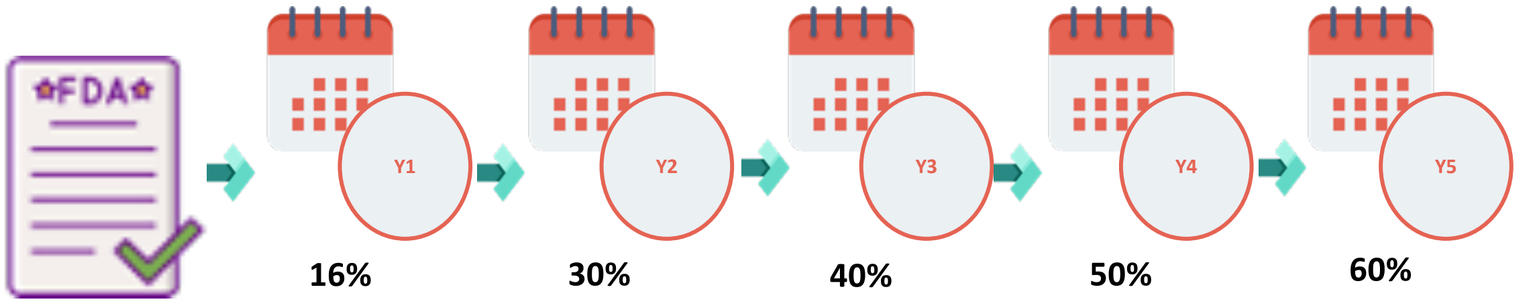
pCODR source: IQVIA, *Provincial Reimbursement Advisor*, Vol. 22, Issue 1 (May 2019), p. 62; pCPA wait time source: PDCI Market Access, *Target Pharma,* Status Summary of Innovative Medicine pCPA Negotiations as of Sep. 30, 2019 (2018 data); pCPA negotiations wait source: PDCI Market Access, *Target Pharma,* Status Summary of Innovative Medicine pCPA Negotiations as of Sep 30, 2019 (2018 data)

Only 3 of 5 DRDs approved by FDA/EMA are submitted to Health Canada and UP to 5 Years later

66.3%
Approved

33.7%
Rejected

Regulatory Success Rate[1]
Phase 3 to FDA/EMA approval



Proportion of DRDs that enter the Canadian market post-FDA/EMA approval[2,3]

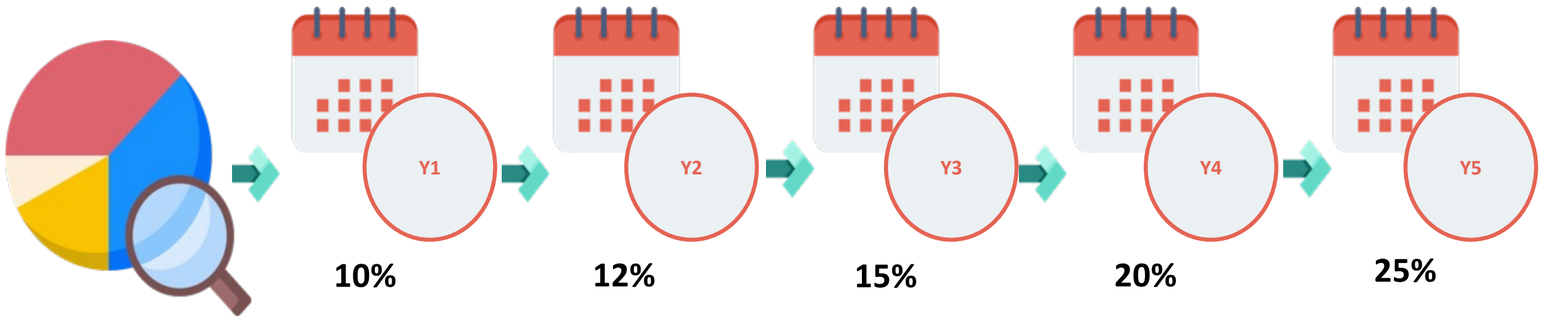
Abbreviations: FDA = Food and Drug Administration; EMA = European Medicines Agency; DRD = drug for rare disease. **Sources**: (1) Wong CH, et al. Estimation of clinical trial success rates and related parameters. Biostatistics, Volume 20, Issue 2, 2019. Available here. (2) Rawson SB. Fewer new drug approvals in Canada: early indication of unintended consequences from new PMPRB regs. 2020. Available here. (3) Canadian Organization for Rare Disorders: key facts. Available here.

Only 7 of 10 DRDs are recommended for reimbursement in public drug plans with only 25% eligible patients treated up to 5 years later

69.15%
Positive

30.85%
Do not list

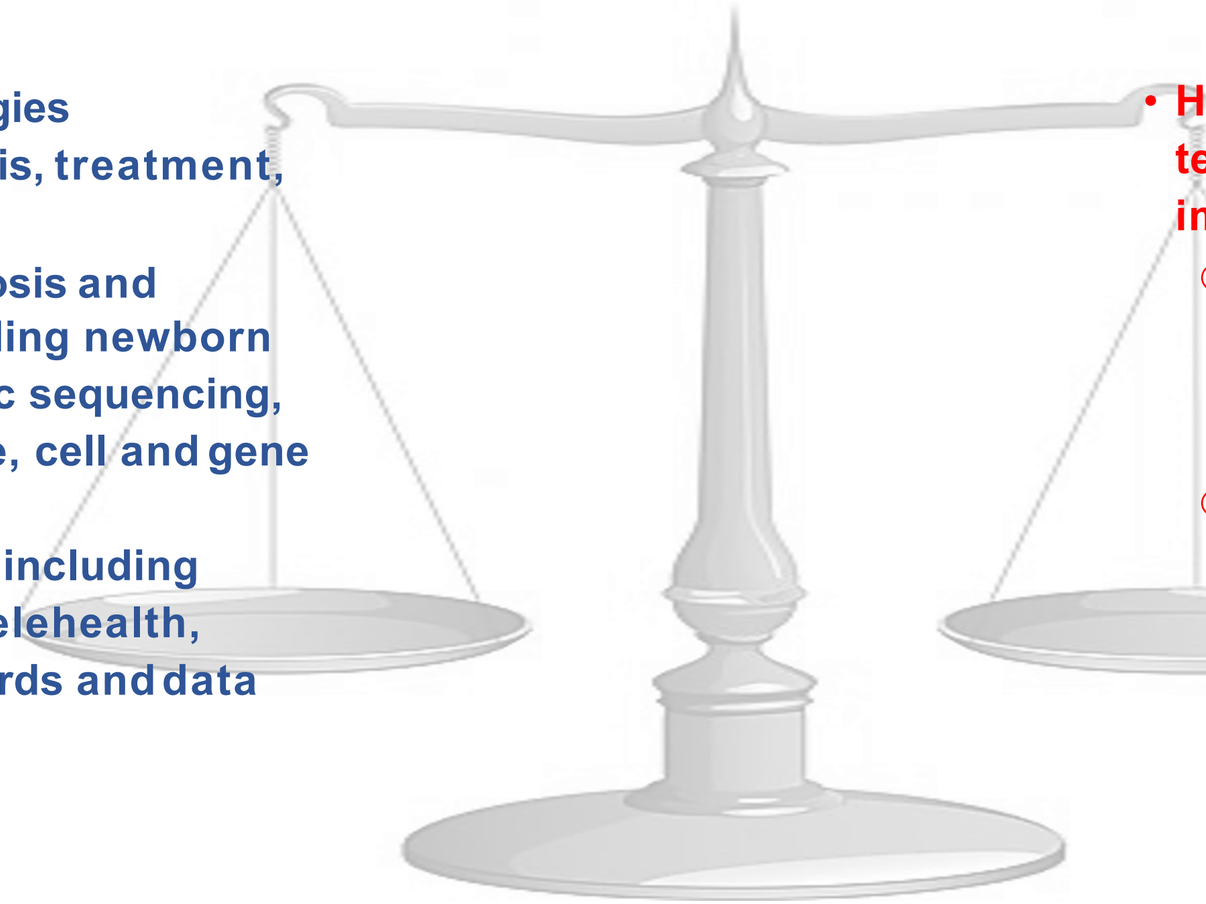
HTA success among DRDs[1,2]



Market penetration rates[3]

Abbreviations: HTA = health technology assessment. **Sources**: (1) McCormick JI, et al. Common drug review recommendations for orphan drugs in Canada: basis of recommendations and comparison with similar reviews in Quebec, Australia, Scotland and New Zealand. Orphanet J Rare Dis. 2018. Available here. (2) EVERSANA Analysis: Positive CADTH recommendations from 2018 to 2021. Data on File. (3) Schey C, et al . Estimating the budget impact of orphan medicines in Europe: 2010 – 2020. Orphanet Journal of Rare Disease. 2011. Available here.

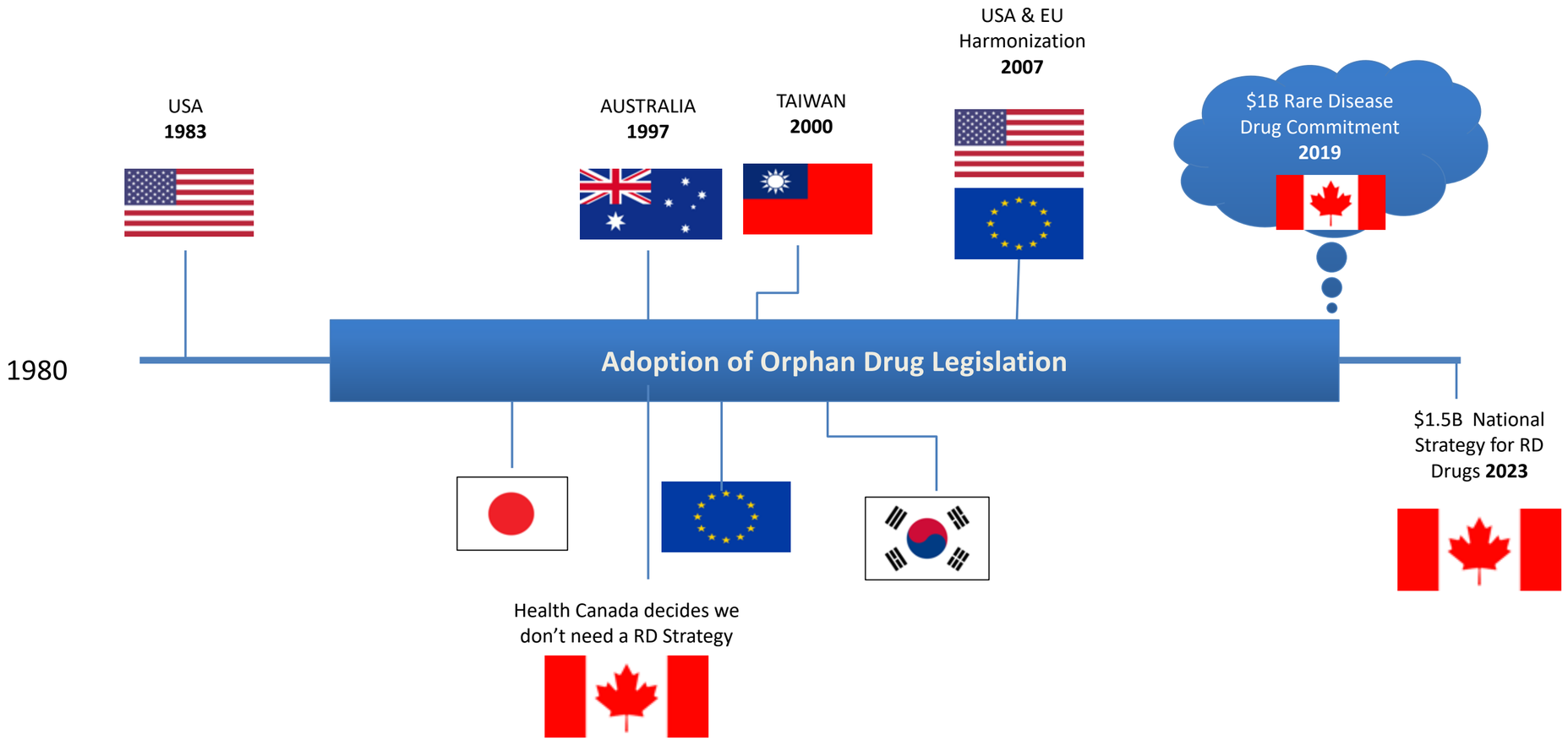
New Technology or Balanced Budgets? Or Can We Have It All?



- Innovative Technologies accelerating diagnosis, treatment, and prevention
  - Gene-based diagnosis and treatments, including newborn screening, genomic sequencing, precision medicine, cell and gene therapies
- Digital-enhanced care including wearables, e-health, telehealth, electronic health records and data registries

- Health Financing of innovative technologies challenged by budget impact
  - Investment in health fueled by technology (prevention, well-being) vs. paying for treatment driven by patient and HCP needs
  - Healthcare competing with other resource needs: among diseases, with other societal priorities, with non-health investment opportunities

Canada is already way behind – 27 years since Health Canada decided Canada doesn't need a rare disease framework because we can rely on the US and EU



Building Canadian Rare Disease Strategy: From Bottom Up … One Step at a Time

1997

Health Canada rejects need for Canadian Orphan Drug Policy

2005

CORD Position Paper calling for Canadian Orphan Drug

2005-06

Advocacy to Expand Newborn Screening in Ontario: 2 to 19 conditions

Advocacy Actions

HCPs: call for TMS across all provinces

Lawsuit: infant death

Petitions, Media, Demonstrations, Ombudsman

2006-09

Advocacy for ERTs for Fabry's/MPS; Orphan Drug Access Policy

2010-12: Government Action

- 1st FPT RD Drug Funding
- Proposal: FPT Funding for "Expensive Drugs for RD"
- Key strategy pillars released in August 2021
- OD Regulatory Framework

May 2015

CORD produces Canadian Rare Disease Strategy

2015-16: Awareness Tour

- CORD submits CA RD Disease Strategy to Parliament
- CORD hosts "x-country" RD Strategy forums to raise awareness and call for implementation

2016-18

Ontario government convenes Working Group to produce Rare Disease Strategy

Ontario RD Initiatives

- Multistakeholder WG to produce RD Strategy Implementation Plan
- Pilot RD Centre: Ehlers-Danlos Syndrome
- Rare Blood Disorders Adult Centre

Many Consultations to Call for Action!

March 2019

Federal budget includes commitment to develop rare disease drug strategy

Federal 2019 budget

$500M in annual funding for rare disease drugs, starting in

2022-23

Sep 20 – Mar 21

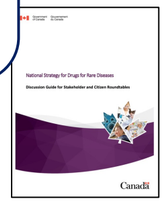
CORD 9 webinars on rare disease drug strategy

CORD Consultation Webinars

- Preventive & Risk Reduction Therapies
- Streaming Regulatory, HTA, and Reimbursement
- Individualized Therapies
- Innovative Funding

Feb – Sep 2021

Health Canada consultations on a rare disease drug strategy

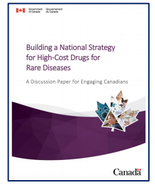


Health Canada consultations

- Townhalls & written submission opportunity
- Release of What We Heard Report by Health Canada
- Key strategy pillars released in August 2021

Spring 2022

Health Canada engagement with stakeholders



Health Canada engagement

- Release of strategy framework in April 2022
- Roundtables with stakeholders and bilateral meetings with patients (April – May 2022)

March 2023

Health Canada announces National Drugs for Rare Diseases Strategy

Health Canada funding announcement

- Investment of up to $1.5B over three years
- Majority of funding ($1.4B) will go to provinces and territories to help improve access to new and existing drugs (subject to bilateral deals)

National Strategy for Drugs for Rare Diseases announced in March 2023

$1.5 billion during the next three years

Up to $1.4 billion to provinces/territories (P/Ts) through agreements with each P/T to help improve access to new and emerging drugs, as well as support enhanced access to existing drugs, early diagnosis, and screening for rare diseases

$68 million for initiatives to improve consistent access to drugs for rare diseases

$32 million over five years to the Canadian Institutes of Health Research (CIHR) to **advance rare disease research** with a focus on developing better diagnostic tools and establishing a robust Canadian **rare disease clinical trials network**

$20 million over three years to the Canadian Agency for Drugs and Technologies in Health (CADTH) and the Canadian Institute for Health Information (CIHI) **to improve the collection and use of evidence to support decision-making**

$16 million over three years to support the establishment of **national governance structures**, such as a **Health Canada secretariat** and a **stakeholder Implementation Advisory Group**, to support the implementation of the strategy

Updates on roll-out of the national strategy

Implementation Advisory Group (IAG) created to advise federal government on implementation of the strategy

- 17 stakeholders to meet monthly with a mandate through to 2026
- First meeting held in October 2023
- Purpose is to explore options and opportunities for building out the strategy

Limited progress on bilateral funding negotiations with provinces – *ONE (1) Non-Negotiable according to Health Canada:* list of emerging therapies funded consistently

First three-year phase of the RDDS is intended as a learning period

FOUR PILLARS
Patient outcomes and sustainability
National consistency
Collection and use of evidence
Innovation

Investments to Support Access to Drugs for Rare Diseases

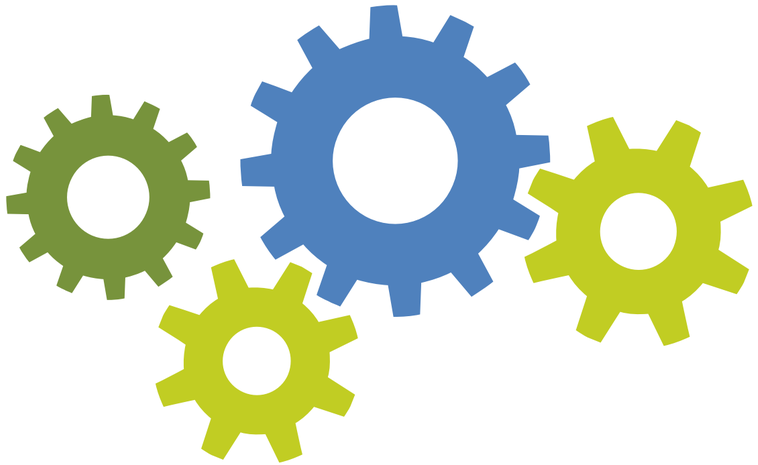
From: Health Canada

Backgrounder

Today, the Government announced a total investment of up to $1.5 billion over three years in support of the first-ever *National Strategy for Drugs for Rare Diseases* to help increase access to, and affordability of, promising and effective drugs for rare diseases to improve the health of patients across Canada.

RARE is interconnected to so many other initiatives

- National Rare Disease Drug Strategy
- Provincial rare disease policy activities
- PMPRB
- National Pharmacare
- CADTH / CDA
- Early access initiatives
- QC Patient d‘exception
- Cross-border trade
- Relevant data and research
- Canadian Rare Disease Network



Meanwhile, the rare disorders community is not waiting – patients have the right to be impatient

FIGHT FOR OUR LIVES

English

For five years, we've been promised hope, but seen little progress. We, the rare disease community, face delays in diagnosis, lack of access to vital drugs, and a system failing to recognize the urgency of our suffering. One child dies from a rare disease every 18 minutes in Canada - our lives are literally on the line.

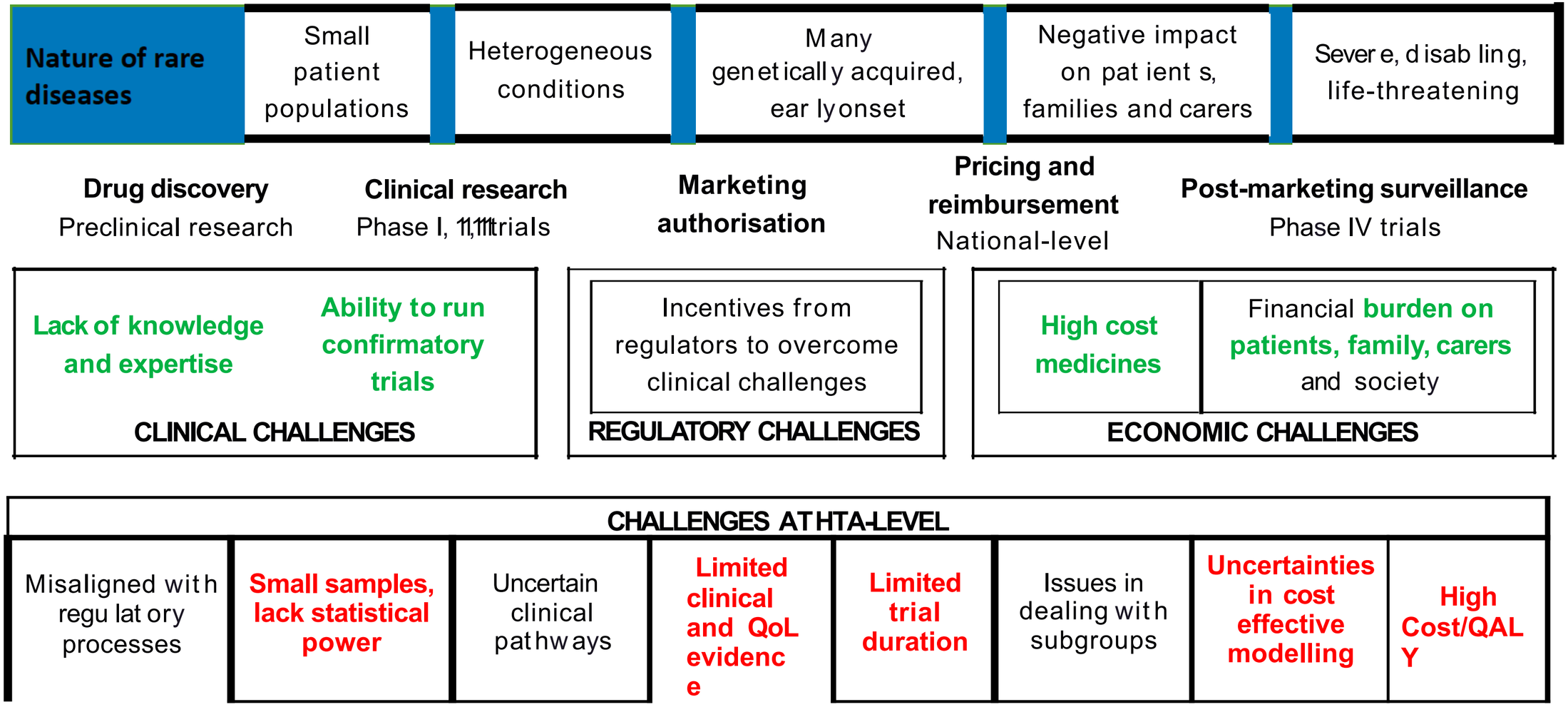
Yet, despite government inaction, we haven't given up. But we need your help. Join us in demanding action:

- **Federal government:** Release the promised funding for rare disease drugs - stop languishing in bureaucracy!
- **Provincial governments:** Follow Quebec's lead and implement rare disease strategies.
- **Everyone:** Join the **#FightForOurLives**. Share our stories, raise awareness, and hold our leaders accountable.

Together, we can turn hope into action.

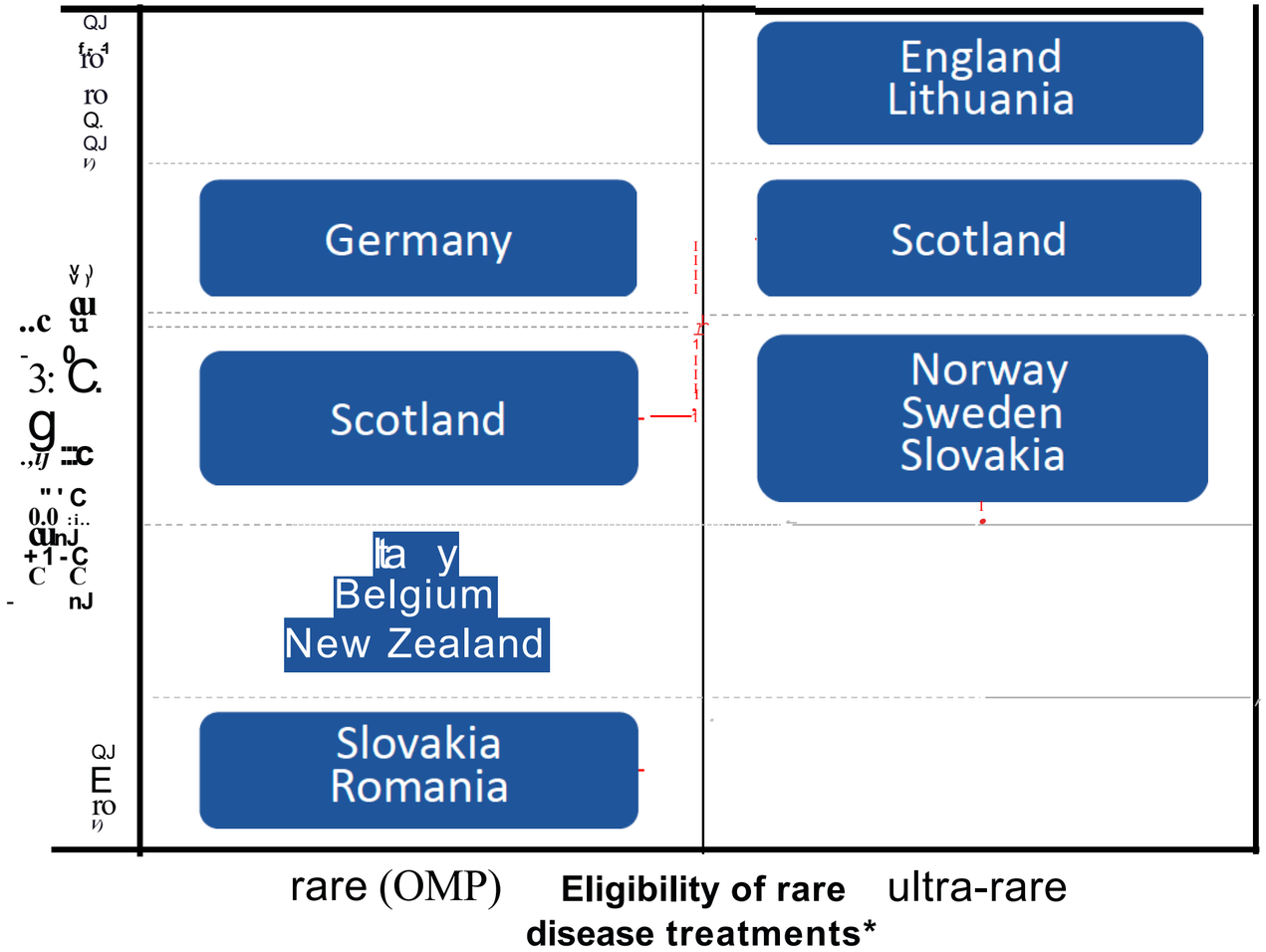
Download and read

Difficulties developing rare disease treatments leading to HTA challenges



Source: Nicod E, Annemans L, Bucsics A, Lee A, Upadhyaya S, Facey K. HTA programme response to the challenges of dealing with orphan medicinal products: Process evaluation in selected European Countries. Health Policy, 2019

13 (41%) of the 32 participating countries have some form of special HTA appraisal process informing routine use of medicines for rare diseases



Eligibility of rare disease treatments*

Separate

Partially separate

Adapted

Expedited

Rarity weighted

Exempt from HTA
Latvia, Bulgaria

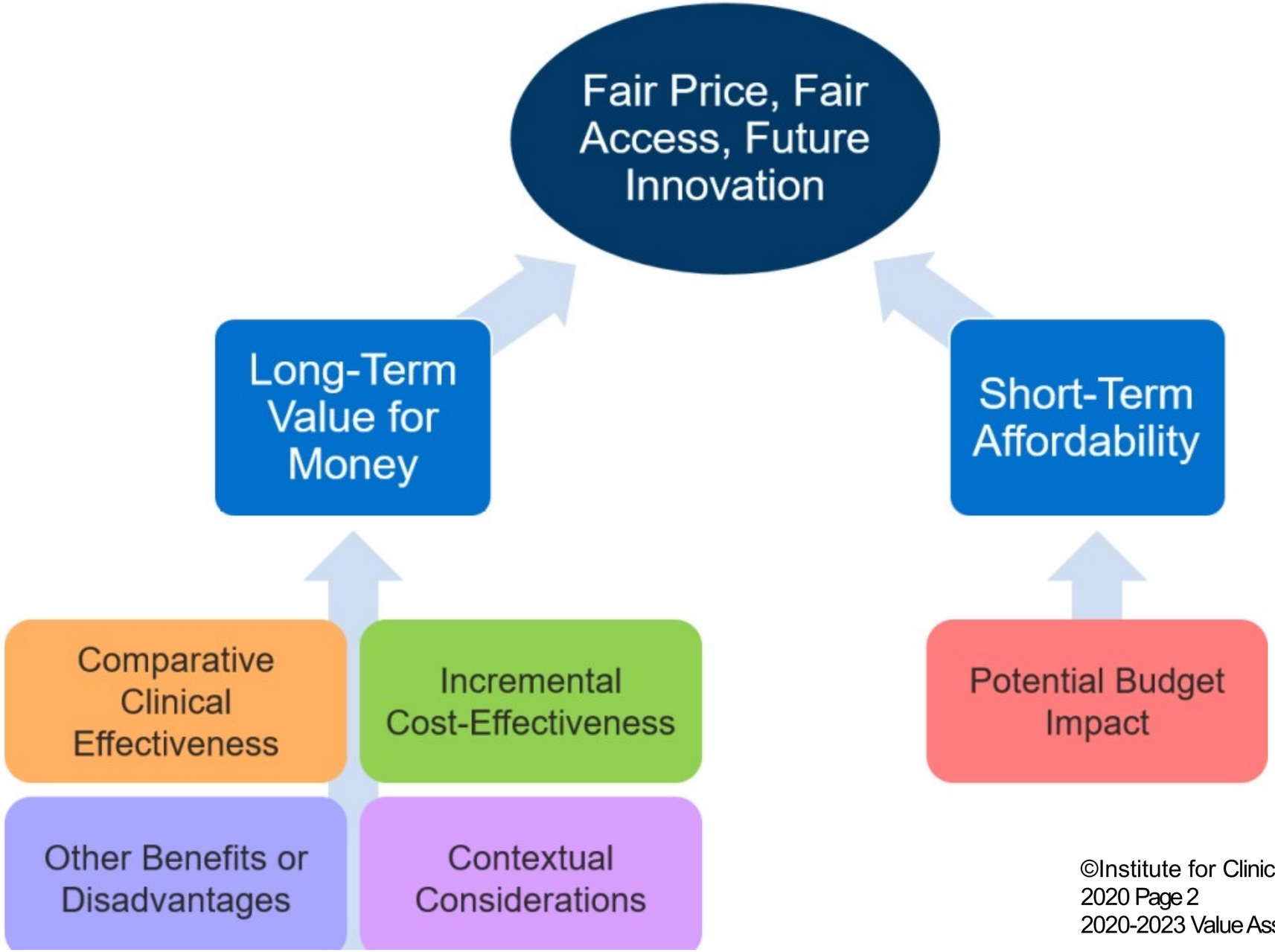
32/37 countries included:

- 41% special process
- 53% standard process
- 6% other programs applicable to rare disease treatments

* Rare disease treatment with orphan designation from European Medicines Agency ("Orphan Medicinal Product", OMP); ultra-rare disease treatments defined by individual country definitions

Source: Nicod E, Whittal A, Drummond M, Facey K. Are supplemental appraisal/ reimbursement processesneeded for rare disease treatments? An international comparison of country approaches. Orphanet Journal of Rare Diseases, 2020; 15:189

Figure 1.1. Conceptual Structure of the ICER Value Assessment Framework

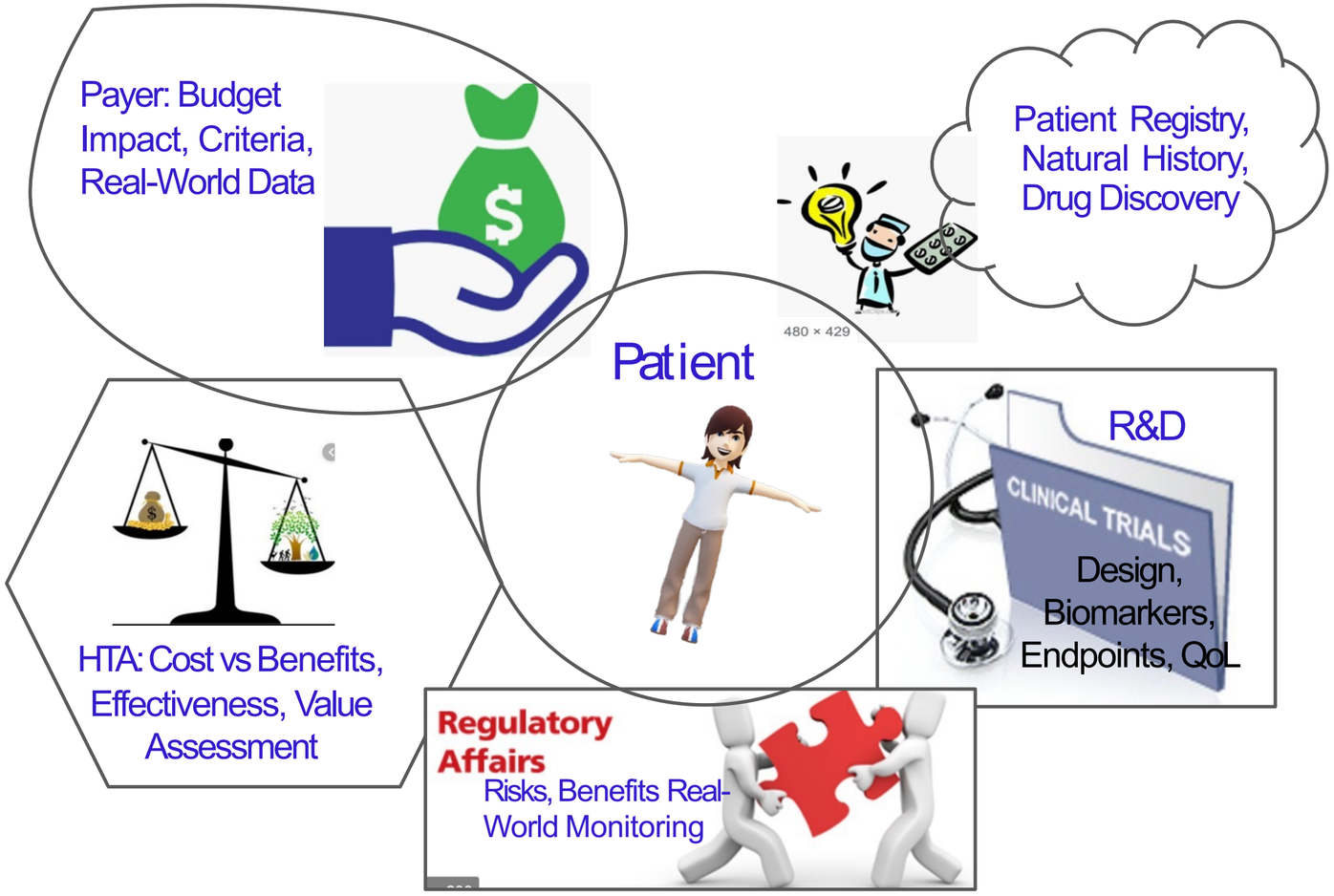


The Way Forward: A Framework for Reasonableness

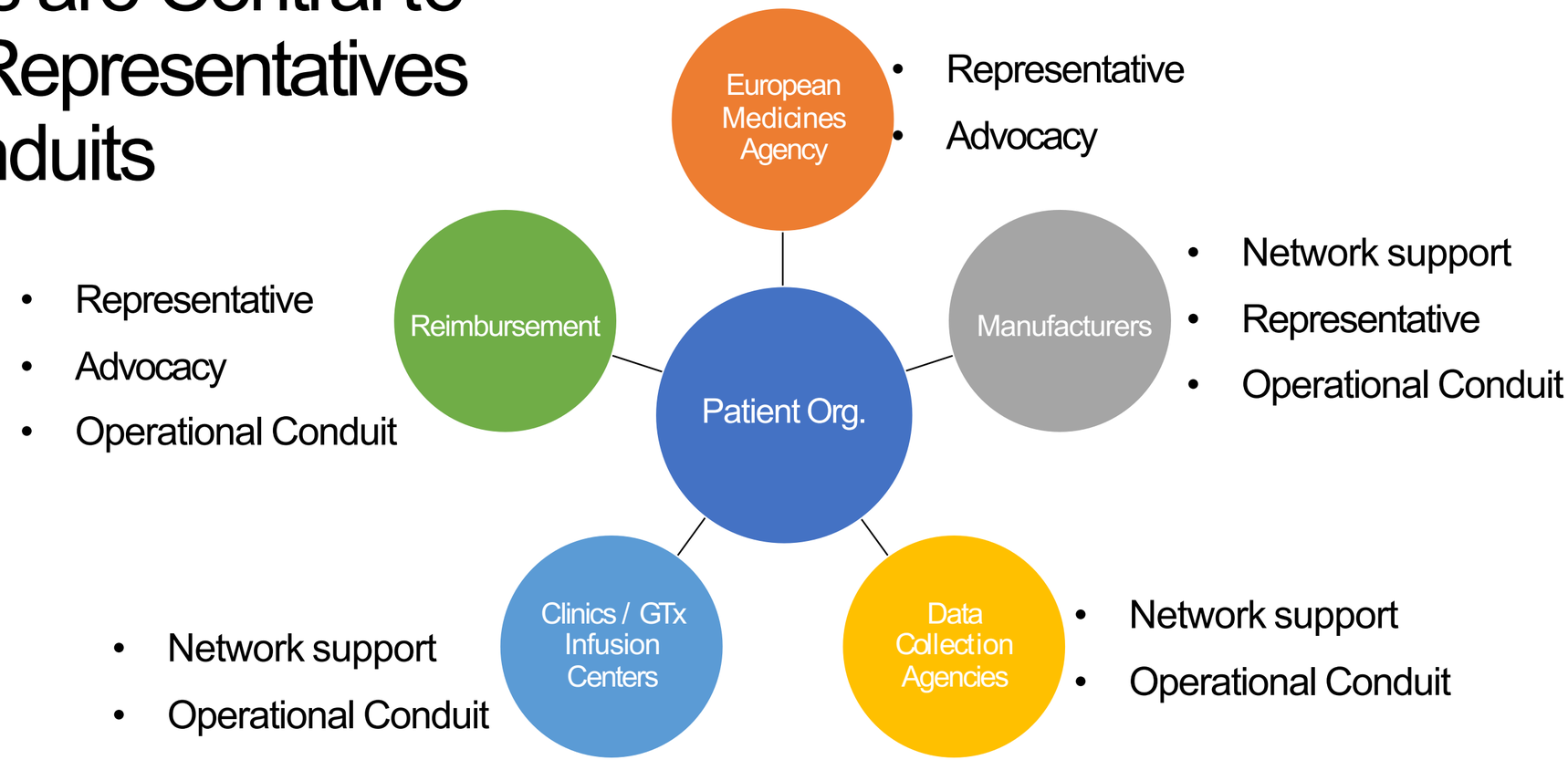
Careful for tunnel vision towards orphan vs. non-orphan drugs
Always balance ethical with more quantitative criteria

Adopt a principle of fairness behind the treatment of orphan vs. non- orphan drugs. For instance, if patient hearings are organised, do so equally for both categories

Key to Access: Patient Engagement Throughout Drug Lifecycle?



Patient Organizations are Central to serve as Networks, Representatives and Operational Conduits



Stakeholders

Clinicals | Manufacturers | Reimbursement & Regulatory bodies | Patients & Caregivers

Ideas

Patient Organizations

Partnering with patient organizations to become the central connecting point

Patient commitment to long-term follow up

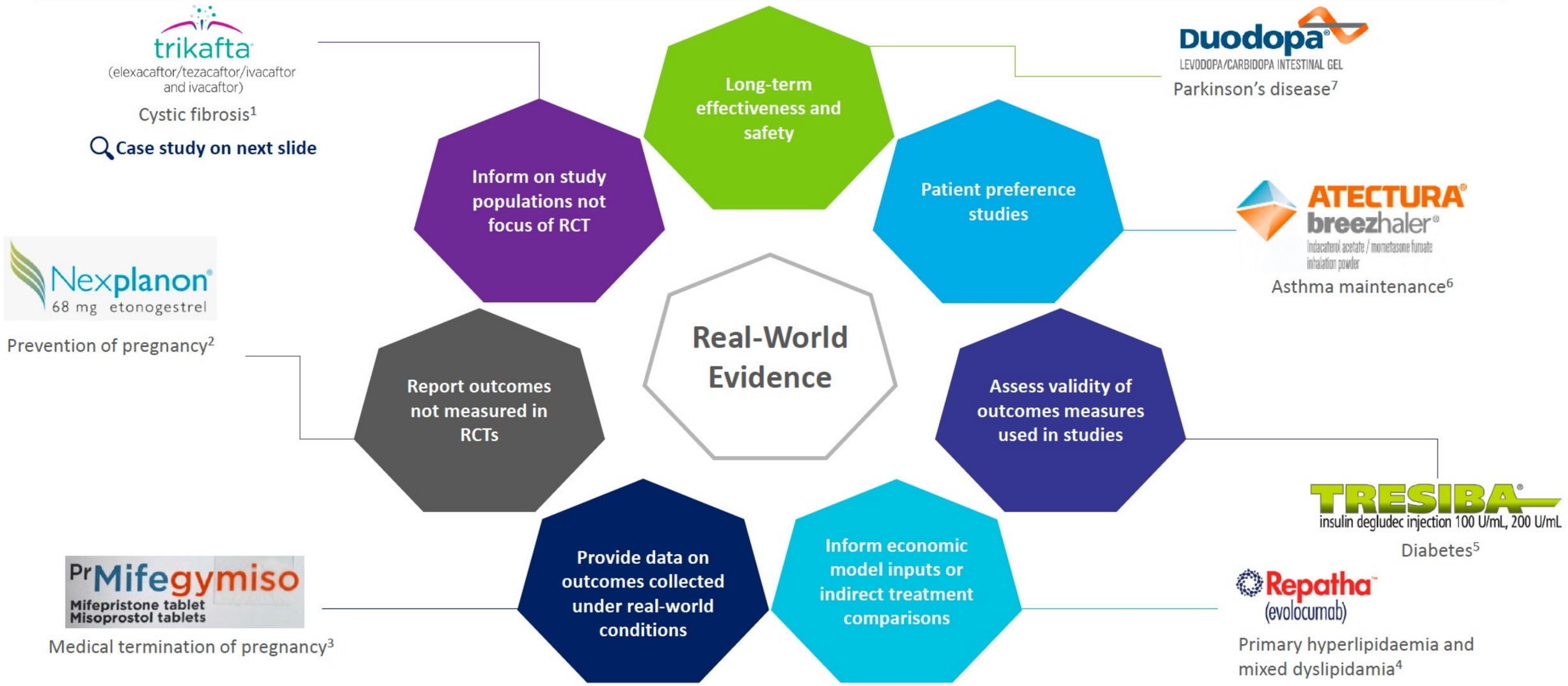
Infrastructure/care network-sharing models

Customize infrastructure/care network-sharing models where both benefit and risk is shared across involved parties

Benefits of real-world data collection and analysis throughout lifecycle

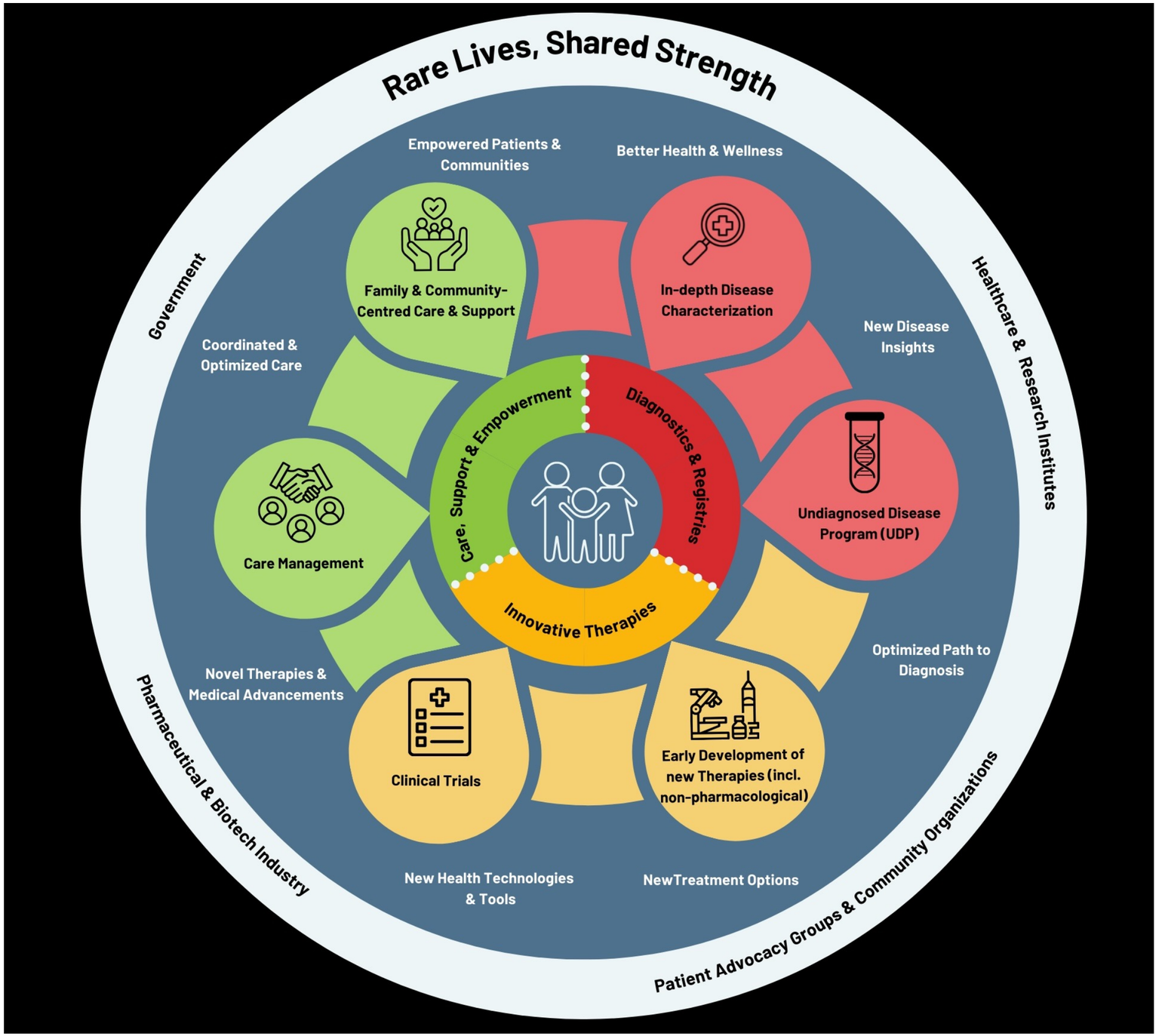
- Data on outcomes of importance to patients, including disease management and quality of life
- Monitoring in real life to know how well treatment is working, or not, across patients not included in clinical rials
- Long-term monitoring for safety and effectiveness, especially with limited historical data (on extended lifespan)
- Subgroups of patient responders (super to ineffective) and individual response guide personalized treatment decisions.
- Comparisons between different treatment options, helping patients and clinicians make more informed choices
- Contribution to knowledge pool (for others like me)
- Demonstration of value (or not)

Results: How RWE Has Been Used to Support CADTH Reimbursement Reviews



1. https://www.cadth.ca/elexacaftor-tezacaftor-ivacaftor-and-ivacaftor; 2. https://www.cadth.ca/etonogestrel-0; 3. https://www.cadth.ca/mifepristone-and-misoprostol-0; 4. https://www.cadth.ca/evolocumab-0; 5. https://www.cadth.ca/insulin-degludec; 6. https://www.cadth.ca/indacaterol-mometasone-furoate; 7. https://www.cadth.ca/levodopa-carbidopa-drug-plan-submission.

CADTH = Canadian Agency for Drugs and Technologies in Health; RCT = randomised controlled trial; RWE = real-world evidence


Rare Lives, Shared Strength
Empowered Patients & Communities
Better Health & Wellness
Family & Community-Centred Care & Support
In-depth Disease Characterization
Government
Coordinated & Optimized Care
New Disease Insights
Healthcare & Research Institutes
Care, Support & Empowerment
Diagnostics & Registries
Care Management
Undiagnosed Disease Program (UDP)
Innovative Therapies
Optimized Path to Diagnosis
Novel Therapies & Medical Advancements
Pharmaceutical & Biotech Industry
Clinical Trials
Early Development of new Therapies (incl. non-pharmacological)
New Health Technologies & Tools
NewTreatment Options
Patient Advocacy Groups & Community Organizations


CORD
Canadian Organization for Rare Disorders
CANADIAN RARE DISEASE NETWORK
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