Health Canada Santé Canada

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Health Canada's role - Drugs for rare diseases

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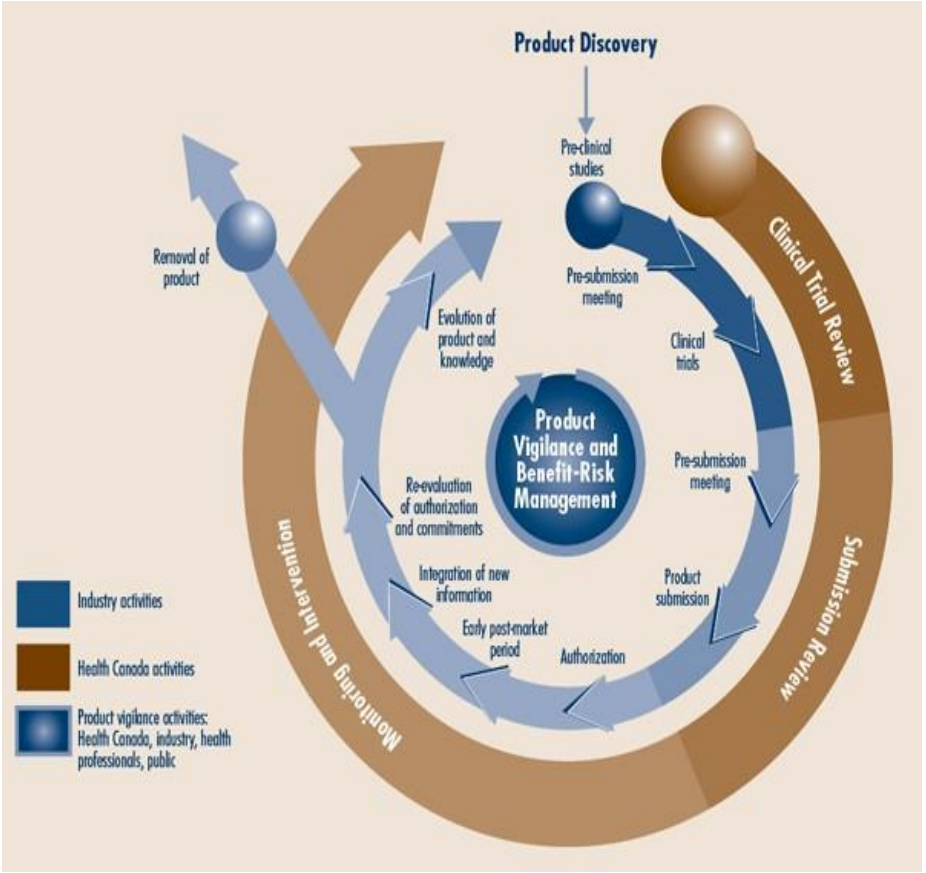
YOUR HEALTH AND SAFETY... OUR PRIORITY.

Challenges – Drugs for Rare Disease Development and Patient Access

Challenging to develop:	Technical challenges (e.g. lack of basic science, small patient populations, need for non-standard trials)
	Regulatory challenges (e.g. different international approaches) Regulation of companion diagnostics
	Business challenges (e.g. range in profitability may be significant for small populations, some lack business incentive to develop)
Once developed and authorized for market:	Can be costly and may be difficult to implement, especially outside of specialist centres.
	May be inequitable access - public and private drug plans may make different reimbursement decisions; funding recommendations may have smaller scope than HC-authorized indications based on cost-effectiveness assessment

Health Canada as Regulator

- Under the *Food and Drugs Act*, Health Canada regulates, evaluates and monitors the safety, efficacy, and quality of therapeutic products
- Includes product oversight at all stages of life cycle, from early testing in clinical trials, to post-market surveillance of adverse drug reactions, and compliance monitoring



Drug Submission Review

- Sponsors must provide sufficient evidence of the product's **safety, efficacy and quality** for assessment, including
  - Manufacturing details and controls to ensure consistent quality
  - Pre-clinical data to establish safety
  - Clinical data to support proposed indication, including “substantial evidence of effectiveness”
  - Proposed labels – claims, instructions for use
  - Assessment of possibility for name confusion

- Health Canada then reviews the evidence and decides whether the benefits of the product outweigh the risks, and whether the risks can be managed

- Health Canada continues to receive supplementary submissions after first Notice of Compliance granted, for any significant change, including:
  - New indications
  - Manufacturing changes

- **~ 30% of the new drugs authorized in 2018 are considered orphan drugs in other jurisdictions**

Pathways to Drug Access

Regulatory authorization:

New Drug Submission/Supplementary New Drug Submission – review for safety, quality efficacy

- Target review time – 300 days [similar review for generics = 180 days]

Priority review policy

- Unmet medical need, serious, life-threatening or severely debilitating disease
- Requirement for “substantial” evidence of effectiveness
- Target review time – 180 days [In 2018, 4 drugs for rare diseases were reviewed under this pathway]

Notice of Compliance with Conditions policy

- Unmet medical need, serious, life-threatening or severely debilitating disease
- “Promising” evidence of effectiveness
- Target review time – 200 days [In 2018, 1 drug for a rare disease was reviewed under this pathway]
- Manufacturer agrees to conduct confirmatory studies

Other Access:

Special Access Programme

- Access to drugs which are not authorized in Canada, when other treatments exhausted or not possible
- Initiated by prescriber for individual patients

Post-market Monitoring and Surveillance

- Once a product has been marketed in Canada, there are ongoing obligations for sponsors, including:
  - Reporting of adverse drug reactions (ADRs)
  - Preparing annual summary reports on safety
  - Managing any changes in the benefit-risk profile

- Health Canada conducts ongoing analysis of ADRs, environmental scanning, ongoing assessment of potential safety issues
  - As needed, issues risk communications to inform Canadians and prescribers of changes
  - Updates product labelling to ensure accuracy and to support safe use
  - If benefit-risk no longer favourable, products can be withdrawn from market
  - Relies on many sources of data, including clinical trials, individual case reports, and real world evidence

Transforming how we regulate

Objective: An agile regulatory system that supports better access to therapeutic products based on healthcare system needs

Expanded collaboration with health partners

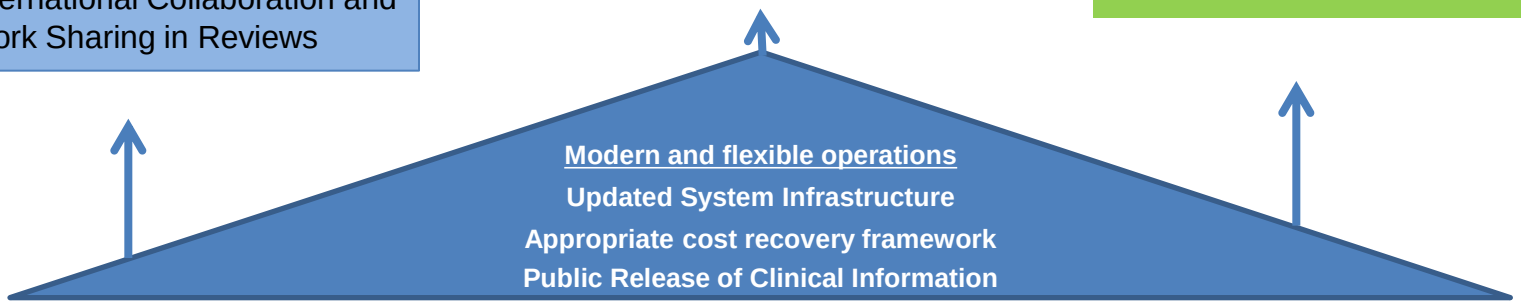
- Alignment of the Health Technology Assessment (CADTH) Review with Health Canada Review
- Implementing a Mechanism for Early Parallel Scientific Advice
- Use of Foreign Reviews/Decisions
- International Collaboration and Work Sharing in Reviews

More timely access to drugs and devices

- Expansion of Priority Review Pathways
- Improving Access to Biosimilars and Biologics
- Improving Access to Generic Drugs
- Building Better Access to Digital Health Technologies
- Pre-Submission Scientific Advice for Medical Devices
- Special Access Programme (SAP) Renewal and Block Release

Enhanced use of real world evidence

- Leveraging Data for Assessing Drug Safety and Effectiveness
- Strengthening the use of real world evidence and regulations for medical devices



R2D2 – Some Accomplishments to Date

Expanded collaboration

- ✓ Established mechanisms for aligned regulatory and health technology assessment reviews, with potential to save overall time to market
  - ✓ **19** aligned reviews completed, **9** ongoing, ~75% are new drugs
- ✓ Created processes to share submission review with other regulators
  - ✓ **2** reviews completed, more ongoing (partnering with Australia)

More timely availability of drugs and devices

- ✓ Draft guidance document describing revised criteria for accelerated review, which can include drugs for rare diseases
- ✓ Ongoing operational updates to the Special Access Program to reduce burden on prescribers

Expanded Use of Real World Evidence

- ✓ Providing guidance on quality of evidence needed to support regulatory decision-making and encouraging submissions using RWE

Modern and Flexible Operations

- ✓ Final publication of regulations supporting release of clinical information from drug submissions

What's Next?

Interim report on implementation of national pharmacare:

"The Council also recognizes the unique challenges of funding and accessing expensive drugs for rare diseases. Special consideration is needed to determine how to address these challenges to ensure a nationally consistent approach for these medications."

Budget 2019 commitments:

- Creation of a "Canadian drug agency" - assessment, pricing negotiation, national formulary
- National strategy for EDRDs – up to $1B over 2 years in 2022-23, $500M ongoing

Creating flexibility in the regulatory system to support innovation in bio-sciences:

- Budget Implementation Act includes new pathway for "advanced therapeutic products"
  - Creation of "regulatory sandboxes" to support innovative products that don't fit well into existing regulatory frameworks
- Modernizing clinical trial regulation
  - To become more risk-based and agile