Health Canada Santé Canada

Canada

Health Canada Regulatory Innovation: Implications for Rare Disease Therapies

CORD Conference – Canada's Rare Disease Drug Landscape

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YOUR HEALTH AND SAFETY... OUR PRIORITY.

Health Canada is a World Class Regulator in a Globalized Landscape

We're the critical first step in the decision-making system that determines if a product is made available to Canadians:

- **Health Canada:** *Is the drug safe, effective, and of high quality? Should it be sold in Canada?*
- **Health Technology Assessors (CADTH, INESS):** *Does the drug offer value for money?*
- **Patented Medicine Prices Review Board (PMPRB):** *What's the maximum allowable price?*
- **Pan-Canadian Pharmaceutical Alliance:** *Can we negotiate a better price?*
- **Provincial Drug Plans:** *Will we cover this drug for patients?*

Pharmaceutical sales in Canada have a 2.1% share of the global market, making Canada the 9th largest world market.

- The health and biosciences sector is composed of many globally interconnected companies developing innovative and generic medicines and medical devices
- We work with many international regulators to share information, harmonize standards and align regulatory approaches to attract more submissions to Canada

FDA U.S. FOOD & DRUG ADMINISTRATION

ICH harmonisation for better health

ICMRA

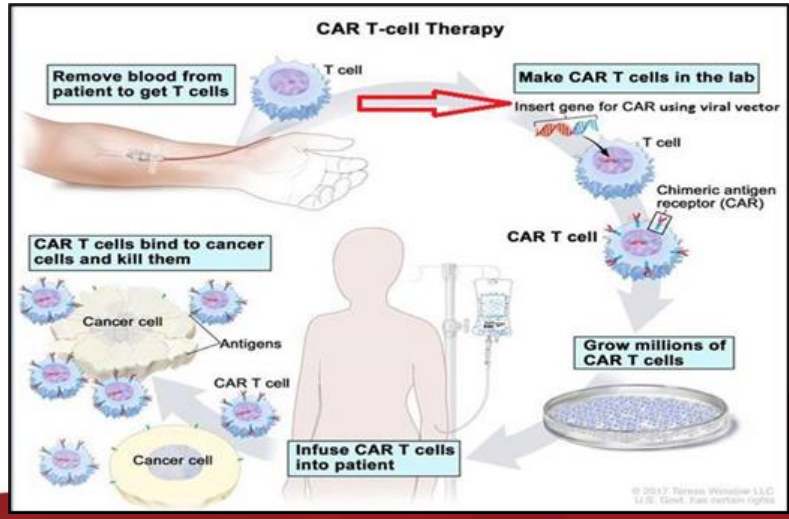
EUROPEAN MEDICINES AGENCY SCIENCE MEDICINES HEALTH

IMDRF International Medical Device Regulators Forum

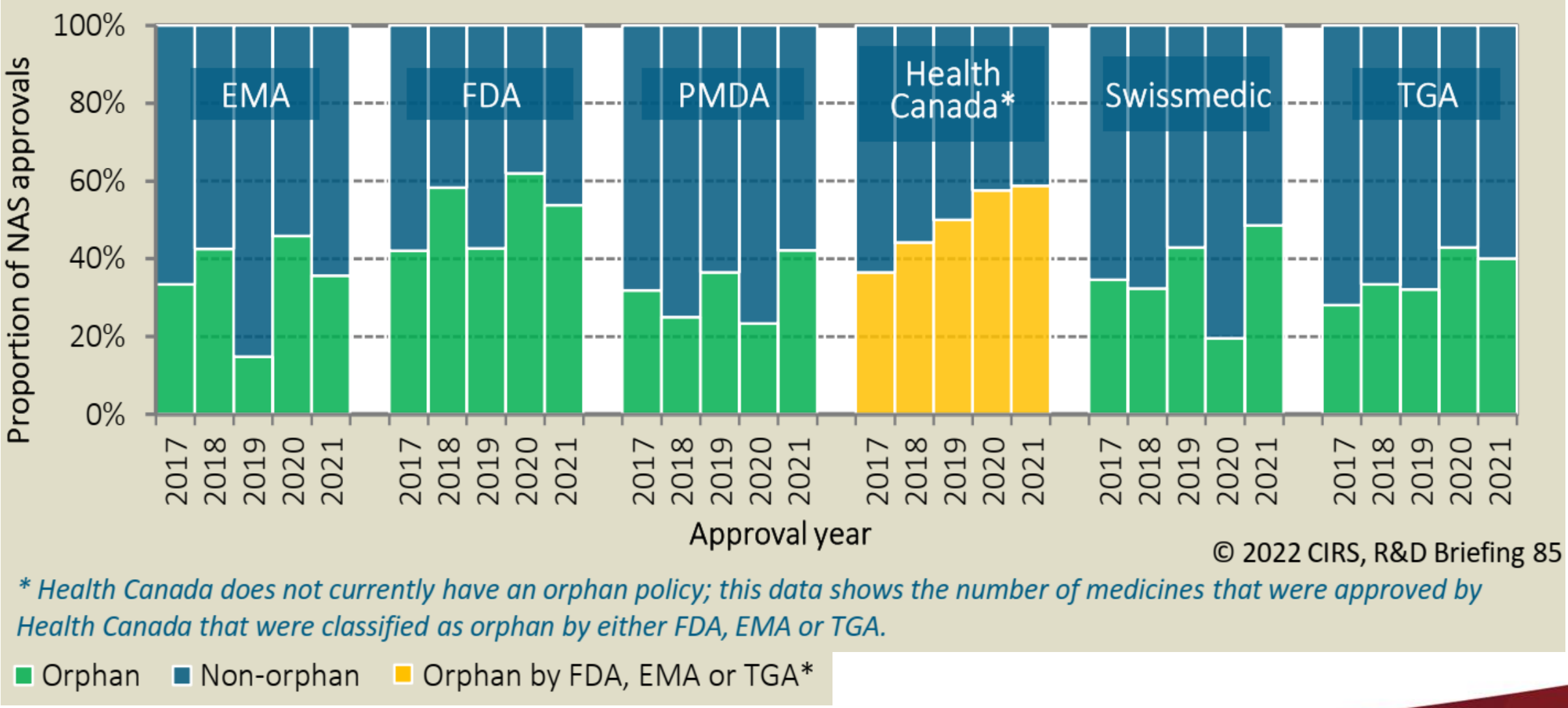
ACCESS CONSORTIUM AUSTRALIA CANADA SINGAPORE SWITZERLAND UNITED KINGDOM

Trends show a clear growth of complex and personalized products seeking approval from Health Canada

- Over the past 7 years, over 40% of new active substances (NAS) authorized in Canada are classified as orphan drugs in Europe or the United States
  - In the next 3 years, 60% of cancer drug submissions anticipated to be for rare diseases
- As of October 2023, 29 New Active Substances were approved in 2023.
  - Over a third of these new drugs were for rare diseases
- Health Canada has also approved a number of innovative cell and gene therapies, such as:
  - **CAR T-cell therapies** where a patient's own T cells are altered in a laboratory and then re-infused to attack specific cancer cells
  - **Targeted gene therapies** such as one to treat spinal muscular atrophy, a rare disease and a leading genetic cause of infant mortality



Proportion of NAS Approvals by Orphan Designation (2017-2021)



*The Centre for Innovation in Regulatory Sciences: Briefing 85 New drug approvals in six major authorities 2012-2021

To support access to drugs for rare diseases, Health Canada offers…

Pre-Submission Meetings

- Scientific advice to sponsors on the design of clinical trials in small populations and to support companies' tailored drug development programs

Accelerated Review Pathways

1) Priority Review of Drug Submissions Policy **(180 days)**
    - Requirement for **substantial** evidence of effectiveness

2) Notice of Compliance with Conditions Policy **(200 days)**
    - **Promising** evidence of effectiveness
    - Manufacturer agrees to conduct confirmatory studies for efficacy

Optional Pathways

- Aligned review with the Health Technology Assessment
- Use of Foreign Reviews

To support access to drugs for rare diseases, Health Canada offers…

Incentives

- Data protection in Canada gives 8 years of market exclusivity for innovative drugs; a six-month pediatric extension is also possible where relevant requirements are met
- Fee mitigation or fee deferral are possible to reduce the potential negative impact of regulatory fees

Regulatory Modernization and International Harmonization

- Health Canada works extensively with international partners (such as ICH) to achieve greater regulatory harmonization worldwide
- Health Canada is actively modernizing our regulatory framework to support international harmonization and contribute to improving access

Special Access Program (SAP)

- Health Canada can consider and grant requests for access to drugs that are unavailable for sale in Canada, which are received from practitioners treating patients with serious or life-threatening conditions when conventional treatments have failed, are unsuitable or unavailable

Modernization Plan to Enhance Regulatory Agility

- Health Canada's modernization plan aims to provide more regulatory flexibility to support innovative research and health product development
- This will also help regulate the risks, benefits and uncertainties of more diverse and complex products, and ensure we have the agility to respond to changes in the biomanufacturing and life sciences sector

Modernizing clinical trial regulations

Enabling Advanced Therapeutic Products

Agile licensing for drugs

Agile licensing for medical devices

Supported by a modernized compliance and enforcement program

Web Link: ***Regulatory Innovation for Health Products***

Modernizing Clinical Trial Regulations

To encourage clinical trials in Canada by creating an environment that supports safe innovation

Legislative changes made in 2019 enable us to make future regulations across product lines to:

- Authorize and oversee a broader range of clinical trials, their conduct, as well as the sale of product(s) within the trial.
  - Enable more streamlined, flexible approaches, oversight over complex trial designs, decentralized trials, investigator-led device trials, and food for a special dietary purpose trials, while continuing to prioritize patient safety.
- Tailor requirements and oversight to the risk of the trial/product(s).
  - Lighten requirements for studies of new uses for approved drugs/natural health products;
  - Add terms and conditions as additional tool to manage risks/uncertainties across the lifecycle of a trial to ensure participant safety.
- Require disclosure of trial information and results in a publicly accessible registry.

Through Clinical Trial Modernization, Health Canada envisions future regulations that align across product lines and internationally, where appropriate, while adapting to better accommodate emerging trends

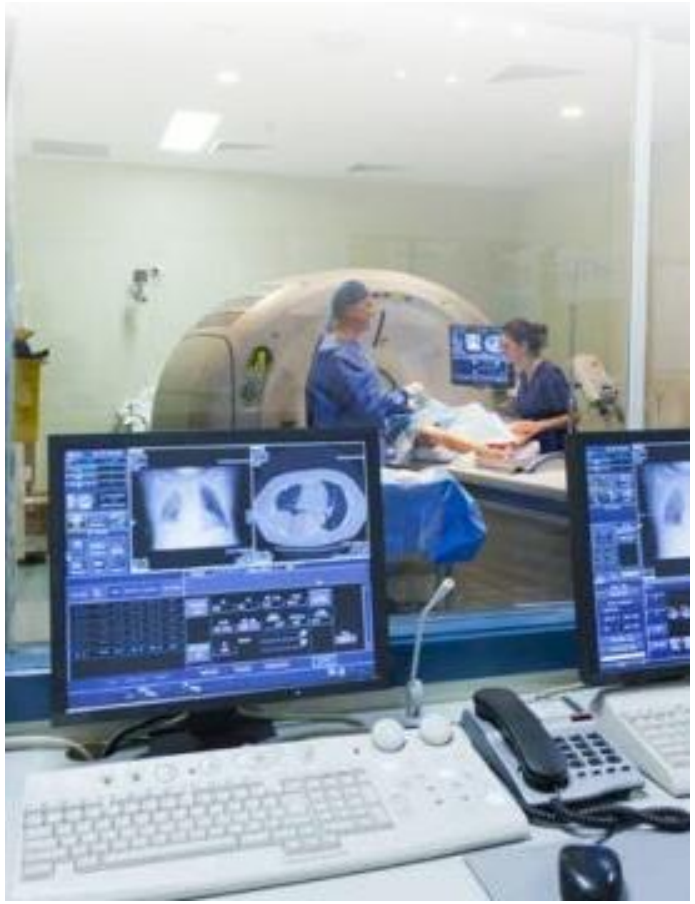
Advanced Therapeutic Products (ATP) Framework

To ensure a flexible approach for innovative drugs and medical devices that challenge the current regulatory system

Advanced Therapeutic Products (ATPs) are drugs and/or devices so unique, complex and distinct that Canada's existing regulatory frameworks and enforcement tools are not equipped to handle them

- Changes to Canada's *Food and Drugs Act* have enabled Health Canada to create a legislative pathway to authorize ATPs
- The use of tailored requirements will address a product's unique characteristics while maintaining Health Canada's high standards for patient safety
- A collaborative and iterative approach with a wide variety of stakeholders, both upfront and throughout, will be used for the implementation of the ATP pathway

Advancing Agile Licensing for Drugs and Devices



In December 2022, Health Canada proposed amendments to the *Food and Drug Regulations* (and *Medical Device Regulations)* based on agilities tested through COVID-19 response and addressing long-standing industry irritants, including:

- Terms and conditions on the drug identification number of any drug
- Risk Management Plans to manage risks and uncertainties
- Rolling reviews for certain drugs
- Disaggregated data as submitted to the EMA or USFDA

Health Canada is reviewing stakeholder comments to determine any updates to be made to the regulatory provisions and guidance documents

International Collaboration: Improved Access to Medicines for Patients

- Health Canada continues to work closely with international partners on a coordinated and aligned approach to regulating medicines
- Multi-national fora coordinate international strategy and guidance
  - **International Coalition for Medicines Regulatory Authorities (ICMRA)** plays a leadership role in aligning policy approaches and regulatory agility (Joint Statements)
  - **World Health Organization** and the **Pan American Health Organization**
- Health Canada also work multilaterally to align and coordinate product specific regulatory work
  - **Parallel reviews and information sharing** to maximize the use of global expertise
    - ❑ Rare Disease Cluster Meetings
    - ❑ European Medicines Agency (i.e. EMA OPEN)
    - ❑ Access consortium – Australia, Canada, Singapore, Switzerland, UK
    - ❑ US Food and Drug Administration (i.e. Project Orbis)
  - **Post-market monitoring** to share international data on potential safety issues

Questions?

Please contact us!

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