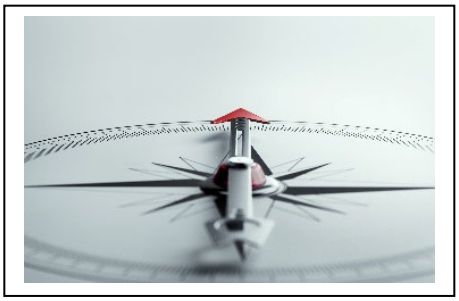
Navigating Health Canada's Drug Regulatory Pathways



At the Frontiers: Advanced Cell and Gene Therapies

Advanced cell and gene therapies are regulated as biologic drugs under the *Food and Drugs Act* and the *Food and Drug Regulations* (FDR). Health Canada has authorized a variety of novel advanced cell and gene therapy products under the FDR, the majority through accelerated pathways. There are numerous advanced cell and gene therapy clinical trials authorized in Canada.

Advanced cell and gene therapies are often personalized or made in small batches, use complex manufacturing, and may have side effects that emerge months or years later (such as prolonged immune effects or rare secondary cancers), requiring long-term patient follow-up. Health Canada supports the development of advanced cell and gene therapies by providing regulatory guidance documents and working with researchers and manufacturers to navigate the regulatory framework. Early engagement to support evidence generation is recommended.

Health Canada Supports for Advanced Cell and Gene Therapies

Guidance Documents

- Regulatory roadmap for biologic (Schedule D) drugs in Canada
- Guidance Document: Preparation of Clinical Trial Applications for use of Cell Therapy Products in Humans
- Health Canada Policy Position Paper – Autologous Cell Therapy Products
- Guidance Document For Clinical Trial Sponsors: Clinical Trial Applications
- Overview: Guidance on management of drug submissions and applications
- Health Canada's webinar materials *Regulatory '101': Manufacturing of CAR T-Cell and Other Advanced Cell & Gene Therapies in Decentralized Settings*

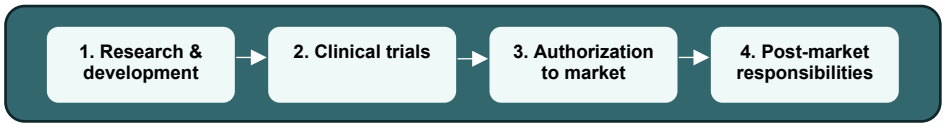
Stakeholder Engagement and Product Classification

- Early engagement: Health Canada strongly encourages cell and gene therapy sponsors to request pre-submission meetings prior to seeking clinical trial or market authorization. These meetings are available at no cost to the sponsor. Contact: brdd.ora@hc-sc.gc.ca
- The Office of Science can assist with the classification of products. Contact: drug.device.classification-drogue.instrument@hc-sc.gc.ca
- Health Canada regularly participates in conferences, workshops, and other outreach activities to facilitate dialogue and gather input from stakeholders across the cell and gene therapy community. These events provide opportunities for industry, academia, and regulators to discuss challenges and share best practices.

International Harmonization

- Health Canada actively participates in international harmonization efforts including the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH), the International Pharmaceutical Regulators Programme (IPRP), Advanced Therapy Medicinal Products (ATMP) Cluster Meetings, and the Access Consortium.
- Health Canada's regulatory requirements for advanced cell and gene therapies under the FDR are consistent with regulatory approaches in the United-States where these therapies are regulated under the biological products framework and in the European Union where they are regulated as ATMPs.
- Other agencies such as the US Food and Drug Administration (US FDA) and European Medicines Agency (EMA) also provide guidelines relevant to advanced cell and gene therapies.

From Bench to Beyond: Charting the Full Journey



1. Research and early development

Key considerations in early product development include:

- Identify a clear biological target and therapeutic rationale
- Generate pre-clinical data to support initial safety and feasibility
- Design a manufacturing approach that can progress toward full Good Manufacturing Practices (GMP) as development advances

2. Clinical trials

Health Canada authorization is required before testing a product in humans in Canada.

Clinical Trial Application (CTA)

- **Purpose:** to protect the safety of the people participating in the clinical trial, to ensure that the best interests of trial participants have been considered, and to make sure that the goals of the trial can be met.
- **Timeline for review:** 30 days. If Health Canada raises no objections, the clinical trial may begin.
- **What's required:** submission of several documents is required: study protocol, investigator and site information, manufacturing and quality information, and preclinical safety data.

For select patients or products, standard clinical trials may not always be suitable. In these exceptional circumstances, additional options are available to address specific clinical needs while maintaining appropriate oversight:

- **Special Access Program (SAP):** Allows practitioners to request access to unauthorized therapies when other treatments have failed or are unavailable
- **Expanded access clinical trials:** Provide access for patients who cannot join other trials
- **Open label individual patient trials:** Designed for a single patient with a serious or life-threatening condition

3. Authorization to market

New Drug Submission (NDS)

- **Purpose:** to sell or supply a new drug in Canada, including use within hospitals.
- **Timeline for review:** Screening takes about 45 days; full review takes about 300 days.
- **Faster review options may be available** for drugs that address **serious or life-threatening conditions**, especially where there is an unmet medical need:
  - **Priority Review:** Shortens the review timeline for drugs that show **significant improvement** in safety or effectiveness
  - **Notice of Compliance with Conditions (NOC/c):** Allows earlier approval based on **promising evidence**, with additional data to be provided after approval

  These pathways can support **earlier access** to important therapies while continuing to gather evidence on safety and effectiveness.
- **What's required:** Clinical and non-clinical evidence, manufacturing and quality data, and proposed labelling.
- **Outcomes:** Health Canada may approve (including conditional approval), request more information, or reject the NDS. Approval results in a Notice of Compliance (NOC) and Drug Identification Number (DIN).
- **Supplemental New Drug Submission (SNDS)** is required when a significant change is made to an already approved drug, such as adding a new use, changing the dose, or updating manufacturing methods.

4. After approval: Post-market Responsibilities

After approval, post-market activities help ensure that a product continues to be safe, effective, and of high quality when used in real-world settings. These activities are important because some risks may only become apparent over time or with broader use. Post-market oversight includes:

- Reporting serious adverse reactions and notifying us of foreign actions involving a serious risk of injury to human health
- Maintaining GMP compliance
- Completing comprehensive assessments of all known safety information through the preparation of annual summary reports and notifying us without delay if there is a significant change in what is known about the risks and benefits
- Keeping labelling and advertising accurate and up to date
- Submitting and updating Risk Management Plans, where applicable
- Conducting Lot Release activities, where applicable (using a risk-based approach)

Pioneering Pathways: Advanced Therapeutic Products (ATP)

If products are identified that are so novel they face a significant barrier under the existing regulations, Health Canada's Advanced Therapeutic Products (ATP) framework may provide flexible, tailored oversight for these cases.

A product may be an ATP if it:

- Uses a highly novel approach that does not fit existing regulatory pathways
- Requires a tailored evidence or oversight approach

Steps for proceeding:

- Initiate early engagement with Health Canada to confirm the regulatory pathway and oversight approach. Contact: atp-ptn@hc-sc.gc.ca
- Complete the ATP Candidate Identification Form.

Fees Demystified: Costs and Reductions

Health Canada charges fees for submissions, establishment licences, and the right to sell a drug.

What to know:

- There are no fees for Clinical Trial Applications (CTAs).
- Fees for New Drug Submissions (NDS) vary depending on submission type, review pathway, and eligibility for fee reductions. They typically ranges from approximately $300K to $500K+, with reductions available, including:
  - Publicly funded healthcare institutions are exempt from fees
  - Small and medium-sized enterprises (SMEs) who may qualify for significant fee reductions

Recommended actions:

- Eligibility for exemptions should be assessed early in the process
- An SME Attestation Form is to be submitted alongside the application
- For questions, contact: osip-bppi@hc-sc.gc.ca

Regulatory Modernization Underway

The Government of Canada is undertaking a system-wide effort to modernize federal regulations, reduce administrative burden and support innovation and economic growth. As a part of the Red Tape Review commitments, Health Canada is advancing several initiatives aimed at providing a more efficient, responsive, and internationally aligned regulatory system that supports timely patient access.

Ministerial Reliance Order

The order allows Health Canada to rely on decisions or documents produced by comparable Foreign Regulatory Authorities (FRAs) to facilitate more efficient review of drug submissions that have already been approved or evaluated by a comparable FRA.

What this looks like:

- The Order "deems" that the requirement for Health Canada to review certain parts of a drug submission is complete if the requirements under the Order are met. Decision to deem is based on the fact that the comparable FRA has either:
  - given approval for the foreign drug or
  - produced a document based on its review of a certain portion of the foreign drug submission
- The Order includes an Incorporation by Reference (IbR) list that sets out classes of drugs paired with certain comparable FRAs within its scope

For developers, this would mean reduced review timelines for drugs within scope of the Order and increasing the number of drugs in Canada.

Clinical Trials modernization

Health Canada is modernizing the existing clinical trials regulations to improve access to new and innovative therapies for Canadians, while maintaining strong safety protections for trial participants.

What's changing:

- Direct regulation of clinical trial conduct
- More flexibility for complex trial designs (e.g., adaptive, master protocol and multiphase trials)
- Proportional risk-based oversight to reduce burden — including terms & conditions on authorizations
- Improved patient access to clinical trials by enabling decentralization (electronic and oral consent as well expanded investigator types)

These changes aim to make it easier to run complex trials and support earlier access to new treatments.

Access Consortium

Health Canada works with four trusted international regulators through the Access Consortium enhancing cooperation to streamline procedures and improve access to safe, effective drugs.

What this looks like:

- Work-sharing initiative where regulators conduct joint reviews
- Participating countries: Australia, Singapore, Switzerland and the UK
- Enhances alignment on evidence requirements and regulatory approaches

For developers, this can reduce duplication and regulatory burden while offering the possibility of accessing multiple markets simultaneously.