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Health Canada Bringing Innovation to the Regulation of Advanced Therapeutic Products

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American Society for Cell and Gene Therapy - Policy Summit

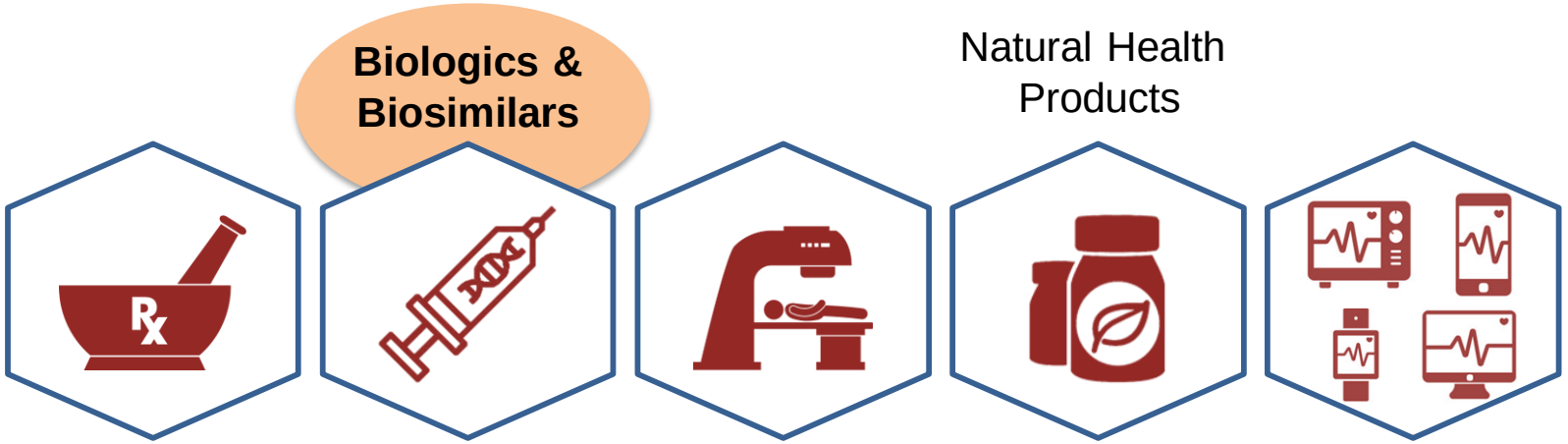
Developments in Gene Therapy Policy Landscape

November, 2019

YOUR HEALTH AND SAFETY... OUR PRIORITY.

Health Canada and the regulation of health products

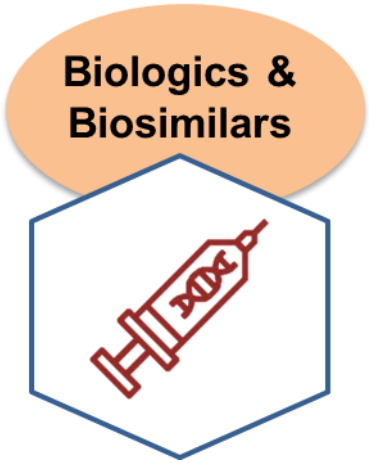
What we regulate



How we regulate

- Clinical Trials
- Product submission review and market authorization
- Post-market surveillance

The regulation of biologic drugs – advanced cell therapies



The Food and Drugs Act

The Food and Drug Regulations

Safety of Human Cells, Tissues and Organs for Transplantation Regulations (CTO)

Cells considered "drugs"
Requirement of pre-market approval; establishment licence; good manufacturing practices; lot release testing; and supporting evidence of safety, quality and efficacy

Investigational cells
Requirement of authorization to perform clinical trial

Cells for transplantation
Requirement to certify the establishment is in compliance and that the cells are safe for transplantation

New Drug Submission

Clinical Trial Application

Establishment Registration

Gene therapies at Health Canada at a glance

- 1994 - First clinical trial for a gene therapy is authorized (for intra-tumoral injection of a plasmid).
- 1994 to present – No Objection Letters issued to some 130 clinical trials for gene therapies.
- 2018 – Kymriah (tisagenlucleucel) CAR-T, an autologous cell-based gene therapy, is authorized for general distribution for two indications through the Priority Review accelerated pathway as it meets unmet medical need.
- 2019 – Yescarta (axicabtagene ciloleucel) CAR-T, an autologous cell-based gene therapy, is authorized for general distribution for one indication through the Priority Review accelerated pathway as it meets unmet medical need.

WHAT IS NEXT IN THE REGULATION OF ADVANCED CELL AND GENE THERAPIES?

Regulatory Challenges: Advanced Therapeutic Products

- The speed at which innovative products can be developed, the method with which they are made or distributed, and how data can be collected, has resulted in a shift away from the traditional product development model for which the current regulations are based
- Some health products are so novel and distinct that it is difficult for them to meet the current regulatory requirements
- Lack of appropriate regulatory oversight for continuously changing products and innovative business practices

The Food and Drugs Act - Additions to Schedule G

21.91 (1) For the purpose of preventing injury to health or preventing a person from being deceived or misled, the Minister may, by order, add a description of a therapeutic product or a class of therapeutic products to Schedule G if the Minister believes that **the therapeutic product or products represent an emerging or innovative technological, scientific or medical development.**

The Food and Drugs Act - The Factors

(2) Before adding a description of a therapeutic product or a class of therapeutic products to Schedule G, the Minister shall consider the following factors:

(a) the degree of uncertainty respecting the **risks and benefits** associated with the therapeutic product or products and the measures that are available to adequately manage and control those risks;

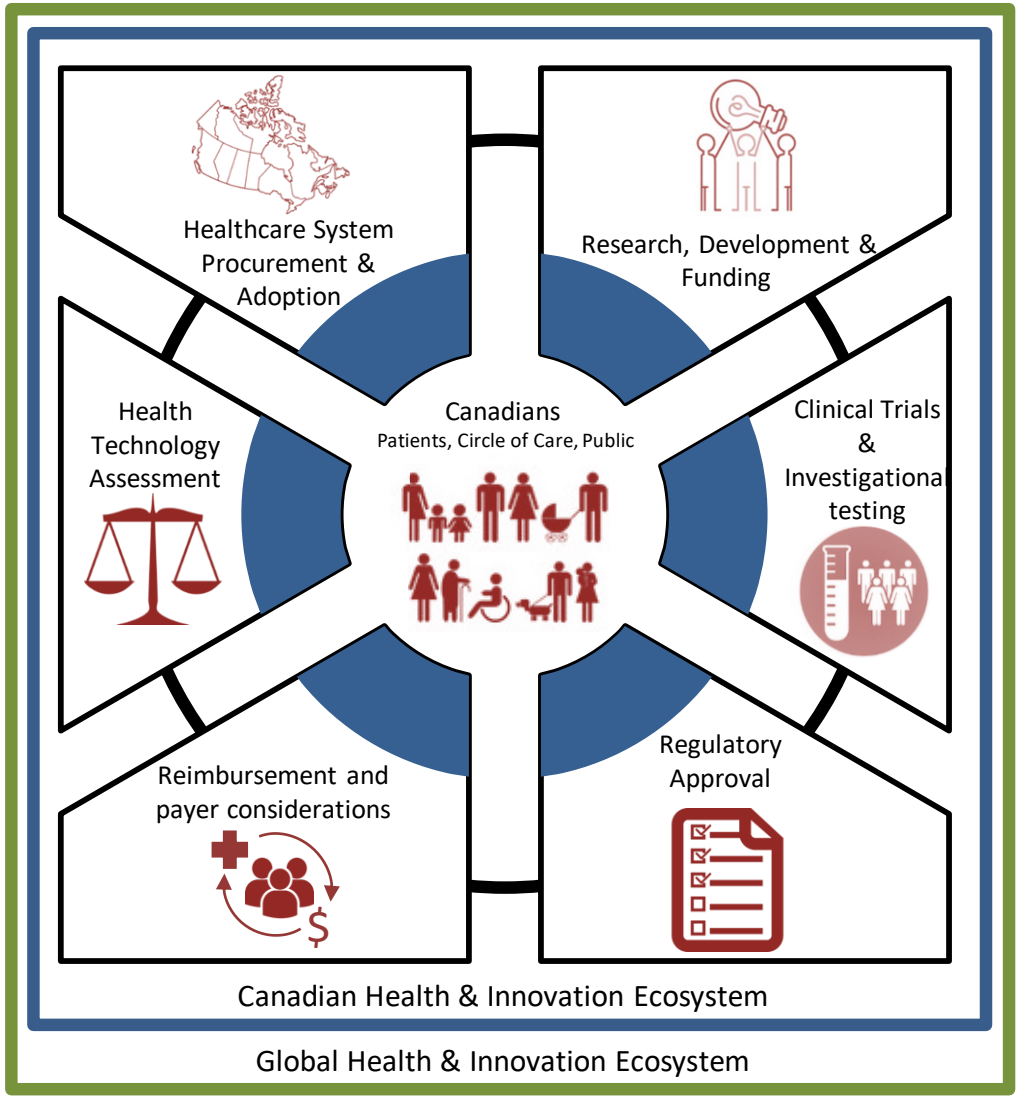
(b) the extent to which **the therapeutic product or products are different** from therapeutic products for which therapeutic product authorizations have been issued under the regulations;

(c) the extent to which **existing legal frameworks are adequate** to prevent injury to health or to prevent persons from being deceived or misled; and

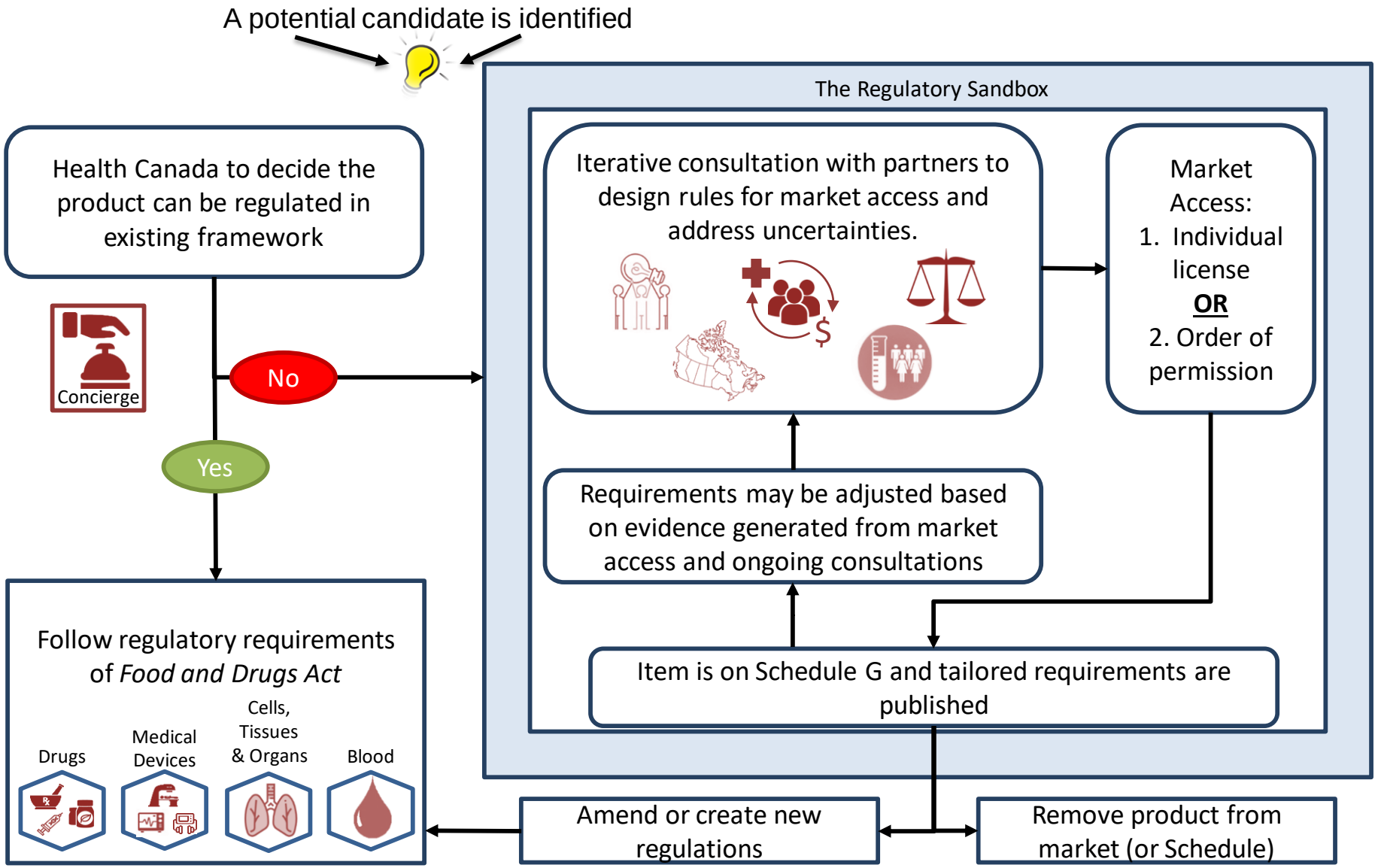
(d) the prescribed factors, if any.

Engaging the Health & Innovation Ecosystem

- Health Canada will view these intersecting relationships as an ecosystem.
- Health Canada's Concierge will engage throughout the ecosystem.



Advanced Therapeutic Products and The Regulatory Sandbox



Key Benefits of The Regulatory Sandbox

Patients	Regulated Parties	Health Practitioners
• broadens access to the most innovative advanced therapeutic products	• facilitates sharing information, which builds trust among all points across the biomedical sector	• support clinicians caring for patients in need of their ingenuity with treatment protocols and options to address unmet needs and improve outcomes

What to Expect with Advanced Therapeutic Products in the Near Future

- Consultation with Stakeholders will continue
  - Meetings with stakeholder groups are anticipated to happen in the new year
- Health Canada is examining options to pilot this exciting new pathway
  - Launch of a website and email address
- Health Canada is developing a Guide to these new innovative authorities
  - Expect a draft for comment in the new year

Questions

Contact:
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