CMC STRATEGY® FORUM
ADVANCING BIOPHARMACEUTICAL DEVELOPMENT
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ADVANCING BIOPHARMA VIRTUALLY

## Session II - Navigating Emerging Technologies: Regulatory Challenges and Opportunities in Pharmaceutical Innovation

# Best Practices, Innovative Regulation, Challenges and New Opportunities in ATMP Review and Approval - ANVISA/Brazil Perspective

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# BRAZILIAN NATIONAL HEALTH SURVEILLANCE SYSTEM

## ANVISA

**Nacional Competent Authority**

**Local Competent Authorities**



Law 9.782/99/Anvisa

- **MEDICINES:** Synthetic, Semi-synthetic, and Herbal Products
  Biological Products / Radiopharmaceuticals
  **Advanced Therapy Medicinal Products (ATMPs)**
- **BLOOD, TISSUES, CELLS, ORGANS FOR TRANSFUSION/TRANSPLANTS**
- **MEDICAL DEVICES**
- **HEALTH SERVICES**
- **FOODS**
- **COSMETIC AND BIOCIDE PRODUCTS**
- **PESTICIDES**
- **TOBACCO AND DERIVATIVES**
- **SANITARY SURVEILLANCE IN PORTS, AIRPORTS AND BORDERS**

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# Specific Regulatory Framework for ATMP in Brazil



✓ **Special class of complex medicinal product, defined by Law no. 14,874/2024**

ANVISA has developed specific regulatory frameworks for ATMP, covering the following requirements:

**Marketing authorization**: RDC 505/2021

**Clinical trials**: RDC 506/2021

**GMP for ATMP**: IN 270/2023, harmonized with PIC/S Annex 2A

**Good Practices in Blood, Tissue and Cells Human** (starting material for ATMPs)

Risk-based, case-by-case regulatory approach

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# Types and Scope of ATMPs in Brazil

- ✓ Advanced Cell Therapy Products: contains or consists of **cells** that have been subject **to substantial manipulation** so that biological characteristics, physiological functions or structural properties relevant for the intended clinical use have been altered, or of cells that are **minimally manipulated not intended for homologous use**

- ✓ Tissue Engineering Product: consisting of human **cells organized into tissues or organs** that present properties that allow the regeneration, reconstruction or replacement of a human tissue or organ, in the **presence or not of structural support** consisting of biological or biocompatible material

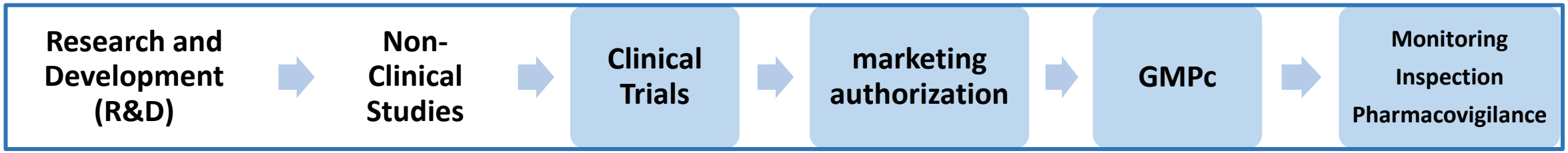
- ✓ Gene Therapy Product: it contains **recombinant nucleic acid used** in or administered to human beings with a view to regulating, repairing, replacing, adding, deleting and editing a genetic sequence.

- ✓ Combined Advanced Therapy Product: an advanced therapy product that incorporates, as an integral part of the product, one or more medical devices, in which the cellular component—containing viable or non-viable cells or tissues—exerts a pharmacological, immunological, or metabolic action

# Specific Regulatory Framework ATMP in Brazil

**ANVISA** - Proof of Safety, Efficacy and Quality

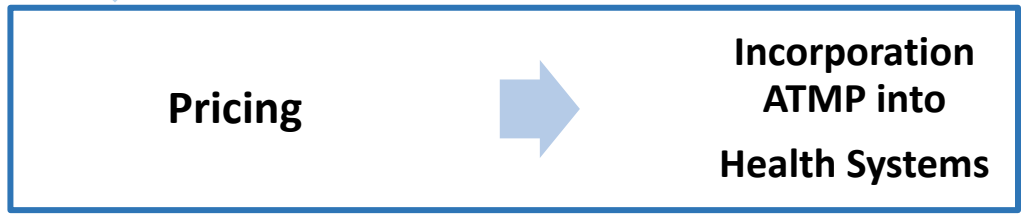
**BENEFITS AND RISKS**




**National Research Ethics Authority - INAEP (Ministry of Health)** –approving the ethical aspects of clinical research

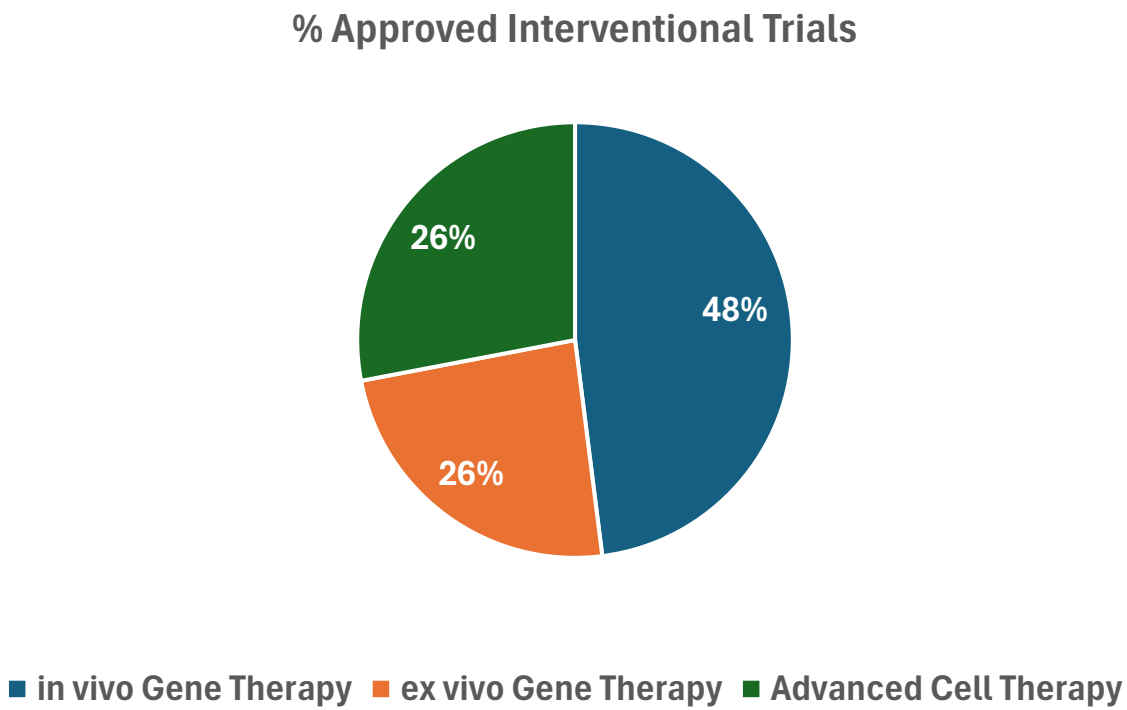
**National Technical Biosafety Commission** (**Ministry of Science, Technology and Innovation**) – authorization of activities involving genetically modified organisms (GMOs) – gene therapy

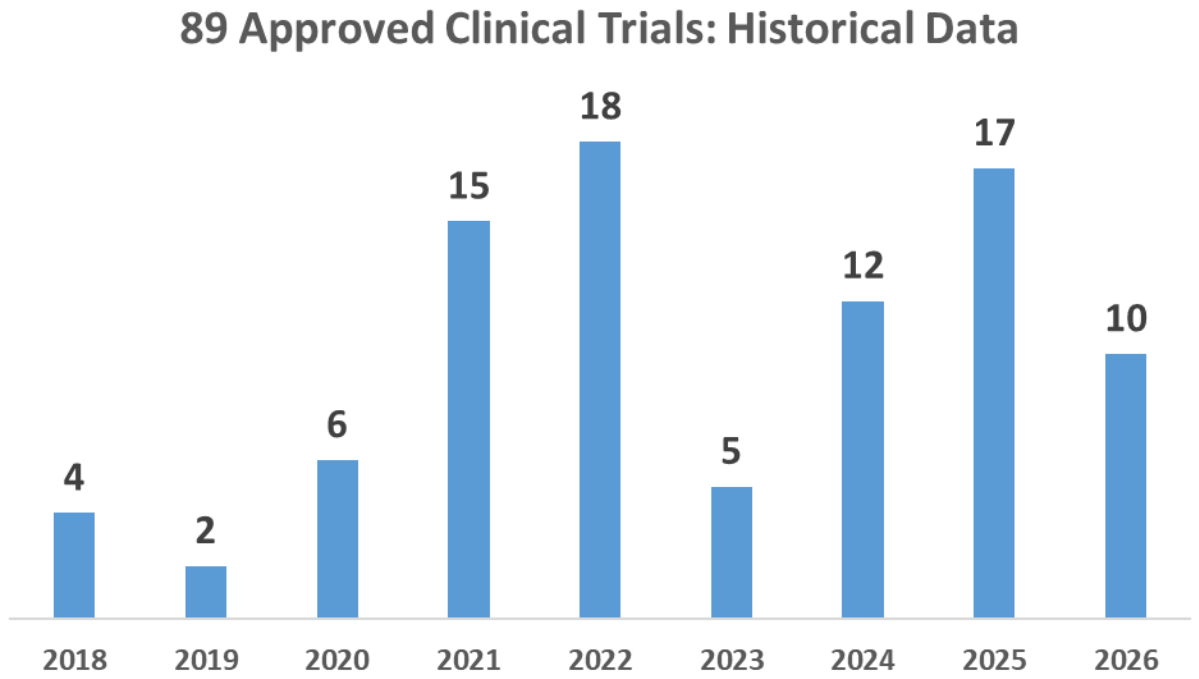
**BENEFITS AND COSTS**




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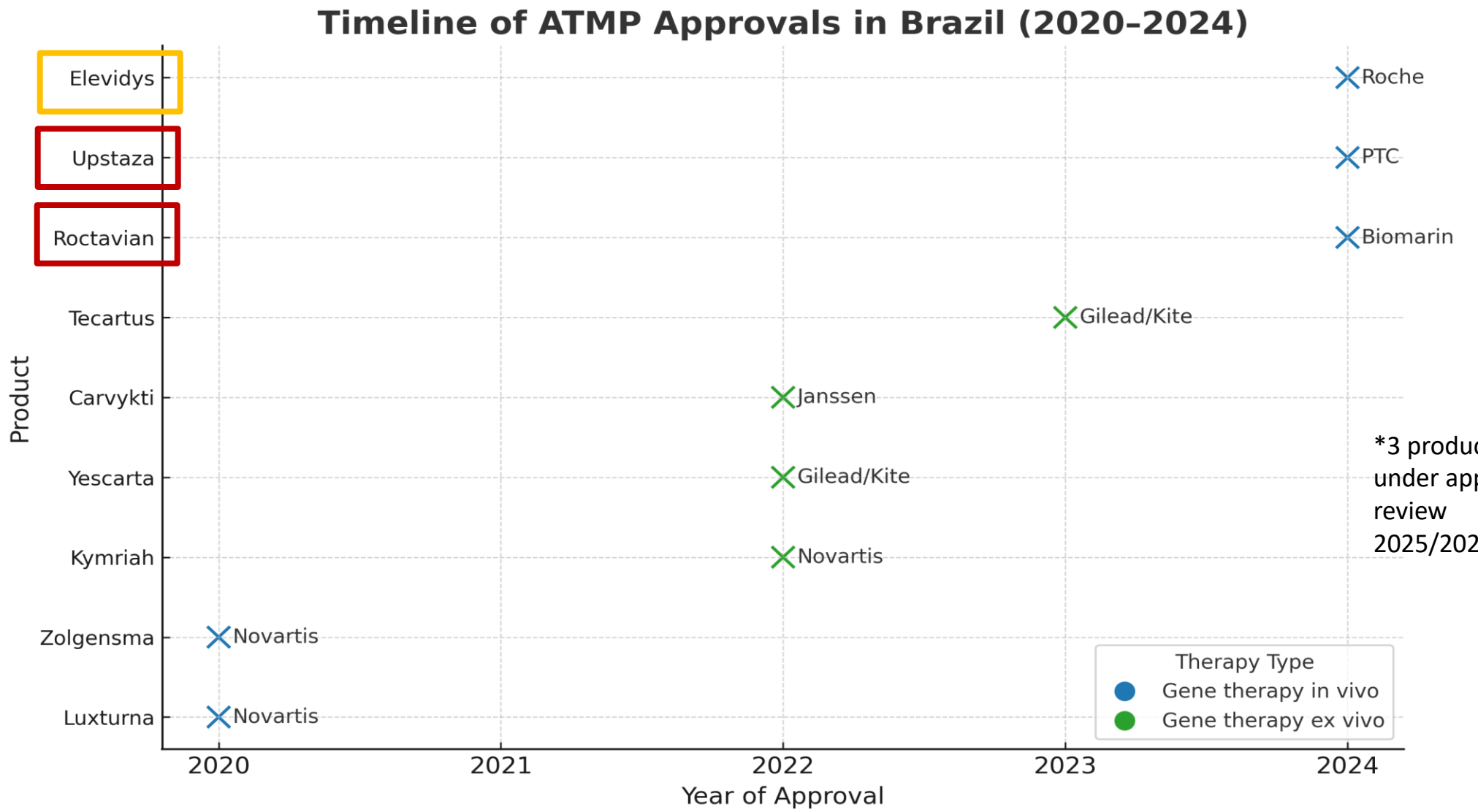
# Clinical Trials with Advanced Therapy Products in Brazil (since 2018)




Source Anvisa, July 2026: https://www.gov.br/anvisa/pt-br/assuntos/sangue/terapias-avancadas/ensaios-autorizados

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*3 products under approval review 2025/2026

**Suspended**

**Cancelled**

Source Anvisa, July 2026: https://www.gov.br/anvisa/pt-br/assuntos/sangue/terapias-avancadas/ensaios-clinicos-autorizados/produtos-registrados

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# REGULATORY INNOVATION



✓ **CONDITIONAL APPROVAL FOR ATMPS**

Treating serious or rare diseases with unmet medical need

Approval based on limited clinical evidence with commitments to long-term studies, specific monitoring and risk mitigation (Term of Commitment).

Annual periodic monitoring (of data to confirm the long-term benefit–risk balance)

✓ **ACCELERATED REGULATORY ASSESSMENT**

Treating serious or rare diseases with unmet medical need, public health priority, emerging or re-emerging disease, pediatric indication, early-stage development (Phase I and II) conducted in Brazil.

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# REGULATORY INNOVATION

✓ **EXCEPTIONAL CLINICAL**

- Use in a specific patient
- For imminently life-threatening  or emergency (non-routine) situations
- No approved therapeutic alternative available in Brazil
- Under medical responsibility – with scientifically demonstrated rationale (request submitted by a physician)
- Provided that the experimental product is manufactured in compliance with GMP standards
- Under ANVISA's supervision and regulatory oversight: prior authorization by Anvisa

# REGULATORY INNOVATION

✓ **LONG-TERM FOLLOW-UP AND ACTIVE PHARMACOVIGILANCE STRATEGIES**

Dynamic and adaptive Risk Management Plans (Active pharmacovigilance)

Post-authorization clinical studies:

- Long-term follow-up studies
- Registry studies and real-world evidence studies

Rapid regulatory decisions (precautionary measures)

International regulatory cooperation

# ONGOING INITIATIVES IN BRAZIL

- ✓ National **expert networks providing scientific advice to Anvisa** on advanced therapies (ANVISA's Committee – CAT official)
- ✓ Implementation of **regulatory advisory** strategies for national and international companies in the development of ATMPs in Brazil
- ✓ Publication of **technical guidelines** on requirements for: specific aspects for preclinical development, clinical trials and quality aspect of ATMPs
- ✓ Strengthening national **inspections in Cell Collection and Processing Centers** (starting materials) **and Good Clinical Practice (GCP) inspections** in clinical trials sites

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# future directions and opportunities



- ✓ Promote and expand regulatory convergence strategies (ICH, ICMRA, WHO, BRICS, Mercosur).
- ✓ Enhance technical cooperation with other competent authorities- Implement joint assessment initiatives
- ✓ Goal: Ensure access to safe, effective, high-quality, and equitable ATMPs for all Brazilians.
- ✓ Collaboration among agencies and stakeholders' bridges innovation and access.

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# OBRIGADO!
# GRACIAS!
# THANK YOU!

GSTCO/GGBIO/DIRE2

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