EMA regulatory update

Rapid Fire Session

Veronika Jekerle

Head of Pharmaceutical Quality,

European Medicines Agency

CASSS-CGT

Basel 23-24 October 2025

Innovation trends: Biologicals (2022 – 24)

- End-to-end continuous manufacturing
- **Gene editing/gene therapy**
- **Cell therapy**
- Microbiota

- **Advanced manufacturing (ATMPs,** mABs)
- Decentralised manufacturing
- Artificial intelligence in manufacturing

- **ATMP manufacturing (closed systems, automated)**
- Advanced manufacturing (e.g. blockchain, in line viral safety testing)
- **Personalised medicines** (i.e. mABs, **gene therapy**)

Trends observed for CMC - Briefing meetings between sponsors and Innovation task force **(ITF)**
Supporting innovation | European Medicines Agency (EMA)

EU pharmaceutical legislation

New pharmaceutical legislation

What will the pharmaceutical sector reform change?

Single Market	creating a Single Market for medicines
Regulatory framework	reducing the administrative burden for medicines to reach patients faster
Medicines for all	ensuring better access to affordable medicines
Medicine supply	addressing shortages of medicines and ensuring security of supply
Innovation	promoting innovation and competitiveness
Environmentally friendly	making medicines more environmentally sustainable
Saving lives	tackling antimicrobial resistance (AMR)
Transparency	informing better about public funding used to develop medicines

Enabling mechanisms for innovation/CMC

Master files
Personalised medicines
Platform approaches
Decentralised manufacturing
Regulatory sandbox

Variation framework & Annex II
Strengthened pre-submission support

Revised variation classification GL: main changes

Procedural part

- **Operational details shifted** to EMA/CMDh guidance for **easiest updates** in the future.
- **Change the current code system (numbering)** to facilitate the implementation of the new framework

A. Administrative variations

- Reduction/simplification list from 8 to 5 scopes.

B. Quality variations

Review of all categories:

- **Downgrade** certain scopes when scientifically justified (risk/based approach).
- **Removed conditions of biological** medicinal products in certain circumstances allowing a Type IA variations.
- **Implementation of PACMP** as Type IB or Type IA also for BIO.
- **Alignment** and **consistency** between Chemicals and Biologics where appropriate
- New section on **In-house reference preparations.**
- New scopes for **Medical devices** in line with MDR.

C. Safety, Efficacy, PhV variations

- **Deletion** of scopes (C.I.9, C.I.10, as now done via Art. 57 database).
- C.I.3 **expanded to include** implementation of **PRAC signals** and **joint recommendations of EU authorities.**
- New scope for submission of **results** of assessments carried out on **target patient groups**.

D. PMF

- Reduction/simplification list from 23 to 16 scopes.

entry into force 15 Jan 2026

Platforms

- **Listen & learn focus group (19-20 November 2024)**
- **Regulators' considerations**: prior knowledge/platform approaches
- **Industry examples of platforms**: vaccines, mRNA, gene therapy, continuous direct compression, oligonucleotides

Discussion points:

- **Benefits of the platform**: streamlined development, data utility, acceleration
- **Prerequisites:** definition, documentation, scientific data maturity, reproducibility
- Difference between Prior knowledge & platform concept
- Modular approach versus end-to-end
- Product experience & the ideal product class
- Lifecycle management of a platform

Personalised medicines

- **Listen & learn focus group (8-9 April 2025)**
- **Current scientific understand & regulatory perspective**
- **Industry examples**: decentralised production of ATMPs, personalised T-cell therapy, synthetic DNA cancern immunotherapy, 3D printing, individualised antisense oligonucleotides

Discussion points:

- **Adaptive regulatory framework + standardisation**
- **Control strategy:** risk-based considerations, surrogate assays, prior knowledge
- AI and modelling approaches (→ link back to EMA Q&A + EU GMP Annex 22)
- Oligonucleotides: mature product class: personalised approached mentioned in EMA's draft guidance
- 3D printing: emerging technology, highly standardised, decentralised manufacturing aspects, hamronisation of QUA control (e.g. for Pharmacopoeias)

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

24 March 2025

Quality Innovation Group (QIG)

Listen and Learn Focus Group (LLFG) meeting on

Personalised Medicines

Agenda

8th-9th April 2025

Virtual meetingroche

Chair: Marcel Hoefnagel

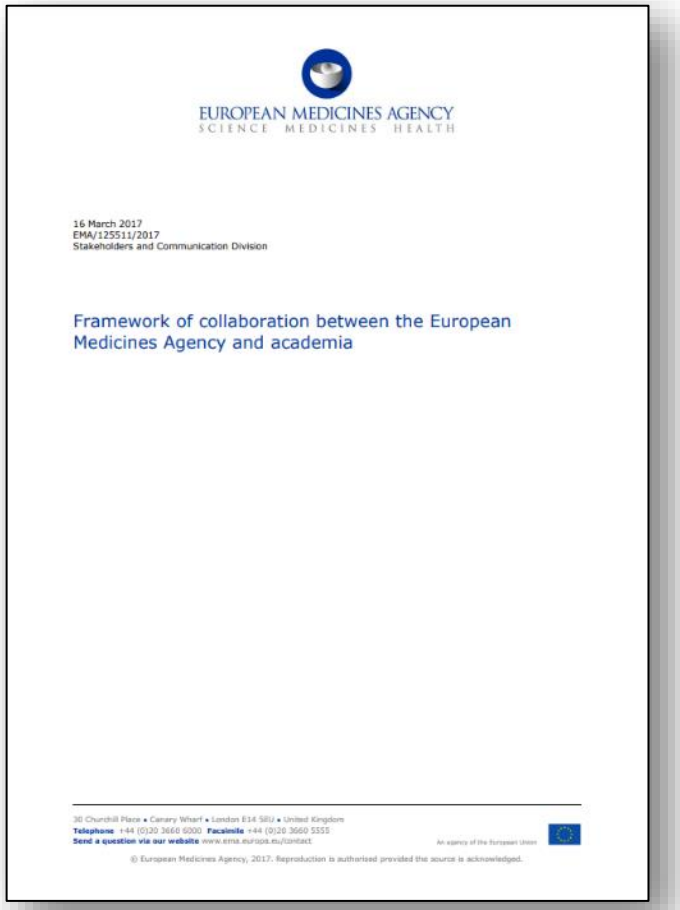
Day 1

Chair: Marcel Hoefnagel

Timing		Contributors:
10:00 – 10:10	**Opening of the LLFG** Welcome to participants. Introduction to QIG, meeting scope and objectives of the QIG LLFG on Personalised Medicines.	Marcel Hoefnagel (QIG Chair, MEB)
10:10 – 10:40	1: Setting the scene: Personalised Medicines	Silke Schüle (QIG Member)
Session 1		
10:40 – 11:10	2: Artificial Intelligence-driven, Decentralized Production for Advanced Therapies in the Hospital	Frederik Erkens (Fraunhofer IPT) John Jacobs (ORTEC), Joe Egan (UCL), Katrin

Academia

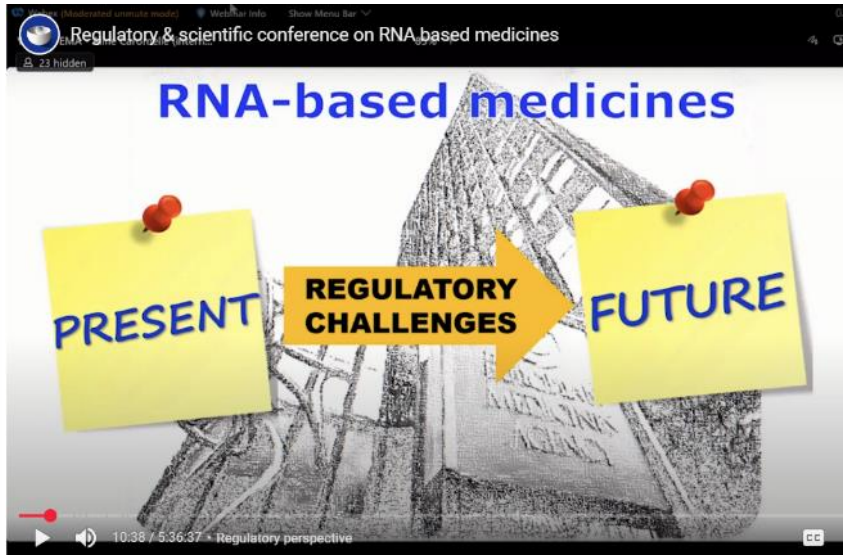
- Support, engagement and collaboration framework



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 March 2017
EMA/125511/2017
Stakeholders and Communication Division

Framework of collaboration between the European Medicines Agency and academia



https://www.ema.europa.eu/en/events/regulatory-and-scientific-virtual-conference-rna-based-medicines

Home > News
> EMA pilot offers enhanced support to academic and non-profit developers of advanced therapy medicinal products

EMA pilot offers enhanced support to academic and non-profit developers of advanced therapy medicinal products

Share

29 September 2022

News | Human | Advanced therapies | Medicines

EMA is launching a pilot to support the translation of basic research developments into medicines that could make a difference in patients' lives in the European Economic Area (EEA). The pilot is open to academic sponsors and non-profit organisations who are developing advanced therapy medicinal products (ATMPs). These medicines for human use are based on genes, tissues or cells and might offer ground-breaking treatment options to patients.

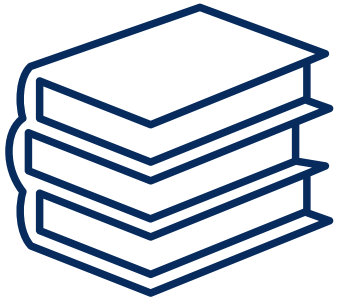
The pilot will focus on the needs of non-profit academic developers. They are a major contributor to the development of ATMPs and diagnostic and delivery devices, but experience has shown that navigating regulatory requirements can be challenging.

During the pilot, EMA will provide enhanced regulatory support for up to five selected ATMPs that address unmet clinical needs and are solely developed by academic and non-profit developers in Europe. EMA will guide the participants through the regulatory process with the aim to optimise the development of the ATMPs, starting from best practice principles for manufacturing to planning clinical development that meets regulatory standards.

The pilot's first participant has already been selected. This ATMP is ARI-0001, a chimeric antigen receptor (CAR) product based on patients' own T-cells, that is developed by the Hospital Clinic Barcelona. In December 2021, the product was

https://www.ema.europa.eu/en/partners-networks/academia

Resources



CAT

→ *Ilona*

CHMP/BWP guidance

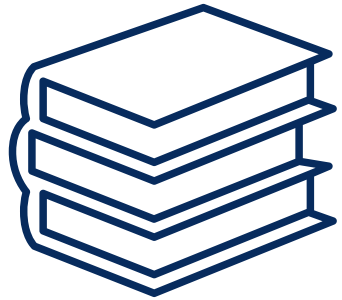
- Guideline on quality aspects of **RNA vaccines** *(revision for finalisation in 2026)*
- Guideline on the development and manufacture of human medicinal products specifically designed for **phage therapy** *(draft in public consultation)*
- QIG Preliminary Considerations on **Pharmaceutical Process Models** *(draft)*

- Guidance to support **new variations framework** (i.e. complex manufacturing, PACMP, PLCM, other)

- **ICH Guidance:**
  - Cell & Gene therapies discussion group (i.e. comparability of ATMPs)
  - ICH Q1/ICH Q5C Stability Testing of Biotechnological/Biological Products

- Parallel scientific advice with FDA (link)

Resources



nature reviews drug discovery

nature › nature reviews drug discovery › biobusiness briefs › article

BIOBUSINESS BRIEFS | 15 March 2024

Genome-editing medicinal products: the EMA perspective

By Anna Tavridou, Dolça Rogers, Giada Farinelli, Iordanis Gravanis & Veronika Jekerle

Genome editing technologies such as CRISPR–Cas9 are being developed as therapeutic platforms, especially for monogenic diseases that could be treated by targeting the associated mutations. The first CRISPR–Cas9-based medicinal product, Casgevy (exagamglogene autotemcel), has recently been granted marketing authorization in the European Union for sickle cell disease and β-thalassemia, and a substantial number of other genome-editing medicinal products (GEMPs) are in development.

Nevertheless, the field of GEMPs is still nascent, with very limited scientific guidance available to developers to fit these complex products into existing regulatory frameworks. A recent horizon scanning report by the EU Innovation Network includes initial regulatory considerations, socioeconomical aspects and recommendations to help better understand general challenges and opportunities of genome editing. Developers seeking to market their products in Europe should also make use of tailored support from national competent authorities and the European Medicines Agency (EMA): that is, Innovation Task Force (ITF) meetings, PRIority MEdicines (PRIME) designation and scientific advice (SA). SA – recommendations from the EMA that may aid developers on aspects of the medicine development supporting a marketing

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ORIGINAL ARTICLE

Towards a better use of scientific advice for developers of advanced therapies

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Scientific advice (SA) is an important tool offered by regulators to help developers generate robust evidence on a medicine's benefits and risks. Drawing on accumulated experience and looking at the SA provided by the European Medicines Agency in 2018 to advanced therapy medicinal products originally developed by public bodies, we discuss most commonly raised issues and the complexity and timings of the questions posed. Earlier and more frequent SA could help advanced therapy medicinal product developers to pre-empt delays at the marketing authorisation stage. Carefully addressing quality and nonclinical issues before entering the pivotal phase of development will clear the path for a smooth clinical development and successful marketing authorisation.

KEYWORDS
advanced therapy medicinal products, drug development, drug regulation, scientific advice

1 | INTRODUCTION

Public bodies (including academic institutions, research organisations, hospitals), public–private partnerships and small and medium-sized enterprises (SMEs) represent an important source of innovative therapeutics.[1] This holds particularly true for the area of advanced therapy medicinal products (ATMPs), as research on these products and their initial development is conducted to a great extent by public bodies and SMEs.[2] This is confirmed by a recent survey, which concluded that the European ATMP field is still in early phase of maturity with a high representation of SMEs (65%) and 72% of reported therapeutics in early clinical development (phases I–II).[3]

Public bodies and SMEs tend to have limited resources to conpharmaceutical companies (e.g. Strimvelis, Imlygic, MACI, Holoclar, Zolgensma). Moreover, academic institutions and SMEs may encounter more challenges in navigating—and complying with—regulatory requirements on various aspects of development compared with large pharmaceutical companies.[4] These challenges could cause delays at different stages of development and even lead to abandonment of potentially promising projects.

The scientific advice (SA) service is provided by regulators around the globe and is a useful tool to support the timely and sound development of high-quality, effective and safe medicines, for the benefit of patients. At the European Medicines Agency (EMA), this service is provided by the Scientific Advice Working Party supported by the Committee of Advanced Therapies.[5] This is,

Drug Discovery Today • Volume 30, Number 9 • September 2025 REVIEWS

ELSEVIER

Learning from the EMA experience: common CMC deficiencies in marketing authorisation applications over the past decade

Dolores Hernán Pérez de la Ossa*, Friederike Haas, Robert N. Bream, Evangelos Kotzagiorgis, Klara Tiitso, Veronika Jekerle

European Medicines Agency, Pharmaceutical Quality Office, Human Division, Amsterdam, the Netherlands

Here, we present an analysis of major objections (MOs) raised on quality aspects during review of marketing authorisation applications (MAAs) for new medicines via the centralised procedure in the European Union (EU). The review covers a 10-year period by analysing data from 2013, 2018, and 2023. We identify common deficiencies, which should help developers prepare dossiers aligned with EU and global standards and avoid delays to approval of medicines, thereby improving patient access to medicines. The most common deficiencies are correlated with specific product types, recent public health crises, new legal frameworks, and the publication or revision of guidance.

Keywords: medicines; marketing authorisation; major objection; quality; deficiencies

Introduction

In the EU, in general, all medicines must be authorised before they can be marketed and made available to patients. Several mechanisms exist: the centralised procedure (CP) [coordinated by the European Medicines Agency (EMA) and authorisation granted by the European Commission (EC)], and the national, decentralised, and mutual recognition procedures [managed by the respective National Competent Authorities (NCAs)]. A medicine approved through the CP can be marketed throughout the EU and, thus, made available to all patients and healthcare professionals in the EU simultaneously. The CP is governed by Regulation (EC) 726/2004[(1)] and adheres to legal timelines, comprising assessment periods and 'clock-stops', which allow the applicant to respond to questions raised at defined milestones during the assessment.[(2)] To apply for a marketing authorisation, an applicant must submit a comprehensive set of information and data [an electronic Common Technical Document (eCTD),[(3)] also 'dossier'] to EMA and NCAs demonstrating that the medicine is safe, efficacious, and of suitable quality for its intended use. The Committee for Medicinal Products for Human use (CHMP) of the EMA is responsible for assessing the quality, safety, and efficacy of the medicine and determining its benefit/risk to provide a scientific opinion to the EC on whether it should be granted a marketing authorisation. During the assessment, deviations from the EU standards are raised as issues in the form of either Major Objections (MOs) or Other Concerns (OCs), which need to be resolved before a marketing authorisation can be granted. MOs are serious concerns on the quality, safety, or efficacy of the medicine, with an impact on its benefit–risk ratio, and can relate, for example, to the way in which the medicine was developed, the way it was manufactured or tested, or to the effects seen in patients. OCs relate to issues that are not deemed crucial for the benefit–risk ratio of the medicine, but are still expected to be addressed before marketing authorisation. In general, the number of MOs and OCs raised is a good indicator of the maturity of a dossier and can highlight challenges that an applicant faced during product development. A dossier that is aligned with the EU regulatory standards decreases the probability of delays to authorisation and sets a good basis for lifecycle management of the medicine.

In this study, we review the questions raised by CHMP during the assessment of quality-related aspects (also known as chemistry manufacturing and controls; CMC) of dossiers for marketing authorisation applications (MAAs) for human medicines

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EMA

Bundesamt für
Sicherheit im
Gesundheitswesen
BASG

CAT regulatory update

Rapid Fire Session
CASSS-CGT
Basel, 23-24 October 2025

Ilona Reischl

Austrian Medicines and Medical Devices Agency

(AGES-MEA)

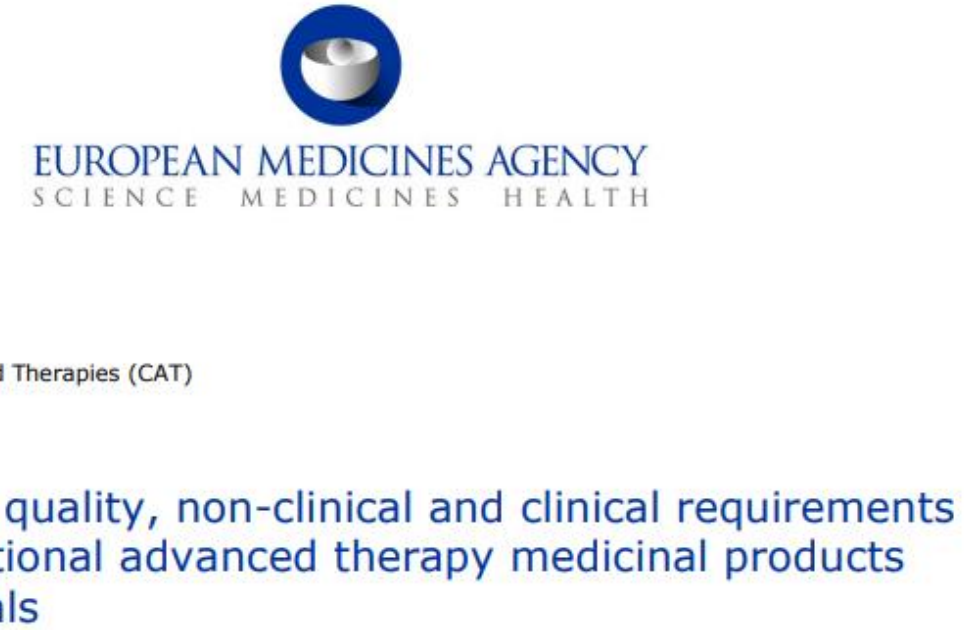
Pre-authorisation activities

Guidance

Bundesamt für Sicherheit im Gesundheitswesen
BASG

Guideline on requirements for investigational ATMPs in clinical trials

- Finalized, accessible via the EMA webpage, EudraLex Volume 10 to come
- Internal training and external training in preparation
- Publication on ATMP clinical trials in Europe 2016-2024


EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

20 January 2025
EMA/CAT/22473/2025
Committee for Advanced Therapies (CAT)

Guideline on quality, non-clinical and clinical requirements for investigational advanced therapy medicinal products in clinical trials

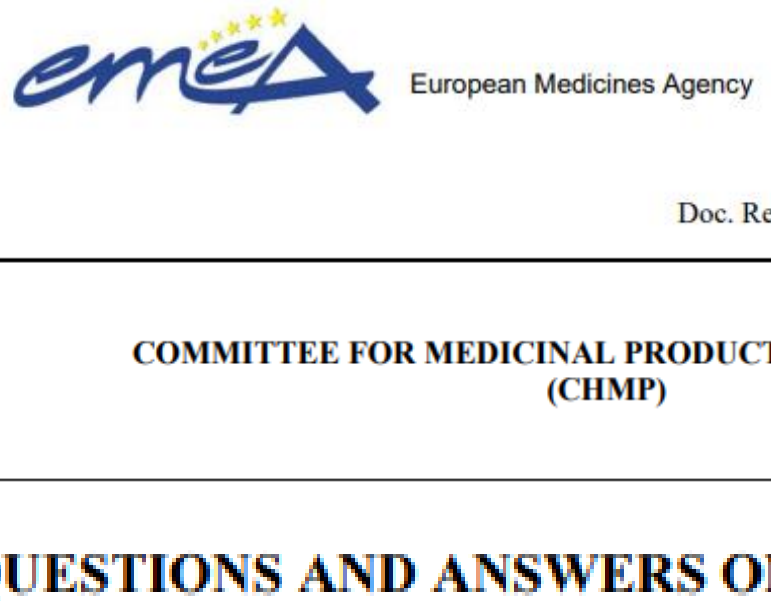
Pre-authorisation activities

Guidance

Bundesamt für Sicherheit im Gesundheitswesen
BASG

Revision of the Questions and Answers on Gene Therapy

- EMA/CHMP/GTWP/212377/2008 was published in 2009 and is outdated.
- Major revision is needed and work is ongoing
- Widen the scope to include all ATMPs and include CAT/BWP lessons learned


European Medicines Agency

London, 17th December 2009
Doc. Ref: EMA/CHMP/GTWP/212377/2008

COMMITTEE FOR MEDICINAL PRODUCTS FOR HUMAN USE
(CHMP)

QUESTIONS AND ANSWERS ON GENE THERAPY

Pre-authorisation activities

Guidance

Bundesamt für Sicherheit im Gesundheitswesen
BASG

Development of guidance on Gene editing

- Workshop co-organized with ARM

https://www.ema.europa.eu/en/events/committee-advanced-therapies-cat-workshop-gene-editing

  - Hybrid setting: CAT secretariat & CAT experts in person, speakers & audience remote.
  - Open session (10.00 – 17.00):

    15 speakers

    474 participants (registered) + life broadcast (participant numbers not known)
  - Closed session - 1 company presentation

- Initiate the development of a concept paper / reflection paper on genome editing

Pre-authorisation activities

Guidance

Bundesamt für Sicherheit im Gesundheitswesen
BASG

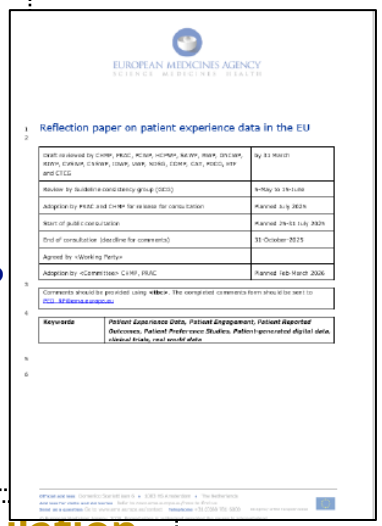
Development of guidance on Gene editing

- Opportunities for further engagement depending on the setting and goals:
  - ITF meetings (informal interaction)
  - Quality Innovation group (QIG) interaction
  - Scientific advice (EMA and NCAs)
  - PRIME: Toolbox guidance on scientific elements and regulatory tools to support quality data packages for PRIME and certain marketing authorisation applications
- All general paths for interaction are also open in the context of gene editing

Reinforcing patient relevance in evidence generation: key priority in EMA

- **Reflection paper on PED (2024)**
  - general EU framework and principles, public consultation Q3 2025
- **International harmonization**
  - ICH guidance
- **HMA/EMA Multistakeholder workshop & publication on Optimising Patient registries for Regulatory Decision Making (2024).**
- **EMA and EORTC workshop: How can patient-reported outcomes (PRO) and health-related quality of life (HRQoL) data inform regulatory decisions? (2024)**
- **Study on SMA registries to support regulatory decision making.**

Still a need for systematic inclusion of PED in medicines development and regulation

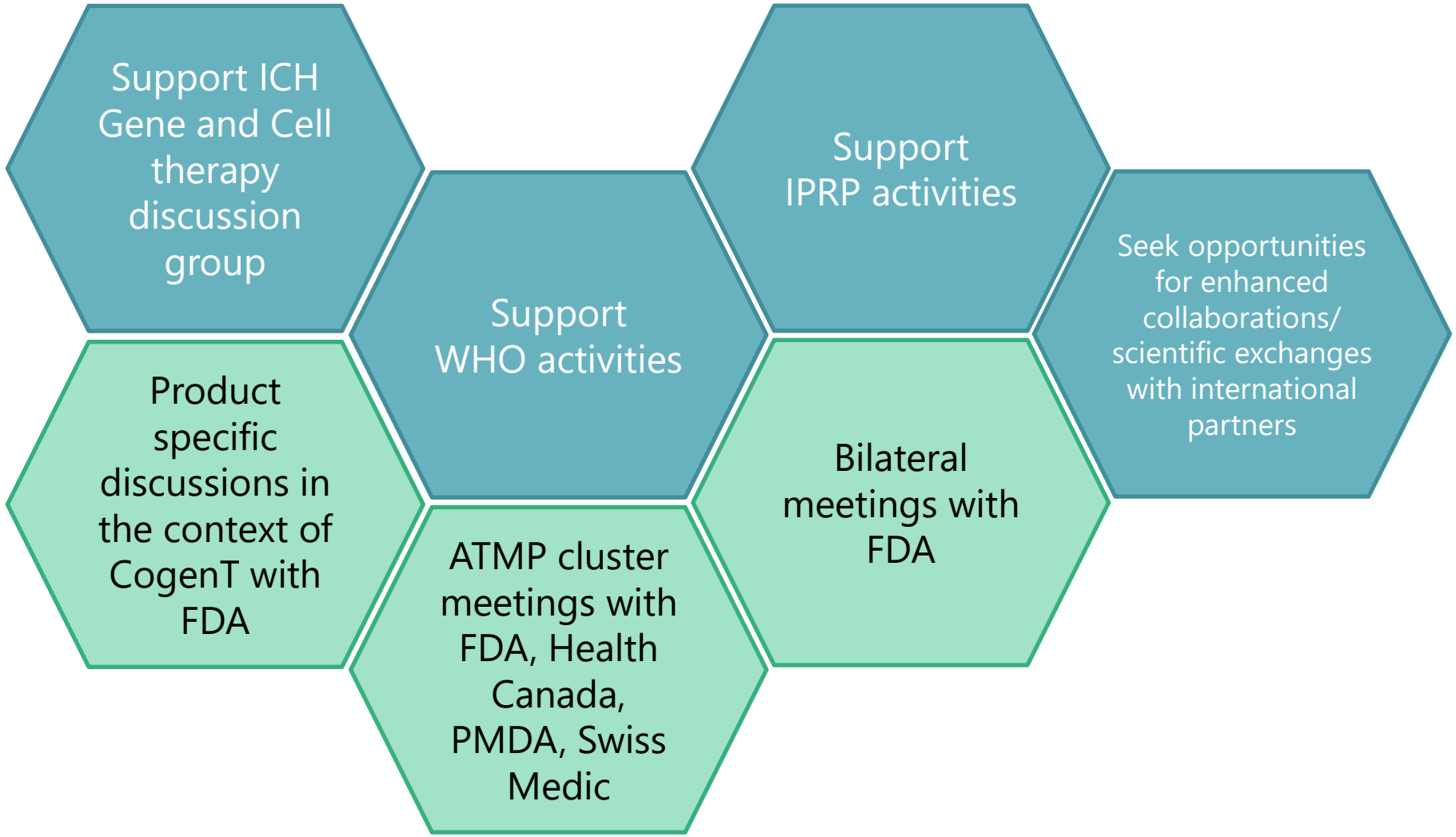


K. Sollerbrant, K. Breen and M. de Lemus Belmonte CAT SRLM Warsaw 2025

Horizontal activities and other areas

Bundesamt für Sicherheit im Gesundheitswesen
BASG

International Regulatory Science Collaboration



Horizontal activities and other areas

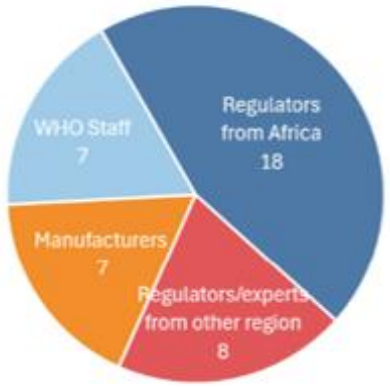
International Regulatory Science Collaboration

Bundesamt für Sicherheit im Gesundheitswesen
BASG

Implementation Workshop on 'WHO Considerations in Developing a Regulatory Framework for Human Cells and Tissues and for Advanced Therapy Medicinal Products';
24-26 September 2025, Brazzaville, Congo

Chair: I. Reischl, Rapporteur: G. Jotwani

https://www.who.int/publications/m/item/executive-summary-regulatory-framework-human-cells-tissue



European Pharmacopoeia – activities in the cell and gene therapy field

Olga Kolaj-Robin, PhD

CASSS CGT Europe

23-24 October, Basel, Switzerland

European Pharmacopoeia (Ph. Eur.) and its structure

- ★ Protecting public health – one common compulsory standard
- ★ Official pharmacopoeia in Europe (+ national pharmacopoeias)
- ★ Legally binding quality standards; at the same date for all Members
- ★ 40 Members, 33 Observers
- ★ Issue 12.2: ~2600 monographs, ~400 general chapters, ~3000 reagents

* EU: European Union; TFDA: Taiwan Food and Drug Administration; WHO: World Health Organization

General chapters & general texts

- provide standard analytical procedures; guidance
- **become mandatory** when referred to in a monograph

General notices

- **Essential reading**
- Apply to all texts, address general topics
- Include rules to understand texts

Individual monographs

- Specific but not a stand-alone text
- Based on approved specifications backed up by batch data

General monographs

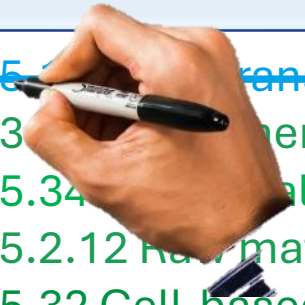
Dosage form monographs

- **Mandatory** for all substances/products within scope of their definition
- Not cross-referenced in individual monographs (exceptions)

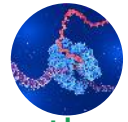
Ph. Eur. Reference standards / preparations & reagents

Cell and gene therapy – Ph. Eur.

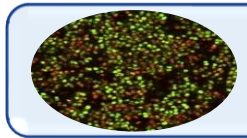
General overarching texts



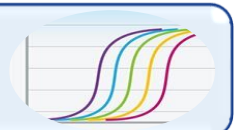
- ~~5.1 Transfer medicinal products for human use~~
- 3 erapy medicinal products for human use
- 5.34 l information on gene therapy medicinal products for human use
- 5.2.12 materials of biological origin for the production of cell-based and gene therapy medicinal products
- 5.32 Cell-based preparations for human use
- 5.47 Gene edited cells for human use



+ mRNA-based GTMPs



General methods: numeration & viability



- 2.7.23 Numeration of CD34+/CD45+ cells in haematopoietic products
- 2.7.24 Flow cytometry
- 2.7.28 Colony-forming cell assay for human haematopoietic progenitor cells
- 2.7.29 Nucleated cell count and viability
- 2.6.35 Quantification and characterisation of host-cell DNA

General chapters: Microbiology aspects & viral safety

- 2.6.1 Sterility
- 5.1.6 Alternative methods for control of microbiological quality
- 2.6.27 Microbiological examination of cell-based preparations
- 2.6.39 Microbiological examination of human tissues
- 5.1.13 Pyrogenicity - 2.6.30 MAT - 2.6.14 BET - 2.6.32 rFC
- 2.6.7 Mycoplasmas
- 5.1.7 Viral safety
- 5.2.8 TSE

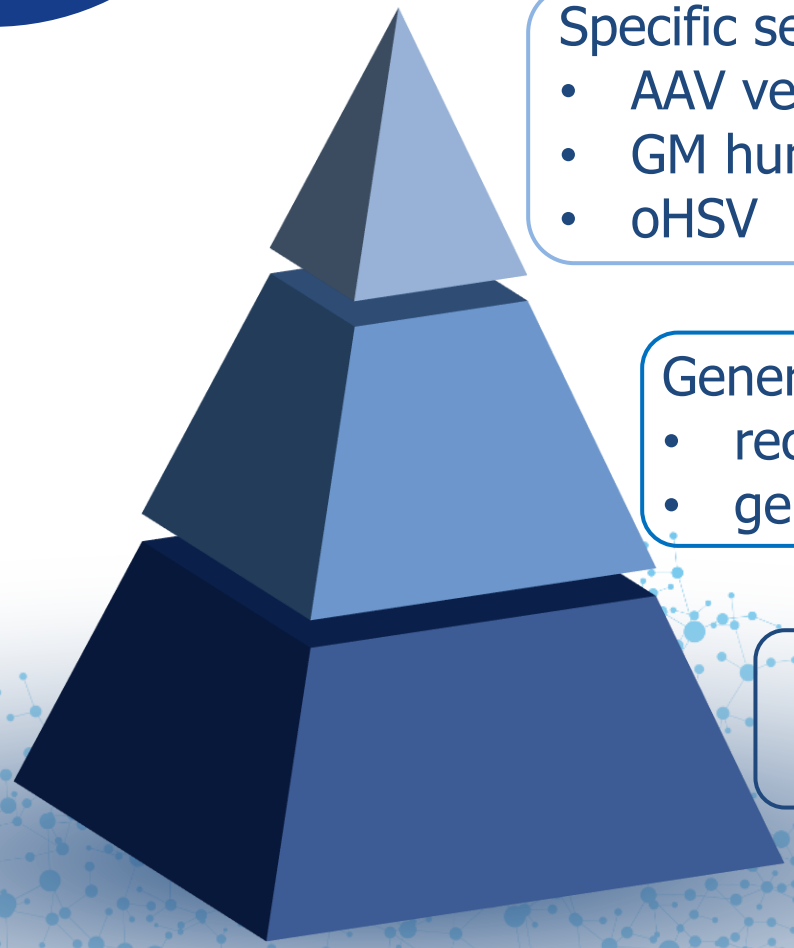
Individual monographs

- Bovine serum (2262)
- Human haematopoietic stem cells (2323)

Under elaboration
Under revision
Recently adopted/published/revised
~~Suppressed~~/planned suppression
Other published

Non exhaustive list

New general monograph 3186 – requirement layers



Specific sections
- AAV vectors
- GM human autologous cells
- oHSV

General requirements
- recombinant vectors
- genetically modified cells

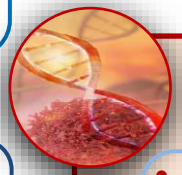
General provisions for GTMP production

Revised from 5.14
New sections

General monograph

Gene therapy medicinal products for human use (3186)

- Definition
- General requirements on:
  - the Production of GTMPs
  - Recombinant vectors (including GM bacterial cells)
  - Genetically modified cells
- Genetically modified autologous human cells modified by integrating retroviral or lentiviral vectors
- Adeno-associated-virus (AAV) vectors for human use
- Oncolytic herpes simplex virus (oHSV) for human use



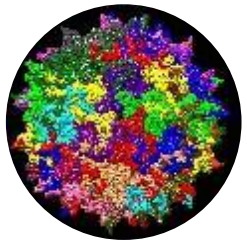
General chapter

Additional information on gene therapy medicinal products for human use (5.34)

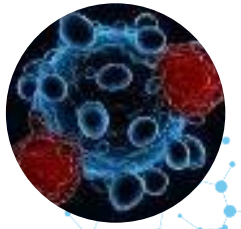
- Plasmid vectors for human use
  including
  Bacterial cells used for the manufacture of plasmid vectors for human use
- Genetically modified bacterial cells for human use
- Adenovirus vectors for human use
- Poxvirus vectors for human use
- Retroviridae-derived vectors for human use

Ph. Eur. gene therapy texts - flexibility

- risk-based approach incorporated (general requirements)
- Flexible wording, reference to suitable methods, no numerical acceptance criteria



- recombinant vectors: possibility to perform the test listed under respective headings at an alternative stage of the process; requirements applicable and performed under approved testing scheme if not possible to distinguish between harvests and final lot (general requirements)



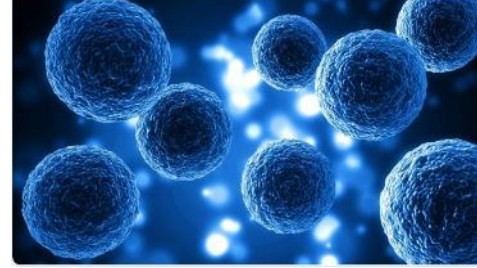
- genetically modified cells: listed tests to be performed at appropriate stages depending on the manufacturing process (general requirements and the individual section)

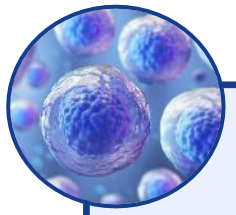
Advancements in gene therapy: the European Pharmacopoeia's new approach

ON DEMAND WEBINAR & PDF PRESEBTATION

https://www.edqm.eu/en/-/advancement-in-gene-therapy-new-approach

Cell-based preparation for human use (5.32)





General chapter

Cell-based preparations (5.32)

- ***Definition***
- ***General requirements***
  - General provisions for the production
    - Substances and materials used in production
    - Source cells
    - In-process control tests
  - Final lot (Identification, Tests, Assays)
- ***Specific requirements***
  - Human haematopoietic stem cells
  - Chondrocytes
  - Limbal stem cells
  - Mesenchymal stem cells

adopted 181st EPC Session

Published 1 October 2025

- Need for a general text covering quality of cell-based preparations
- encompassing products already on the market and new products to come

Scope

- autologous, allogeneic and xenogeneic cell-based preparations
- including living, damaged and dead cells
- Could be applicable to cells used in further processing

- Cells already covered in *Human hematopoetic stem cells (2323)*
- Specific consideration related to genetic modifications and their associated requirements → see GTP texts

Rapid microbiological methods (RMM) in the Ph. Eur.

The Ph. Eur. facilitates the use of RMM

5.1.6 Alternative methods for control of microbiological quality

General principles of alternative microbiological methods
Guidance on how to implement alternative microbiological methods

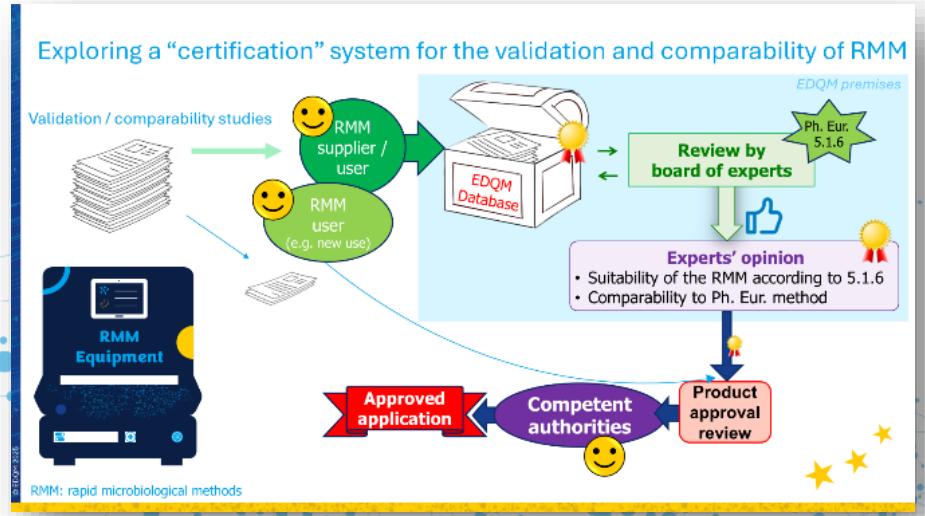
Examples of validation protocols booklet (https://go.edqm.eu/expprotmicrobioe)

2.6.27 Microbiological examination of cell-based preparations

Approaches to microbiological examination of cell-based preparations

5.1.9 Guidelines for using the test for sterility

Reflect the use of alternative methods in accordance with chapter *5.1.6*



- Implementation of RMM remains a challenge due to complex validation and comparability
- EDQM has initiated a project to develop a "*certification*" system for the validation and comparability of RMM to support their implementation based on 5.1.6

swissmedic

Regulatory revisions in Switzerland and International harmonization efforts

Julia Djonova, Head Division Advanced Therapy Medicinal Products

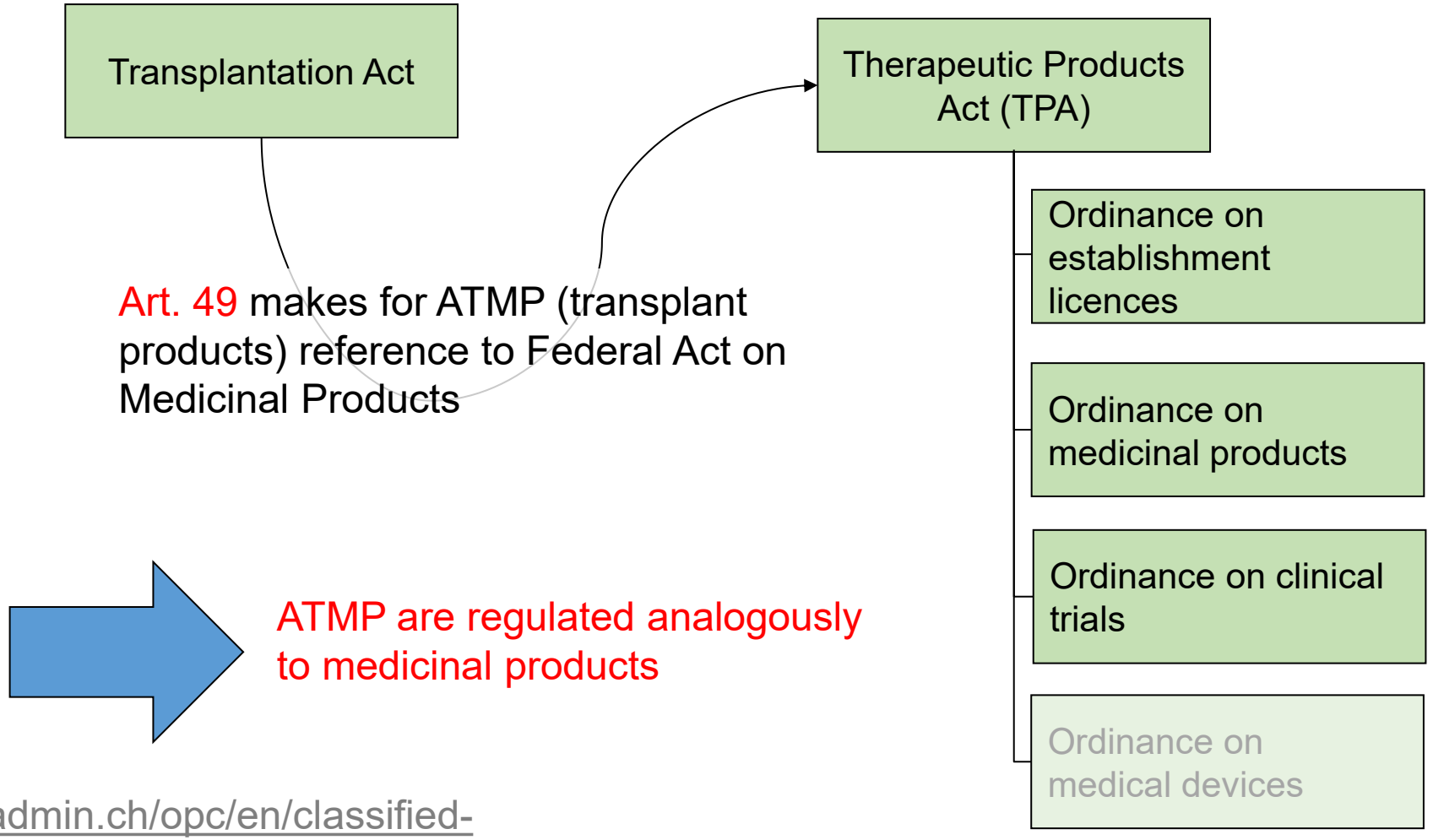
CASSS-CGT

Basel 23-24 October 2025

Schweizerisches Heilmittelinstitut
Institut suisse des produits thérapeutiques
Istituto svizzero per gli agenti terapeutici
Swiss Agency for Therapeutic Products

Hallerstrasse 7, 3012 Bern
www.swissmedic.ch

Current regulation applies to ATMP but is not specific



http://www.admin.ch/opc/en/classified-compilation/20002716/index.html
https://www.fedlex.admin.ch/en/cc/internal-law/81#812

“Intent Document ”on the revision of the law adopted in September 2025 from the Federal Council

- The ATMP’s regulation will be transferred from the Transplantation Act to the Therapeutic Product Act , which will create a specific and comprehensive framework for complex, innovative therapies
- Considering the scientific advances and new technologies in the field of ATMP and updated regulatory expectations around quality, traceability and safety
- Providing biotech innovators with a clearer framework for early development and clinical translation and strengthening the position as a forward-looking environment for ATMP development

What is new – 1

Products with nucleid acides modifications will be introduced as a special category of ATMP's

- Products whose active ingredient contains recombinant biological or synthetic nucleoid acid sequences or genetically modified microorganismes (DNA plasmids , mRNA, iRNA, antisense oligonucleotides, viral vectors containing RNA or DNA, aptamers, CRISPR CAS) with therapeutic or preventive effect

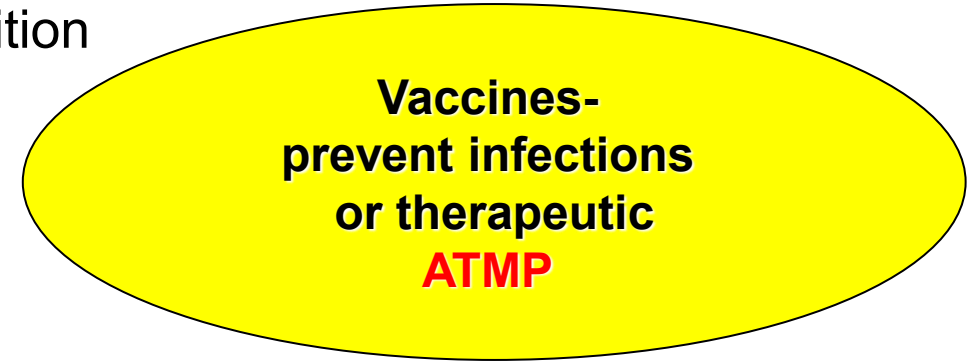
- Genetically modified organisms are organisms whose genetic material has been modified in a way that does not occur under natural conditions through crossing or natural recombination (Swiss Genetic Act and Release Ordinance)

- Excluded are: Nucleic acids which are used only as "adjuvants" and are therefore not directly involved in the intended medical effect of the preparation

- No distinction is made between recombinant or synthetic production, nor between short or long nucleic acid sequences

Why expansion of the definition

- To respond to the new developments, the term nucleic acid products covers more product types than are currently regulated in the EU by Regulation (EC) 1394/2007
- In the near future DNA or RNA products can be produced purely synthetically
- The processes /requirements should be identical for all nucleic acid products containing genetic information, regardless of whether they are used preventively or therapeutically
- Products comparable to ATMP in terms of their functionality and risk profiles serving other purposes (anti-aging, esthetical) will be included in the definition

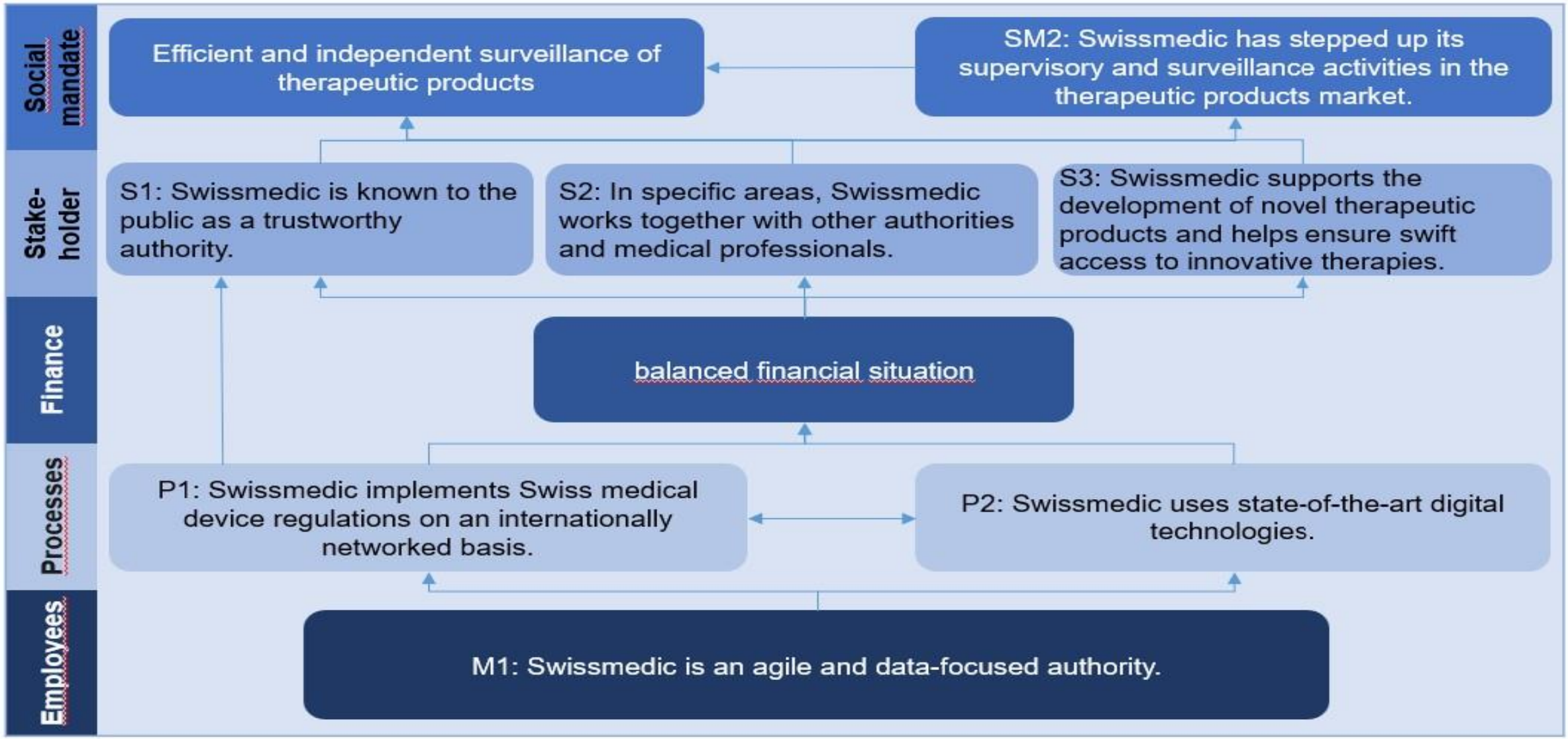


What is new –2

- **Temporary Authorization for the Use of Unapproved Advanced Therapy Medicinal Products (“hospital exemptions”)**
- The development of ATMP has highly experimental nature and often originates in academic research environments
- Most of the products are for rare diseases and are subsequently manufactured on a patient-specific basis or in small quantities, «unmet medical needs»

- **Biovigilance**
- At the time of approval limited information available on benefits and long-term effects - appropriate measures to ensure that long-term treatment and safety data are documented and evaluated (e.g. mandatory Register)

Swissmedic Strategy-Map 2023 – 2026



International Procedures

- **ACCESS Consortium**: Worksharing Initiative with Canada, Australia, Singapore and UK -> Market Size of 160Mio
- **Project Orbis**: collaboration / Parallel Review with FDA
  -> focus on oncologic drugs
- **OPEN** with EMA – just started, pilot during Covid-19 pandemic – focus on PRIME products – partners: Japan, Canada, Australia, Brazil
- **MAGHP** Marketing Authorisation for Global Health Products

International Authorisation Procedures

International collaboration and harmonization

Benefits of work-sharing

- Regulatory convergence , harmonisation
- Sharing and expanding scientific expertise
- Shorter timelines, faster access for patients
- Simultaneous market access in several countries
- Consolidated List of Questions
- Predictability: evaluation plan specified in advance
- Saving resources

Swissmedic supports the early access of ATMP's at all stages

Innovation Office (Start 2023) :

- Regular dialogue with industry, facilitation of international approval procedures
- Scientific advice meetings (Wyss Translation Center, Allschwil Basel , Biopole Lausanne; in 2024 : >50 meetings and 32 «regular» scientifc advice meetings
- The needs of the stakeholders were assessed through an online survey of 39 ATMP companies: further and more intensive dialogue with Swissmedic to reduce uncertainty about the regulatory process and its requirements is welcome; increased demand was observed

Reimbursement: Support on request of FOPH through Early Dialogue

Saudi Food and Drug Authority (SFDA) Regulatory Updates on Advanced Therapies

Wala Turkistani
Head of Advanced Therapies
SFDA

CGTP Europe 2025
23- 24 October 2025
Basel, Switzerland

Supportive Programs

Breakthrough Designation
Accelerate development and review of new drugs that address unmet medical need in the treatment of serious or life-threatening conditions

Enhanced interaction and early dialogue with drug developers, to optimise development plans and speed up evaluation

Research and Investigational Drugs (RAID)
Designation for investigational products that are under development, not yet authorized in Saudi Arabia, and intended for future submission as marketing authorization application (MAA)

Enhanced interaction and early dialogue with drug developers, to optimise development plans and speed up evaluation

Guidance Documents

SFDA Guideline for Technology Transfer of Biological Products *(Under development)*

Clarify the regulatory requirements for the technology transfer of human biological medicinal products and to outline the key technical considerations

Special annex for considerations for tech transfer of ATMPs

Guidance on Submission of Chemistry, Manufacturing, and Control (CMC) Information for Cell-based Clinical Trial Applications

Guidance on Submission of Chemistry, Manufacturing, and Control (CMC) Information for Gene Therapy Clinical Trial Applications

SFDA Guideline on Classification of Advanced Therapy Medicinal Products

Regulatory Support and Innovation

SFDA in National Industrial Development and Logistics Program (NIDLP)

Support the local manufacturing for biological, genetics, and advanced therapy medicinal products (ATMPs)

This initiative contributes to achieving Vision 2030 strategic objective to localize promising manufacturing industries.

- Workshops with stakeholders
- Scientific and regulatory advice
- Training

SFDA Regenerative Medicine Scheme

Regulatory framework governing the classification, evaluation, and oversight of minimally manipulated (MM) cell-based products for human use in Saudi Arabia.

Thank You