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Human Medicines Division

Committee for Advanced Therapies (CAT): Work Plan 2025

Adopted by the Committee on 22 January 2025

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The activities outlined in the CAT work plan for 2025 have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme 2024-2026.

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1. Evaluation activities for human medicines

1.1. Pre-authorisation activities

1.1.1. Guideline on requirements for investigational ATMPs in clinical trials

This guideline provides guidance to ATMP developers to understand the requirements for ATMPs during clinical development. The aim is to facilitate the development of ATMPs and the preparation of EU clinical trial applications.

Key objectives

Following the finalisation of the guideline on quality, non-clinical and clinical requirements for investigational ATMPs in clinical trials, training of assessors and stakeholders will be organised.

Activities in 2025

CAT activities to achieve the objectives set for this area:

- Organise a training on the new guideline for assessors of ATMP clinical trials.
- Organise a webinar on the new guideline for the ATMP developers.
- Prepare a scientific publication on the guideline and the analysis of clinical trials with ATMPs in the EU

CAT will collaborate with the BWP and the Clinical Trial Coordination Group (CTCG) for the organisation of the training.

CAT topic leaders: Ilona Reischl (quality), Claire Beuneu / Rune Kjeken (non-clinical), Olga Kholmanskikh (clinical)

Other committee participants:

Member/alternate	Name	MS
Member	Silke Dorner	Austria
Member	Heli Suila	Finland
Member	Emmely de Vries	Netherlands
Member	Denisa Marilena Margina	Romania
Member	Suzana Vidic	Slovenia
Member	Kieran Breen	Patients' organisation representative
Alternate	Tineke van den Hoorn	Netherlands
Alternate	Ole Henrik Myrdal	Norway

1.1.2. Revision of the Questions and Answers on Gene Therapy

A questions and answer document on matters related to the development of gene therapy medicinal products (EMA/CHMP/GTWP/212377/2008) was published in 2010. This document is in need of revision, to remove topics that are either incorporated in scientific guideline or are no longer relevant, and where needed, to update the responses to reflect the current regulatory position. Additional questions might also be identified.

Key objectives

Revision of the Q&A on gene therapies to reflect the current regulatory position.

Activities in 2025

CAT activities to achieve the objectives set for this area:

- Identify new topics to be included in the Q&A (by Q2 2025)
- Update the existing Q&A and draft new Q&A (by Q4 2025)

CAT topic leader: Claire Beuneu

Other committee participants:

Member/alternate	Name	MS
CAT chair	Ilona Reischl	Austria
Member	Violaine Closson Carella	France
Alternate	Barbara Bonamassa	Italy
Alternate	Tineke van den Hoorn	Netherlands
Alternate	Maria Isabel Vieira	Portugal
Alternate	Marcos Timon	Spain
Alternate	Alessandra Renieri	Clinicians' representative
Alternate	Federica Chiara	Patients' organisation representative

1.1.3. Development of guidance on Genome editing

Genome editing is becoming an established technology in ATMP development. Clinical trials with *in vivo* and *in vitro* genome editing products are taking place and the first product based on cells genetically modified by means of *in vitro* genome editing has been authorised. Some guidance has been included on *in vitro* genome editing in the *Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells* (EMA/CAT/BTWP/671639/2008 Rev.1 – corr): this might need updating in the light of the experience gained and the evolution of science. For *in vivo* genome editing, limited guidance is currently included in the published guidelines and it is therefore relevant to initiate the development of regulatory guidance on *in vivo* genome editing.

Key objectives

To reflect on regulatory requirement for *in vitro* and *in vivo* genome editing.

Activities in 2025

- Organise a workshop to support the development of the concept paper (for a guideline) or reflection paper (Q2/Q3 2025)
- Initiate the development of a concept paper / reflection paper on genome editing (Q4 2025)

CAT will collaborate with BWP for the organisation of the workshop and the development of the quality aspects of the concept paper or reflection paper on genome editing.

CAT topic leader: Emmely de Vries / Tineke van den Hoorn

Other committee participants:

Member/alternate	Name	MS
Member	Martin B Oleksiewicz	Denmark
Member	Rune Kjeken	Norway
Alternate	Marcos Timon	Spain
Alternate	Alessandra Renieri	Clinicians' representative

1.1.4. Enabling the safe and responsible use of Artificial Intelligence in the medicine lifecycle

The European Medicines Regulatory Network (EMRN), through the Big Data Steering Group's oversight, developed a multi-annual workplan on AI to enable the safe and responsible use of AI in the medicine lifecycle and extract value for public and animal health. In addition, the Network strategy to 2028 reflects on the need to focus on improving decision-making, optimising processes and increasing efficiency through the use of data, digitalisation and AI.

Key objectives

- Continued overview of the developments in the multi-annual AI workplan, in particular on guidance, policy and product support, change management and experimentation and implementation of AI
- Ensure coordination of change management activities, including information and training needs on AI, in particular generative AI solutions
- Build assessors knowledge and experience by sharing of case studies of applications of AI across the medicine's lifecycle, in particular non-clinical, clinical, safety, quality, product information and general knowledge mining in regulatory science.

Activities in 2025

CAT activities to achieve the objectives set for this area (in collaboration with CHMP and PRAC):

- Develop knowledge sharing and training material on AI, in particular generative AI, including on ethical and data protection issues, to support assessors.
- Onboard a group of experts on AI at CAT to support the committee, and interact with working parties, on regulatory science topics related to AI.
- Establish a procedure to strengthen the EMA support on product-specific AI topic discussions.
- Establish and maintain a cooperation on AI experimentation to improve efficiency and quality of decision-making.

CAT topic leader: Dariusz Sladowski

Other committee participants:

Member/alternate	Name	MS
Member	Kieran Breen	Patients' organisation representative
Alternate	Bernd Gänsbacher	Doctors' representative
Expert	Torbjörn Callréus	Malta

1.2. Initial evaluation activities

1.2.1. Benefit/Risk methodology and communication

Benefits and risks require continuous evaluation throughout the lifecycle of a medicine. The objective is to balance benefits and risks in a way that is as robust, consistent and as transparent as possible.

Key objectives

- Facilitate the exchange of experiences and best practices in benefit-risk evaluation to enhance consistency and how this is communicated in the assessment reports

Activities in 2025

CAT activities to achieve the objectives set for this area:

- To develop knowledge sharing and training material for the EMRN to implement the reflection paper on single-arm trials that are submitted as pivotal evidence in marketing authorisation dossiers across therapeutic areas
- Promote development and implementation of best practices for the assessment and communication of the benefit:risk in the new optimised assessment report templates (contribution to CHMP).

CAT topic leader: Jan Mueller-Berghaus

Other committee participants:

Member/alternate	Name	MS
Member	Maria Gazouli	Greece
Member	Emmely de Vries	Netherlands
Member	Maria Lüttgen	Sweden

1.2.2. Real world data (RWD) in regulatory decision making of ATMPs

RWD are used in the development, authorisation, safety and efficacy follow-up and monitoring of ATMPs. Enhanced analysis of RWD has the potential to further support regulatory decision-making and offers the possibility to provide an additional perspective on the use and performance of medicines in everyday clinical use, complementing the evidence obtained from clinical trials.

Key objective

To expand further the use of RWD including natural history data, patient treatment data, etc. from registries or other valid sources to support regulatory decision making pre-and post-authorisation and in-patient access to ATMPs.

Activities in 2025

CAT activities to achieve the objectives set for this area:

- Continue the conduct of RWD studies to support CAT decision-making thanks to identification of potential use cases.
- Upon consultation, CAT will provide expert input to the yearly review of the experience gained with RWD studies conducted across the regulatory network to support regulatory decision making and provide expert input in support of the development of guidance on use of real world evidence (RWE) for regulatory purpose.
- Participate as experts in the DARWIN EU® activities that has the scope to generate robust and reliable RWE using appropriate data partners.

CAT topic leader: Kieran Breen

Other committee participants:

Member/alternate	Name	MS
Member	Rozalina Kulaksazova	Bulgaria
Member	Emmely de Vries	Netherlands
Member	Kerstin Sollerbrant	Patients' organisation representative
Alternate	Olga Kholmanskikh	Belgium
Alternate	Alessandra Renieri	Clinicians' representative
Alternate	Federica Chiari	Patients' organisation representative
Alternate	Mencía de Lemus Belmonte	Patients' organisation representative

Additional experts from the assessment teams of ATMP marketing authorisation applications will contribute to this activity.

1.2.3. Scientific consultation involving other decision makers to facilitate optimisation of clinical evidence generation in drug development programmes

Clinical evidence generated during drug development is intended to serve different decision making. It is therefore desirable that evidence requirements do address regulatory needs as well as those of other down-stream decision makers.

Key objectives

- Engage with down-stream decision makers with the aim of improving the clinical evidence generation for ATMPs.
- To prospectively identify post-licensing evidence needs considering the expected evidence available at time of initial decision making by regulators and HTAs, respectively.

Activities in 2025

CAT activities to achieve the objectives set for this area:

- Collaborate with the Member State Coordination Group on HTA (HTACG) on prospective evidence planning for development programmes through provisions of parallel joint scientific consultation under new HTA Regulation.
- Review experience with evidence at time of regulatory and HTA decision making, respectively, to inform prospective guidance on future developments.

- In view of the initial scope of the HTA Regulation, engage with HTAs through product-specific discussions on newly approved ATMPs.

CAT topic leader: Maria Lüttgen

Other committee participants:

Member/alternate	Name	MS
CAT chair	Ilona Reischl	Austria
Member	Rune Kjeken	Norway
Member	Rapporteurs of approved ATMP	
Member	CoRapporteurs of approved ATMP	
Member	Kerstin Sollerbrant	Patients' organisation representative
Alternate	Mencia de Lemus Belmonte	Patients' organisation representative

1.2.4. Provision of information to support Joint Clinical Assessments

Activity areas

The Regulation on Heath Technology Assessment (Regulation EU 2021/2282) will come into application in January 2025. One of the areas for regulatory/HTA collaboration under this Regulation concerns the provision of information from the regulatory assessment of an application for marketing authorisation or extension of indication for medicinal products to inform the respective Joint Clinical Assessment (JCA) for this technology performed by the Member State Coordination Group on HTA (HTACG) and its subgroups. The reason being that the assessment scope for JCA is based on the therapeutic indication of the medicinal product. Therefore, while preserving the separation of the respective remits of the Coordination Group and the European Medicines Agency, information shall be provided via the respective secretariats. The Implementing Regulation on JCA for medicinal products (Commission Implementing Regulation (EU) 2024/1381) specifies further the type of information provided.

Key objectives

- To provide relevant and necessary information from the regulatory assessment to support the preparation of Joint Clinical Assessments for ATMPs led by the HTACG.

Activities in 2025

- Development of guidance on provision of information on substantial questions or outstanding issues from the CAT/CHMP review that might impact the JCA assessment scope, in collaboration with the HTA secretariat and the JCA Subgroup (support to CHMP).

CAT topic lead: Charlotte Anderberg

Other committee participants:

Member/alternate	Name	MS
CAT chair	Ilona Reischl	Austria

1.2.5. Implementation of the medical device regulation and strengthening of the assessment of Companion Diagnostics

The legislation for medical devices and in-vitro diagnostics requires reflection by CAT in relation to their implementation for the field of ATMPs. Specifically, per the Regulation (EU) 2017/746, a companion diagnostic (CDx) is defined as essential for defining patients' eligibility for specific treatment with a medicinal product. As part of the conformity assessment of a CDx, the notified body is therefore required to seek a scientific opinion on the suitability of the CDx with the concerned medicinal product(s) from the competent authorities in accordance with Directive 2001/83/EC before issuing an EU technical documentation assessment certificate or an EU type-examination certificate, or a supplement to them for the CDx. Whilst a process has been developed in 2022, the CAT in collaboration with the CHMP seeks to strengthen the assessment process.

Key objectives

- Collaborate in the framework for identification of overarching issues in the assessment of CDx consultation procedures.
- Continued identification of general principles that can be later used for training of assessment teams and to update if needed procedural guidance or assessment templates.
- Reflect on the implications of the MDR on the development and authorisation of (combined) ATMPs.

Activities in 2025

CAT activities to achieve the objectives set for this area (in collaboration with CHMP):

- Collaborate with the CDx expert group to consolidate the evaluation of consultation procedures across committee members.
- Monitor assessments to capture input in CAT procedures at initial MAAs.
- Collaborate with MWP on the guideline on biomarker development

CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
Member	Silke Dorner	Austria
Member	Heli Suila	Finland
Member	Violaine Closson Carella	France
Member	Jan Mueller-Berghaus	Germany
Member	Una Riekstina	Latvia
Alternate	Olga Kholmanskikh	Belgium
Alternate	Liga Kunrade	Latvia

1.3. Post-authorisation activities

1.3.1. Post-authorisation safety and efficacy follow-up and RMP for ATMPs

Post-authorisation follow-up of patients treated with ATMPs is essential to collect data on long-term safety and efficacy of the authorised ATMP. It is essential that appropriate guidance is available to

ATMP developers in order that appropriate post-authorisation studies can be planned that allow the generation of information.

Key objectives

Develop guidance on post-authorisation follow-up and risk management planning for ATMPs, taking into account the need to adapt regulatory requirements of patient follow-up to incremental scientific knowledge and clinical experience.

Activities in 2025

CAT activities to achieve the objectives in this area:

- Review and analyse the comments (related to ATMPs) received during the public consultation on the guideline on safety and efficacy follow-up and risk management of ATMPs and initiate the revision of the GVP Module V to incorporate ATMP specific information based on the feedback during the public consultation. This activity will be undertaken in collaboration with PRAC.
- Initiate a reflection on the duration of follow-up of patients treated with CAR-T cells.

CAT topic leader: Olga Kholmanskikh

Other committee participants:

Member/alternate	Name	MS
Member	Concetta Quintarelli	Italy
Member	Rune Kjeken	Norway
Alternate	Egbert Flory	Germany
Expert	Torbjörn Callréus	Malta

2. Horizontal activities and other areas

2.1. Partners and stakeholders

2.1.1. Interaction with Stakeholders

Engagement with ATMP developers, both from industry and not-for-profit organisations, and with patient organisations is important to ensure a mutual understanding of important issues affecting ATMP development and approval. Enhanced collaboration between regulators and developers will also allow to identify potential guidance and/or training needs with the aim of improving the access and use of ATMPs by patients.

Key objectives

Engage with key stakeholders from industry, academia, not-for-profit and patient organisations.

Activities for 2025

- Organise a meeting with the CAT stakeholders focussing on current scientific and regulatory issues related to the development of ATMPs
- Organise a regulatory session jointly with one of the academic learned societies.
- Contribute to a meeting with Patients' organisations

CAT topic leader: Dariusz Sladowski

Other committee participants:

Member/alternate	Name	MS
CAT chair	Ilona Reischl	Austria
Member	Kerstin Sollerbrant	Patients' organisation representative

2.1.2. International Regulatory Science Collaboration

ATMP development has become a global activity which can be facilitated by international harmonisation and convergence of regulatory requirements. CAT will play an active role in the development of international guidance documents for ATMPs.

Key objectives

Offer the best scientific support to international convergence / harmonisation activities related to ATMPs.

Activities for 2025

- Support to ICH Gene and Cell therapy drafting group (CAT Representatives: Jan Mueller Berghaus)
- Support to WHO activities (lead: Ilona Reischl)
- Support to IPRP activities (lead: Pille Säälik)
- Seek opportunities for enhanced collaborations and exchanges of scientific knowledge with international partners

CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
Member	Claire Beuneu	Belgium
Member	Jan Mueller-Berghaus	Germany
Alternate	Pille Säälik	Estonia
Alternate	Tineke van den Hoorn	Netherlands
Alternate	Isabel Vieira	Portugal