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Human Medicines Division

Committee for Advanced Therapies (CAT): Work Plan 2024

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The activities outlined in the CAT work plan for 2024 have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme 2022-2024.

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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

Development of a guideline on quality, non-clinical and clinical requirements for investigational ATMPs in support of clinical trial applications.

Activities in 2024

CAT activities to achieve the objectives set for this area:

- Finalise the guideline after the second external consultation.
- Organise a training on the new guideline for assessors of ATMP clinical trials.

CAT will collaborate with the BWP for the development of the quality part of this guideline.

CAT topic leaders: Ilona Reischl (quality), Claire Beuneu / Rune Kjekken (non-clinical), Alessandro Aiuti (clinical)

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Ilona Reischl	CAT chair
CAT topic leader	Claire Beuneu	Belgium
CAT topic leader	Rune Kjekken	Norway
CAT topic leader	Alessandro Aiuti	Clinicians' representative
Member	Silke Dorner	Austria
Member	Heli Suila	Finland
Member	Violaine Closson-Carella	France
Member	Concetta Quintarelli	Italy
Member	Silviu Istrate	Romania
Member	Metoda Lipnik-Stangelj	Slovenia
Member	Kieran Breen	Patients' organisation representative
Alternate	Barbara Bonamassa	Italy
Alternate	Tineke van den Hoorn	Netherlands
Alternate	Maria Isabel Vieira	Portugal
Alternate	Suzana Vidic	Slovenia
Expert	Maura O'Donovan	Ireland
Expert	Marcel Hoefnagel	Netherlands
Expert	Andreaa Barbu	Sweden

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 2024

CAT activities to achieve the objectives set for this area:

- Identify the questions and answer that are obsolete or in need for revision, and new topics to be included in the Q&A (by Q2 2024)
- Update the existing Q&A and draft new Q&A (by Q4 2024)

CAT topic leader: Claire Beuneu

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Claire Beuneu	Belgium
CAT chair	Ilona Reischl	Austria
Member	Violaine Closson Carella	France
Member	Marcos Timon	Spain
Alternate	Kristyna Rehorova Hradilkova	Czechia
Alternate	Tineke van den Hoorn	Netherlands
Alternate	Maria Isabel Vieira	Portugal
Alternate	Alessandra Renieri	Clinicians' representative

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

- Continued review of developments in assessing and communicating benefits and risks. Develop training material about assessing and communicating benefits and risks.
- Build assessors' knowledge and experience with different approaches and describing value-judgments in the current benefit risk assessment framework/template.

Activities in 2024

CAT activities to achieve the objectives set for this area:

- To contribute to the finalisation of the reflection paper on single-arm trials that are submitted as pivotal evidence in marketing authorisation dossiers across therapeutic areas following the public consultation.
- To produce training material on benefit-risk assessment and communication in the new optimised assessment report templates.
- Set up a focus group in collaboration with CHMP to explore the usefulness of preference elicitation in the context of advisory meetings with experts (e.g. SAGs, AHEGs).

CAT topic leader: Jan Mueller-Berghaus

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Jan Mueller-Berghaus	Germany
Member	Alessandro Aiuti	Italy
Member	Maria Gazouli	Greece
Member	Emmely de Vries	Netherlands
Expert	Maura O'Donovan	Ireland

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 objectives

To further the understanding of the use of RWD including natural history data, retrospective patient level treatment data and registry-based data in regulatory decision making pre-and post-authorisation and in-patient access to ATMPs.

Activities in 2024

CAT activities to achieve the objectives set for this area:

- Continue the pilot on RWE studies to support CAT decision-making including identification of use cases.
- Upon consultation, CAT will provide expert input to a continuous review of the experience gained with RWE 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 RWE for regulatory purpose.
- Participate as experts in the DARWIN EU® activities that has the scope to perform and follow-up real world evidence (RWE) studies using selected data partners.

CAT topic leader: Mencia de Lemus Belmonte

Other committee participants:

Member/alternate	Name	MS
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Member/alternate	Name	MS
CAT topic leader	Mencia de Lemus Belmonte	Patients' organisation representative
Member	Rozalina Kulaksazova	Bulgaria
Member	Emmely de Vries	Netherlands
Member	Alessandro Aiuti	Clinicians' representative
Member	Kieran Breen	Patients' organisation representative
Alternate	Olga Kholmanskikh	Belgium
Alternate	Alessandra Renieri	Clinicians' representative
Expert	Maura O'Donovan	Ireland

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

1.2.3. Improve interactions with Health Technology Assessment (HTA) bodies to optimise clinical evidence generation

Recognising that the regulatory outcome is the first important step along the path for access to patients, it is important that there is mutual understanding and appropriate knowledge sharing between decision makers. It is expected that fostering exchanges between regulators and down-stream decision makers on product specific matters will enhance the access to innovative medicines for patients. Whilst scientific advice on evidence requirements for regulatory purpose is well established, in recent years the opportunities for engagement with additional stakeholders during such discussions have been increasingly recognised. The new HTA Regulation, which will apply from January 2025, includes ATMPs in the initial scope for Joint Clinical Assessment by the new HTA structure.

Key objectives

Engage with down-stream decision makers with the aim of improving the clinical evidence generation for ATMPs.

Activities in 2024

CAT activities to achieve the objectives set for this area:

- Collaborate with HTA bodies on prospective evidence planning for development programmes through provisions of parallel EMA/HTA scientific advice until the new HTA Regulation is in operation.
- 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 topic leader	Maria Lüttgen	Sweden
Member	Rapporteurs of approved ATMP	
Member	CoRapporteurs of approved ATMP	
Alternate	Mencia de Lemus Belmonte	Patients' organisation representative

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

New 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 2024

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.
- Update the procedure for consultation of notified bodies during the assessment of combined ATMPs.

CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Ilona Reischl	CAT chair
Member	Silke Dorner	Austria
Member	Heli Suila	Finland
Member	Violaine Closson Carella	France
Member	Jan Mueller-Berghaus	Germany
Alternate	Olga Kholmanskikh	Belgium
Expert	Maura O'Donovan	Ireland

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 2024

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.
- Prepare (a) scientific publication(s) on the follow-up of patients treated with CAR-T cells.

CAT topic leader: Olga Kholmanskikh

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Olga Kholmanskikh	Belgium
Member	Concetta Quintarelli	Italy
Member	Rune Kjeklen	Norway
Member	Kerstin Sollerbrandt	Patients' organisation representative
Alternate	Egbert Flory	Germany
Expert	Maura O'Donovan	Ireland
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 2024

- Organise a meeting with the CAT stakeholders
- 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 topic leader	Dariusz Sladowski	Poland
CAT chair	Ilona Reischl	Austria
Member	Kerstin Sollerbrandt	Patients' organisation representative

2.1.2. Scientific symposium on the future of ATMPs

CAT has become an international benchmark for the evaluation and authorisation of ATMPs. In 2024, CAT will exist for 15 year and this calls for forward looking on what the future will bring for ATMP development and for the access of patients to these novel therapies.

Key objectives

Organise a scientific symposium on the work of the CAT and the future of ATMPs.

Activities for 2024

- Organisation of a scientific symposium with contributions from experts in the field, CAT stakeholders from industry and academia and previous CAT chairs.
- As part of the scientific symposium, communicate on the work of the CAT and its achievements. The aim is to reach out to the public and patients (by means of a video recording) and to the developers and academia (by means of a scientific publication).

CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Ilona Reischl	CAT chair
Member	Violaine Closson Carella	France
Member	Kieran Breen	Patients' organisation representative
Alternate	Olga Kholmanskikh	Belgium
Alternate	Isabel Vieira	Portugal

2.1.3. 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 2024

- 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 Säälük)
- 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
CAT topic leader	Ilona Reischl	Austria
Member	Claire Beuneu	Belgium
Member	Jan Mueller-Berghaus	Germany
Alternate	Pille Säälük	Estonia
Alternate	Tineke van den Hoorn	Netherlands
Alternate	Isabel Vieira	Portugal