EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Division

Committee for Advanced Therapies (CAT): Work Plan 2023

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The activities outlined in the CAT work plan for 2023 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 2023

CAT activities to achieve the objectives set for this area:

- Finalise the guideline after external consultation.

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 Kjeken (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 Kjeken	Norway
CAT topic leader	Alessandro Aiuti	Clinicians' representative
Member	Heli Suila	Finland
Member	Violaine Closson-Carella	France
Member	Maura O'Donovan	Ireland
Member	Concetta Quintarelli	Italy
Member	Carla Herberts	Netherlands
Member	Silviu Istrate	Romania
Member	Metoda Lipnik-Stangelj	Slovenia
Member	Kieran Breen	Patients' organisation representative
Alternate	Silke Dorner	Austria
Alternate	Barbara Bonamassa	Italy
Alternate	Maria Isabel Vieira	Portugal
Expert	Ivana Haunerova	Czech Republic
Expert	Marcel Hoefnagel	Netherlands

1.1.2. New Active Substance (NAS) status of ATMPs

Compared to chemical or biological substances, specific considerations are needed to establish the NAS status of ATMPs. The reflection paper on criteria to be considered for the evaluation of NAS status of biological substances encompasses ATMPs and provides guidance on the elements required to be submitted by applicants to substantiate a NAS claim and describes the criteria that should be applied during NAS assessment.

Key objectives

To provide guidance on the criteria to be considered for the evaluation of the NAS status of ATMPs.

Activities in 2023

CAT activities to achieve the objectives set for this area:

- Review, analyse and address where appropriate the comments (related to ATMPs) received during the public consultation of the draft reflection paper on criteria to be considered for the evaluation of new active substance (NAS) status of biological substances;
- Contribute to the finalisation of the reflection paper on criteria to be considered for the evaluation of new active substance (NAS) status of biological substances.

CAT will provide input on ATMP specific aspects to the BWP for the finalisation of this reflection paper.

CAT topic leader: Marcos Timon

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Marcos Timon	Spain
Member	Ilona Reischl	CAT chair
Member	Heli Suila	Finland
Alternate	Niamh Curran	Ireland
Alternate	Barbara Bonamassa	Italy
Expert	Jürgen Scherer	Germany
Expert	Marja van der Bovenkamp	Netherlands

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 overview of developments in assessing and communicating benefits and risks.
- To pilot the feasibility of using a more explicit approach in describing value-judgments in the current benefit risk assessment framework/template

Activities in 2023

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.
- Initiate a pilot in collaboration with CHMP to quantify impact of expert elicitations in the context of hypothetical SAGs and determine in SAGs deliver a more comprehensive advice to committee.

CAT topic leader: Carla Herberts

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Carla Herberts	Netherlands
Member	Maura O'Donovan	Ireland
Member	Alessandro Aiuti	Italy
Member	Maria Gazouli	Greece

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 2023

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.
- Finalise the CAT expert support to the first EMA-funded pilot study, a registry-based cohort study of Spinal muscular atrophy (SMA) disease, with the aim to describe the natural history of SMA, the evolution of SMA care management over time and disease progression considering the new disease modifying therapies (including ATMPs) based on real world data from EU registries.
- Upon consultation, CAT will provide expert input to a 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: Maura O'Donovan

Member/alternate	Name	MS
CAT topic leader	Maura O'Donovan	Ireland
Member	Carla Herberts	Netherlands
Member	Lisbeth Barkholt	Sweden
Member	Alessandro Aiuti	Clinicians' representative
Member	Kieran Breen	Patients' organisation representative
Alternate	Alessandra Renieri	Clinicians' representative
Alternate	Mencia 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. 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.

Key objectives

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

Activities in 2023

CAT activities to achieve the objectives set for this area:

- Improve interactions with HTAs through product-specific discussions on newly approved ATMPs: set up meetings/webinars to present to HTA the scientific grounds for the approval.

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	

1.2.4. Strengthen 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 following first procedures received in 2022.

Key objectives

Reflection on the implications of the new Medical Device (MD) / In vitro Diagnostics Regulations (IVD) on ATMP development.

- Create a framework for identification of overarching issues in the assessment of CDx consultation procedures.
- Identify general principles that can be later used for training of assessment teams and to update if needed procedural guidance or assessment templates.

Activities in 2023

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

- Contribute to the implementation and enhancement of a process and documents related to companion diagnostics consultation process.
- Review experience gained with medicinal products involving biomarker identification and record learnings.

CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Ilona Reischl	CAT chair
Member	Heli Suila	Finland
Member	Violaine Closson Carella	France
Member	Maura O'Donovan	Ireland
Member	Jan Mueller-Berghaus	Germany
Member	Carla Herberts	Netherlands
Member	Lisbeth Barkholt	Sweden

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 2023

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 AAV-based gene therapies and patients treated with CAR-T cells.

CAT topic leader: Carla Herberts

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Carla Herberts	Netherlands
Member	Maura O'Donovan	Ireland
Member	Concetta Quintarelli	Italy
Member	Kerstin Sollerbrandt	Patients' organisation representative
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 2023

- Organise a meeting with the CAT stakeholders in the first half of 2023
- Organise a regulatory session jointly with one of the academic learned societies.
- Contribute to a meeting with Patients' organisations due to take place in the second half of 2023.

CAT topic leader: Dariusz Sladowski

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Dariusz Sladowski	Poland
Member	Ilona Reischl	CAT chair
Member	Kerstin Sollerbrandt	Patients' organisation representative