21 January 2022
EMA/CAT/716769/2021
Human Medicines Division

Committee for Advanced Therapies (CAT): Work Plan 2022

adopted by the Committee on 21 January 2022

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The activities outlined in the CAT work plan for 2022 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 ATMPs in clinical trials

This guideline will help 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 applications for clinical trials for ATMPs.

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Finalise the guideline after external consultation (held from 1 February to 1 August 2019).

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

CAT topic leaders: Tiina Palomäki, Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Tiina Palomäki	FI
CAT topic leader	Ilona Reischl	AT
CAT Chair	Martina Schüssler-Lenz	DE
Member	Heli Suila	FI
Member	Violaine Closson-Carella	FR
Member	Maura O'Donovan	IE
Member	Concetta Quintarelli	IT
Member	Carla Herberts	NL
Member	Metoda Lipnik-Stangelj	SI
Member	Kieran Breen	Patients' organisation representative
Alternate	Silke Dorner	AT
Alternate	Barbara Bonamassa	IT
Alternate	Maja Sommerfelt Grønvold	NO
Alternate	Maria Isabel Vieira	PT
Expert	Ivana Haunerova	CZ
Expert	Marcel Hoefnagel	NL

1.1.2. Guideline on non-clinical biodistribution studies for gene therapy products

This guideline will provide support in the design of the non-clinical development programme for gene therapy medicinal products.

Key objectives

Development of the ICH-S12 guideline on non-clinical biodistribution studies for gene therapy products.

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Provide dedicated support from CAT to the appointed EU Rapporteurs of the ICH S12 guideline

CAT topic leaders: Rune Kjeken, Claire Beuneu (EU Rapporteurs)

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Rune Kjeken	NO
CAT topic leader	Claire Beuneu	BE
Member	Toivo Maimets	EE
Alternate	Egbert Flory	DE
Alternate	Isabel Vieira	PT
Expert	Brigitte Anliker	DE
Expert	Tineke van den Hoorn	NL

1.1.3. New Active Substance (NAS) status of ATMPs

Compared to chemical or biological substances, specific considerations are needed to establish the NAS status of ATMPs. This reflection paper will provide the criteria that should be applied.

Key objectives

To define criteria for the determination of the NAS status of ATMPs.

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Review and analyse 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;
- Contribution 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 development of this reflection paper.

CAT topic leader: Marcos Timon

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Marcos Timon	ES
Member	Ilona Reischl	AT
Member	Heli Suila	FI
Alternate	Niamh Curran	IE
Alternate	Barbara Bonamassa	IT
Expert	Jürgen Scherer	DE

Member/alternate	Name	MS
Expert	Marja van der Bovenkamp	NL

1.1.4. 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 2022

CAT activities to achieve the objectives set for this area:

- Investigate patients' course of disease and standards of care delivery over time in an ATMP relevant disease entity based on real world data from EU registries
- Develop research questions and contribute to an EMA-funded study in an ATMP relevant disease entity to explore scientific and operational aspects

CAT topic leader: Martina Schüssler-Lenz

Member/alternate	Name	MS
CAT topic leader	Martina Schüssler-Lenz	DE
Member	Maura O'Donovan	IE
Member	Romaldas Maciulaitis	LT
Member	Carla Herberts	NL
Member	Lisbeth Barkholt	SE
Member	Alessandro Aiuti	Clinicians' representative
Member	Kieran Breen	Patients' organisation representative

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

1.2. Initial evaluation activities

1.2.1. Comprehensiveness of clinical data in marketing authorisations

Criteria have been developed to evaluate the comprehensiveness of clinical data. CAT will review the use of the comprehensiveness criteria in marketing authorisation evaluations between 2021 and end of 2022.

Key objectives

Review of the criteria of comprehensiveness of clinical data in marketing authorization applications.

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Analyse the use and adherence to the comprehensiveness criteria (as adopted by CAT/CHMP in June 2021) in all ATMP marketing authorisation applications from Q3 2021 to Q4 2022;
- Report to CAT for discussion and if needed adaptation of the comprehensive criteria.

CAT topic leader: Martina Schüssler-Lenz

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Martina Schüssler-Lenz	DE
Member	Jan Mueller-Berghaus	DE
Member	Romaldas Mačiulaitis	LT
Member	Carla Herberts	NL
Member	Rune Kjeken	NO
Member	Sol Ruiz	ES

1.3. Post-authorisation activities

1.3.1. 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 post-authorisation evidence generation for ATMPs.

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Improve interactions with HTAs;
- Collaborate with HTA bodies on prospective evidence planning for development programmes through provisions of parallel EMA/HTA scientific advice during the transition phase until the new HTA Regulation is in operation;
- Contribute to a pilot on a specific project related to an ATMP.

This topic will be developed in collaboration with CHMP (CHMP work plan topic: Multi-stakeholder consultations to facilitate optimisation of clinical evidence generation in drug development programmes).

CAT topic leader: Martina Schüssler-Lenz

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Martina Schüssler-Lenz	DE
Member	Jan Mueller-Berghaus	DE
Member	Maura O'Donovan	IE
Member	Dariusz Sladowski	PL
Member	Kerstin Sollerbrant	Patients' organisation representative
Alternate	Maria Lüttgen	SE

1.4. Other specialised areas and activities

1.4.1. Implementation of the Medical Device and in-vitro diagnostics Regulations

New legislation for medical devices and in-vitro diagnostics requires reflection by CAT on their implications for the field of ATMPs.

Key objectives

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

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Revision/updating of the '*Procedural advice on the evaluation of combined advanced therapy medicinal product and the consultation of Notified Bodies in accordance with Article 9 of Regulation (EC) No. 1394/2007*' (EMA/354858/2010) in the light of the new MD Regulation;
- Contribute to the development of the process and guidelines related to the IVD consultation process.

CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Ilona Reischl	AT
Member	Heli Suila	FI
Member	Violaine Closson Carella	FR
Member	Maura O'Donovan	IE
Member	Jan Mueller-Berghaus	DE
Member	Carla Herberts	NL
Member	Lisbeth Barkholt	SE

2. Horizontal activities and other areas

2.1. Partners and stakeholders

2.1.1. Revision of the pharmaceutical legislation

The European Commission has earmarked the pharmaceutical legislation for revision. CAT will provide input to EMA and the European Commission to ensure that the specificities of ATMPs are adequately reflected in the revised legislation.

Key objectives

Support EMA and the European Commission on any proposals for legislative changes with relevance to ATMPs.

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Provide input on proposals identified as CAT priority topics and contribute to other proposals potentially relevant to ATMPs.

CAT topic leader: Martina Schüssler-Lenz

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Martina Schüssler-Lenz	DE
Member	Ilona Reischl	AT
Member	Claire Beuneu	BE
Member	Violaine Closson Carella	FR
Member	Concetta Quintarelli	IT
Member	Rune Kjeken	NO
Member	Dariusz Sladowski	PL
Member	Marcos Timon	ES
Member	Kieran Breen	Patients' organisation representative
Alternate	Silke Dorner	AT
Alternate	Barbara Bonamassa	IT
Alternate	Maria Lüttgen	SE
Expert	Jürgen Scherer	DE
Expert	Marcel Hoefnagel	NL
Expert	Martijn van der Plas	NL

2.1.2. Training activities

Training of both the EU network (assessor and experts) and the ATMP developers and stakeholders is identified as an important initiative to support ATMP development, authorisation and patient access.

Key objectives

Training and education on development and assessment of ATMPs.

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Provide training to the EU network on assessment of marketing authorisation applications of ATMPs;
- Develop training for developers (academic, SME and other), stakeholders and outside partners on the development of ATMPs.

CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Ilona Reischl	AT
CAT chair	Martina Schüssler-Lenz	DE
Member	Concetta Quintarelli	IT
Member	Una Riekstina	LV
Member	Dariusz Sladowski	PL
Member	Alessandro Aiuti	Clinicians' representative
Alternate	Isabel Vieira	PT
Alternate	Roland Pochet	Patients' organisation representative

2.1.3. International 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

Contribute to the development of international guidance or convergence documents related to ATMPs.

Activities in 2022

CAT activities to achieve the objectives set for this area:

- Provide CAT input in international harmonisation and convergence activities by WHO, ICMRA, ICH or IPRP, as appropriate.

CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Ilona Reischl	AT
CAT chair	Martina Schüssler-Lenz	DE
Alternate	Pille Säälik	EE
Expert	Ivana Haunerova	CZ