



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Division

CAT work plan 2021

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The activities outlined in the CAT work plan for 2021 have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme 2021-2023.

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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

Key objectives

Development of a guideline on quality, non-clinical and clinical requirements for applications for clinical trials for ATMPs.

Activities in 2021

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.

EMA topic leader: Patrick Celis; CAT topic leaders: Tiina Palomäki, Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Tiina Palomäki	Finland
CAT topic leader	Ilona Reischl	Austria
CAT Chair	Martina Schüssler-Lenz	Germany
Member	Ivana Haunerova	Czech Republic
Member	Heli Suila	Finland
Member	Violaine Closson-Carella	France
Member	Maura O'Donovan	Ireland
Member	Carla Herberts	Netherlands
Member	Metoda Lipnik-Stangelj	Slovenia
Member	Kieran Breen	Patients' organisation representative
Expert	Guiseppa Pistritto	Italy
Expert	Marcel Hoefnagel	Netherlands

1.1.2. Comprehensiveness of clinical data in marketing authorisations

Key objectives

Reinforce, if necessary, update, the common understanding of the comprehensiveness of data in CAT and CHMP.

Activities in 2021

CAT/CHMP inter-committee ad hoc group on comprehensiveness will:

- Identify key issues and collect different views.
- Develop consensus positions or clearly outline alternative positions
- Report to CAT/CHMP for discussion and adoption

EMA topic leader: Ana Hidalgo Simon; CAT topic leader: Martina Schüssler-Lenz

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Martina Schüssler-Lenz	Germany
Member	Jan Mueller-Berghaus	Germany
Member	Maura O'Donovan	Ireland
Member	Romaldas Mačiulaitis	Lithuania
Member	Carla Herberts	Netherlands
Member	Rune Kjeklen	Norway
Member	Sol Ruiz	Spain

1.1.3. New Active Substance (NAS) status of ATMPs

Key objectives

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

Activities in 2021

CAT activities to achieve the objectives set for this area:

- Contribution to the development of the Guidance on the structure and properties for the determination 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 guidance document.

EMA topic leader: Patrick Celis; CAT topic leader: Rocio Salvador Roldan

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Rocio Salvador Roldan	European Commission
Member	Ilona Reischl	Austria
Member	Heli Suila	Finland
Alternate	Niamh Curran	Ireland
Expert	Marja van der Bovenkamp	Netherlands
Expert	Barbara Bonamassa	Italy

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

Key objectives

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

Activities in 2021

CAT activities to achieve the objectives set for this area:

- Development of the ICH S12 guideline: dedicated support from CAT to the appointed EU Rapporteurs for this guideline
- Reflections on the definition of gene therapy medicinal products (GTMP) in the ICH S12 guideline and considerations for a revision of the EU GTMP definition.

EMA topic leader: Patrick Celis ; CAT topic leaders: Rune Kjekken, Claire Beuneu (EU Rapporteurs)

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Rune Kjekken	Norway
CAT topic leader	Claire Beuneu	Belgium
Member	Ilona Reischl	Austria
Member	Anne Pastoft	Denmark
Member	Toivo Maimets	Estonia
Member	Violaine Closson Carella	France
Alternate	Belaid Sekkali	Belgium
Alternate	Egbert Flory	Germany
Alternate	Isabel Vieira	Portugal
Alternate	Marcos Timon	Spain
Experts	Brigitte Anliker	Germany
Expert	Tineke van den Hoorn	Netherlands
	Rocio Salvador Roldan	European Commission

1.1.5. Real World Data (RWD) in regulatory decision making of ATMPs

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 2021

CAT activities to achieve the objectives set for this area:

- Organise a stakeholder meeting with ATMP developers in the second half of 2021.
- Agree key principles and identify use cases for real world data in the lifecycle of ATMPs for a pilot of real-world data analytics to be performed in 2022.

EMA topic leader: Gianmario Candore; CAT topic leader: Martina Schüssler-Lenz

Member/alternate	Name	MS
CAT topic leader	Martina Schüssler-Lenz	Germany
Member	Romaldas Maciulaitis	Lithuania
Member	Lisbeth Barkholt	Sweden
Member	Alessandro Aiuti	Physicians' 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. Other specialised areas and activities

1.2.1. Implementation of the Medical Device Regulation

Key objectives

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

Activities in 2021

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.

EMA topic leader: Patrick Celis; CAT topic leader: Ilona Reischl

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Ilona Reischl	Austria
Member	Ivana Haunerova	Czech Republic
Member	Heli Suila	Finland
Member	Violaine Closson Carella	France
Member	Maura O'Donovan	Ireland
Member	Jan Mueller-Berghaus	Germany
Member	Lisbeth Barkholt	Sweden

1.2.2. Requirements for manufacturers of viral vector starting materials for genetically modified cells

Key objectives

Clarification of the quality assurance system specific for manufacturers of viral vector used as starting materials for the production of genetically modified cells.

Activities in 2021

CAT activities to achieve the objectives set for this area:

- Develop a question and answer document on principles of GMP

CAT will collaborate with the BWP and the GMDP IWG¹ for the development of this document.

EMA topic leader: Roberto Conocchia; CAT topic leader: Marcos Timon

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Marcos Timon	Spain
Member	Ivana Haunerova	Czech Republic
Member	Heli Suila	Finland
Member	Violaine Closson Carella	France
	Rocio Salvador Roldan	European Commission

¹ Good Manufacturing Practise and Good Distribution Practise inspectors working group

2. Horizontal activities and other areas

2.1. Committees and Working Parties

2.1.1. Additional objectives and activities

Key objectives

- Further strengthen the interactions between CAT and COMP and CAT and PDCO.

Activities in 2021

CAT activities to achieve the objectives set for this area:

- Optimise the working practices of the COMP-CAT and PDCO-CAT working groups (both groups were set up in 2020)

CAT topic leader: Maura O'Donovan

Member/alternate	Name	MS
CAT topic lead	Maura O'Donovan	Ireland
Chair	Martina Schüssler-Lenz	Germany
Member	Anne Pastoft	Denmark
Member	Olli Tenhunen	Finland
Member	Carla Herberts	Netherlands
Member	Kerstin Sollerbrant Melefors	Patients' organisation representative
Alternate	Maja Sommerfelt	Norway