EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

24 January 2020
EMA/CAT/520483/2019
Inspections, Human Medicines Pharmacovigilance & Committees

CAT work plan 2020

Table of Content

The activities outlined in the CAT work plan for 2020 has been agreed taking into consideration that activities are gradually reinstated following a phase of business continuity.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

1. Evaluation activities for human medicines

1.1. Pre-authorisation activities

1.1.1. Revision of the guideline on genetically modified cells

Key objectives

Revision of the Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells.

Activities in 2020

CAT activities to achieve the objectives set for this area:

- Finalise the revision of the guideline after external consultation

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

CAT topic leader: Marcos Timón

Other committee participants:

Member/alternate	Name	MS
CAT topic leader	Marcos Timón	Spain
CAT chair	Martina Schüßler-Lenz	Germany
Member	Ilona Reischl	Austria
Member	Heli Suila	Finland
Member	Una Riekstina	Latvia
Member	Paolo Gasparini	Italy
Alternate	Belaid Sekkali	Belgium
Alternate	Olli Tenhunen	Finland
Expert	Tiina Palomäki	Finland
Expert	Barbara Bonamassa	Italy
Expert	Giuseppa Pistritto	Italy
Expert	Matthias Renner	Germany (PEI)
Expert	Brigitte Anliker	Germany (PEI)
Expert	Marcel Hoefnagel	Netherlands

1.1.2. Development of a 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 2020

CAT activities to achieve the objectives set for this area:

- Finalise the guideline after external consultation

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	Alina Musetescu	Romania
Member	Metoda Lipnik-Stangelj	Slovenia
Member	Kieran Breen	Patients' organisation representative
Expert	Barbara Bonamassa	Italy
Expert	Guiseppa Pistritto	Italy
Expert	Marcel Hoefnagel	Netherlands

1.2. Post-authorisation activities

1.2.1. Timely implementation of registries and post-authorisation studies for ATMPs

A large proportion of ATMPs are developed for rare to ultra-rare disease. This has an impact on the type of clinical trials (often without control arm) and the size of the safety and efficacy database at the time of approval. It is a legal requirement to have a follow-up of safety and efficacy of ATMPs after approval: post-authorisation safety studies (PASS) and post-authorisation efficacy studies (PAES) can be imposed for post-authorisation evidence generation of the ATMP. PASS and PAES studies can be observational studies, which can be based on disease or product registries. When registries are used as data source for such studies, case-by-case decisions on data elements, ATMP-specific data sets, data quality, consistency, accuracy and completeness will have to be made.

Key objectives

- Co-ordinate with existing EMA initiatives relating to registries; cross-committee collaboration (including PRAC and CHMP).
- Provide a framework for optimising regulatory requests for registries, so that requests are feasible, and study designs fit for purpose (i.e. capable of answering a specific safety/efficacy question).

Activities in 2020

CAT activities to achieve the objectives set for this area:

- Reflect, in collaboration with EMA, on how to enhance regulatory support on PAES, PASS and registries during product development (e.g. via PRIME / Scientific Advice).
- Ensure, in collaboration with PRAC, that PASS protocols are submitted and evaluated during the marketing authorisation procedure.
- Contribute to the development (by CHMP) of guidance on best use of and standards for registries for regulatory purposes (taking into account existing guidance, engagement with patient registries and interactions with HTAs).

CAT topic leader: Martina Schüssler-Lenz

Member/alternate	Name	MS
CAT topic leader	Martina Schüssler-Lenz	Germany
Member	Maura O'Donovan	Ireland
Member	Romaldas Maciuliatis	Lithuania
Member	Metoda Lipnik-Stangelj	Slovenia
Member	Kieran Breen	Patients' organisation representative
Alternate	Angeliki Roboti	Greece
Alternate	Alessandra Renieri	Clinicians' representative
European Commission Representative	Rocio Salvador-Roldan	European Commission

1.3. Other specialised areas and activities

1.3.1. Implementation of the Medical Device and In vitro Diagnostics Regulation

Key objectives

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

Activities in 2020

CAT activities to achieve the objectives set for this area:

- Establish a CAT medical device focus group. This (informal) group, composed of CAT members/alternates/experts with medical device competence, will follow the implementation of the new Regulations for MD/IVD and will become a point of call when MD/IVD issues will arise during the CAT discussion.
- Reflect on the need for 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.
- Reflect, in collaboration with EMA, on how to enhance regulatory support on MD/IVD- related aspects during product development (e.g. via PRIME / Scientific Advice).
- Provide training to CAT members on MD/IVD.

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
Alternate	Giulio Pompilio	Italy

2. Horizontal activities and other areas

2.1. Committees and Working Parties

2.1.1. Additional objectives and activities

Key objectives

- Establish a COMP-CAT working group to optimise the interaction and output of the two Committees in assessment of orphan ATMPs.
- Establish a PDCO-CAT working group to optimise the interaction and output of the two Committees in development and assessment of paediatric ATMPs.

Activities in 2020

CAT activities to achieve the objectives set for this area:

- Organise a CAT-COMP brainstorm meeting to discuss the establishment of a COMP-CAT working group. During the brainstorming exercise, the scope and topics for above working group and frequency of meetings will be discussed.
- Establish a COMP-CAT working group.
- Establish a PCDO-CAT working group.

CAT topic leader: Maura O'Donovan

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