19 February 2025
EMA/CAT/73821/2025
Human Medicines Division

# Committee for Advanced Therapies (CAT)

Minutes of the meeting on 22-24 January 2025

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

**Health and safety information**

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

**Disclaimers**

Some of the information contained in these minutes is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the CAT meeting reports once the procedures are finalised.

Of note, these minutes are a working document primarily designed for CAT members and the work the Committee undertakes.

**Note on access to documents**

Some documents mentioned in the minutes cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

**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
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# 1. Introduction

## 1.1. Welcome and declarations of interest of members, alternates and experts

The Chairperson opened the meeting by welcoming all participants. The meeting was held in person with some members connecting remotely.

In accordance with the Agency's policy on handling of declarations of interests of scientific committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and on the topics in the agenda of the current meeting, the Committee Secretariat announced the restricted involvement of some Committee members, alternates and experts for concerned agenda topics.

Participants were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions were declared. Restrictions applicable to this meeting are captured in the List of participants included in the minutes.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The Chair welcomed the new member from Czechia.

The EMA Secretariat announced the names of the Committee members who delegated their vote via proxy and the Committee members who received such proxy.

The Chair announced the start of the Polish presidency of the Council of the European Union (EU).

## 1.2. Adoption of agenda

The CAT agenda for 22-24 January 2025 meeting was adopted.

## 1.3. Adoption of the minutes

The CAT minutes for 04-06 December 2024 meeting were adopted. CAT members were informed that CHMP at their PROM meeting on 20 January 2025 adopted the *Guideline on quality, non-clinical and clinical requirements for investigational ATMPs in clinical trials*, which will be published on the EMA website shortly.

Post-meeting note: the Guideline was published on the EMA website on 06.02.2025.

# 2. Evaluation of ATMPs

## 2.1. Opinions

No items

## 2.2. Oral explanations

No items

## 2.3. Day 180 list of outstanding issues

No items

## 2.4. Day 120 list of questions

No items

## 2.5. Day 80 assessment reports

No items

## 2.6. Update on ongoing initial applications

No items

## 2.7. New applications

### 2.7.1. Nadofaragene firadenovec - EMEA/H/C/005856

Treatment of adult patients with high-grade (HG), Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladder cancer (NMIBC)

Scope: Timetable for assessment

**Action:** for adoption

The information was noted.

### 2.7.2. Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding human Wiskott-Aldrich syndrome protein - Orphan - EMEA/H/C/006525

Fondazione Telethon Ets; Treatment of patients with Wiskott-Aldrich Syndrome (WAS)

Scope: Timetable for assessment

**Action:** for adoption

The information was noted.

## 2.8. Withdrawal of initial marketing authorisation application

No items

## 2.9. Re-examination of initial application procedures under Article 9(2) of Regulation No. 726/2004

No items

## 2.10. GMP and GCP inspections requests

No items

## 2.11. Type II variations and variations of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008

### 2.11.1. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/II/0043/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli; PRAC Rapporteur: Gabriele Maurer

Scope: Safety & Quality, opinion

Extension of indication for Breyanzi to include treatment of adult patients with 3rd line + follicular lymphoma (FL) based on final results from the pivotal study JCAR017-FOL-001 (FOL-001, TRANSCEND-FL). This is a phase 2, open-label, single-arm, multicohort, multicentre study to evaluate efficacy and safety of JCAR017 in adult subjects with relapsed or refractory (r/r) FL or marginal zone lymphoma (MZL). As a consequence, sections 4.1, 4.4, 4.8, 5.1 and 5.2 of the SmPC are updated. The Package Leaflet is updated in accordance. Version 5.0 of the RMP is being submitted. Furthermore, as part of the application the MAH is requesting a 1-year extension of the market protection.

**Action:** for adoption

Request for supplementary information adopted on 08.11.2024.

The Rapporteur presented the outcome of the assessment of the responses to the request for supplementary information. All questions were resolved. The Rapporteur concluded that the benefit risk for Breyanzi in the adult patients with third line + FL is positive. The opinion was adopted.

### 2.11.2. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0034

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Jo Robays

Scope: Safety, request of supplementary information

Submission of an updated RMP version 5.2 in order to add a new important identified risk of "Secondary malignancy of T-cell origin", to change the important potential risk of "Second primary malignancies" to "Second primary malignancy except secondary malignancy of T-cell origin", and to include an additional pharmacovigilance activity for testing of secondary malignancies of T-cell origin, following the PRAC recommendation for the secondary malignancy of T-cell origin signal (EPITT no: 20040).

**Action:** for adoption

The Rapporteur provided feedback from the PRAC discussion and the questions raised by PRAC. The request for supplementary information was adopted.

### 2.11.3. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0035

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

**Action:** for adoption

The opinion was adopted.

### 2.11.4. Imlygic - Talimogene laherparepvec - EMEA/H/C/002771/II/0068

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Quality, opinion

**Action:** for adoption

The opinion was adopted.

### 2.11.5. Kymriah - Tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0092

Novartis Europharm Limited

Rapporteur: Rune Kjeken, PRAC Rapporteur: Gabriele Maurer

Scope: Safety, request for supplementary information

Update of section 4.2 of the SmPC in order to update the 'monitoring after infusion' recommendations, based on existing clinical trial data as well as literature references reporting real word experience. The Package Leaflet is updated accordingly. The RMP version 8.0 has also been submitted. In addition, the MAH took the opportunity to introduce a minor change to the Healthcare Professional (HCP) educational programme in the Annex II in order to enhance readability.

**Action:** for adoption

The MAH wants to relax the safety monitoring after infusion. The request for supplementary information was adopted.

### 2.11.6. Strimvelis - Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence - Orphan - EMEA/H/C/003854/II/0040

Fondazione Telethon ETS

Rapporteur: Sol Ruiz, PRAC Rapporteur: Liana Martirosyan

Scope: Safety, opinion

Submission of an updated RMP version 7.0 in order to propose amendments to the STRIM-005 and STRIM-003 study protocols, as well as revised timelines for completion of both studies. In addition, the Annex II is updated accordingly.

**Action:** for adoption

Request for supplementary information adopted on 13.09.2024.

A short feedback was provided from the PRAC discussion. The amendments to the study protocol for both studies are acceptable. The opinion was adopted.

### 2.11.7. Zolgensma - Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/II/0055

Novartis Europharm Limited

Rapporteur: Emmely de Vries

Scope: Quality, opinion

**Action:** for adoption

The opinion was adopted.

### 2.11.8. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2771

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Karin Erneholm

Scope: Safety, request for supplementary information

Submission of an updated RMP version 4.3 for Tecartus and version 11.1 for Yescarta following the PRAC recommendation for the secondary malignancy of T-cell origin signal (EPITT no: 20040), and of a PASS protocol for a framework for the sampling and testing of secondary malignancies of T-cell origin

**Action:** for adoption

The Rapporteur provided feedback from the PRAC discussion and the questions raised by PRAC. The request for supplementary information was adopted.

## 2.12. Extension applications

No items

## 2.13. Other Post-Authorisation Activities

### 2.13.1. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/REC/022.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, opinion

**Action:** for adoption

The outcome of the assessment was adopted.

### 2.13.2. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/MEA/007.3

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Pharmacovigilance, opinion

Protocol amendment / PASS study PCSONCA0014 (v. 1)
Post-authorization Safety Study Survey to Evaluate the Effectiveness of the Ciltacabtagene Autoleucel Healthcare Professional (HCP) Educational Program and the Product Handling Training.

**Action:** for adoption

The outcome of the assessment was adopted.

### 2.13.3. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/REC/018

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

**Action:** for adoption

The outcome of the assessment was adopted.

### 2.13.4. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/PSA/S/0116

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: PASS protocol (PRAC-led procedure)

An updated protocol (Amendment 2) for study 68284528MMY4004 "An Observational Post-authorisation Safety Study to Evaluate the Safety of Multiple Myeloma Patients Treated with Ciltacabtagene Autoleucel

**Action:** for information

The outcome of the assessment was adopted.

### 2.13.5. BEQVEZ - Fidanacogene elaparvovec - Orphan - EMEA/H/C/004774/ANX/003

Pfizer Europe MA EEIG

Rapporteur: Jan Mueller-Berghaus

Scope: PAES protocol

Protocol for PAES C0371007: in order to further characterise the long-term efficacy and safety of Beqvez in adults with severe and moderately severe haemophilia B (congenital factor IX deficiency) without a history of factor IX inhibitors and without detectable antibodies to variant AAV serotype Rh74, the MAH should conduct and submit the final results of registry-based study C0371007, according to an agreed protocol.

**Action:** for adoption

The Rapporteur presented the outcome of the assessment of the protocol for the PAES. Some additional information needs to be provided by the MAH. Yearly progress reports of the study are expected. The outcome of the assessment was adopted.

### 2.13.6. Ebvallo - Tabelecleucel - Orphan - EMEA/H/C/004577/REC/008

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, opinion

**Action:** for adoption

The outcome of the assessment was adopted.

### 2.13.7. Type IB

Scope: To summarise the regulatory and procedural aspects of type IB procedures, as handled by EMA

**Action:** for information

EMA presented the regulatory and procedural aspects of type IB procedures: for this type of variations, only a single request for information can be issued

## 2.14. Companion diagnostics - initial consultation

No items

## 2.15. Companion diagnostics – Follow-up consultation

No items

# 3. Certification of ATMPs

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

## 3.1. Opinion

## 3.2. Day 60 Evaluation Reports

No items

## 3.3. New Applications

# 4. Scientific Recommendation on Classification of ATMPs

## 4.1. New requests – Appointment of CAT Coordinator

### 4.1.1. Lab grown red blood cells

Treatment of anemia

Scope: Withdrawal of scientific recommendation on classification of ATMPs

**Action:** for adoption

The CAT noted the withdrawal of scientific recommendation on classification of ATMPs

## 4.2. Day 30 ATMP scientific recommendation

### 4.2.1. mRNAs encoding IL-12 and IL-18

Treatment of gastric cancer

Scope: ATMP scientific recommendation

**Action:** for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 07.02.2025.

### 4.2.2. BCMA targeting Chimeric Antigen Receptor expressing mRNA transfected autologous T cells

Treatment of myasthenia Gravis

Scope: ATMP scientific recommendation

**Action:** for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 07.02.2025.

### 4.2.3. Autologous Tumor Infiltrating Lymphocytes (TILs)

Treatment of adult patients with advanced or 2/4 metastatic solid tumors who have not responded to standard therapies (chemotherapy, radiation therapy, molecule-targeted therapy) or are ineligible for alternative treatment options or in associated treatment with chemotherapy in multimodel therapy

Scope: ATMP scientific recommendation

**Action:** for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 07.02.2025.

### 4.2.4. Adeno-associated virus serotype 5 containing the human RORA gene (AAV5-hRORA)

Treatment of adult and paediatric patients with vision loss due to Geographic Atrophy secondary to dry age-related macular degeneration and Stargardt Disease

Scope: ATMP scientific recommendation

**Action:** for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 07.02.2025.

## 4.3. Day 60 revised scientific recommendation (following list of questions)

No items

## 4.4. Finalisation of procedure

### 4.4.1. Autologous primary urothelial cells expanded

For use of cystoplasty/orthotopic neobladder

Scope: European Commission raised no comments. ATMP scientific recommendation

**Action:** for adoption

The classification report was adopted. The product does fulfil the definitions of a tissue engineered product as provided in Article 2(1) of Regulation (EC) No. 1394/2007.

### 4.4.2. Adeno-associated virus serotype 5 containing the human NR2E3 gene (AAV5-hNR2E3)

Treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy, specifically retinitis pigmentosa or Leber congenital amaurosis, and who have sufficient viable retinal cells

Scope: European Commission raised no comments. ATMP scientific recommendation

**Action:** for adoption

The classification report was adopted. The product does fulfil the definition of a gene therapy medicinal product as provided in Article 2(1) of Regulation (EC) No. 1394/2007.

### 4.4.3. CD70 CAR+, TCRαβ− viable cells

Treatment of renal cell carcinoma

Scope: European Commission raised no comments. ATMP scientific recommendation

**Action:** for adoption

The classification report was adopted. The product does fulfil the definitions of a gene therapy medicinal product and a somatic cell therapy medicinal product and is therefore classified as a gene therapy medicinal product as provided in Article 2(5) of Regulation (EC) No. 1394/2007.

## 4.5. Follow-up and guidance

No items

# 5. Scientific Advice

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

## 5.1. New requests - appointment of CAT Rapporteurs

### 5.1.1. Ongoing scientific advice procedures - Appointment of CAT Peer Reviewers

Timetable:

| | |
|---|---|
| - Start of procedure at SAWP: | 13-16.01.2025 |
| - Appointment of CAT Peer Reviewers: | 22-24.01.2025 |
| - SAWP first reports: | 03.02.2025 |
| - CAT Peer Reviewer comments (NC/C): | 07.02.2025 |
| - CAT Peer Reviewer comments (Q): | 12.02.2025 |
| - Discussion at SAWP: | 10-13.02.2025 |
| - Discussion at CAT and feedback to SAWP: | 19-21.02.2025 |

### 5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

| | |
|---|---|
| - Start of procedure at SAWP: | 10-13.02.2025 |
| - Appointment of CAT Peer Reviewers: | 19-21.02.2025 |
| - SAWP first reports: | 03.03.2025 |
| - CAT Peer Reviewer comments (NC/C): | 07.03.2025 |
| - CAT Peer Reviewer comments (Q): | 12.03.2025 |
| - Discussion at SAWP: | 10-13.03.2025 |
| - Discussion at CAT and feedback to SAWP: | 19-21.2025 |

## 5.2. Procedures discussed at SAWP – 1st reports, D40 JRs, LoIs

## 5.3. Finalisation of D70 procedures – feedback from the discussion meeting

## 5.4. Final Advice Letters for procedures finalised the previous month

# 6. Pre-Authorisation Activities

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

## 6.1. Paediatric investigation plans

No items

## 6.2. ITF briefing meetings in the field of ATMPs

## 6.3. Priority Medicines (PRIME) – Eligibility requests

### 6.3.1. Month 0 - Start of the procedure

| | |
|---|---|
| Timetable for assessment: | |
| Procedure start: | 13-16.01.2025 |
| SAWP recommendation: | 13.02.2025 |
| CAT recommendation: | 21.02.2025 |
| CHMP adoption of report and final recommendation: | 27.02.2025 |

### 6.3.2. Month 1 – Discussion of eligibility

### 6.3.3. Month 2 – Recommendation of eligibility

### 6.3.4. Ongoing support

No items

# 7. Organisational, regulatory and methodological matters

## 7.1. Mandate and organisation of the CAT

### 7.1.1. CAT membership

The Chair welcomed Eva Kolouchová as the new member for Czechia.

### 7.1.2. Vote by proxy

Concetta Quintarelli gave a proxy to Jan Mueller-Berghaus to vote on behalf of Italy covering the entire meeting.

Alessia Pochesci gave a proxy to Claire Beuneu to vote on behalf of Luxembourg covering the entire meeting.

### 7.1.3. CAT Strategic Review & Learning meeting (SRLM) under the Polish presidency

CAT: Dariusz Sladowski

Scope: Preparation for the meeting

**Action**: for discussion

CAT noted the information provided by the Polish CAT member. There was a short discussion on the agenda topic for the upcoming SRLM meeting, which will be in part held jointly with the COMP. CAT members were asked to send proposals for additional topics to the CAT secretariat and Dariusz Sladowski by 14.02.2025 at the latest.

### 7.1.4. CAT Strategic Review & Learning meeting (SRLM) under the Danish presidency

CAT: Martin Bronislaw Oleksiewicz

Scope: Dates for the meeting

**Action**: for information

The dates for the SRLM under the Danish presidency was noted.

## 7.2. Coordination with EMA Scientific Committees

### 7.2.1. Revision of the procedural advice on CHMP/CAT/PRAC Rapporteur/Co-Rapporteur appointment principles

Revision of the procedural advice on CHMP/CAT/PRAC Rapporteur/Co-Rapporteur appointment principles, objective criteria and methodology in accordance with Article 62(1) of Regulation (EC) No 726/2004.

**Action:** For information

The revised procedural advice was presented. CAT members to provide comments by 14.02.2025. The procedural advice will be adopted at the February CAT meeting.

## 7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

### 7.3.1. Revision of ATMP GMP guideline

Scope: Presentation of the main changes for the revision of the GMP ATMP guideline and next steps

**Action**: for information

The points to be considered during the revision of the GMP for ATMP guideline were presented: this will be a targeted revision, to align to the revised Annex I on sterile manufacturing. A concept paper for the revision of the guideline will be circulated to CAT after discussion in the Inspectors Working Party. CAT asked to be consulted on the draft revised GMP for ATMP guideline.

### 7.3.2. Question and Answer on decentralised manufacturing

GMP Inspectors working group

Scope: Update on comments received

**Action**: for discussion

The key elements for decentralised manufacturing were presented. The question and answer (Q&A) document aims to help Marketing Authorisation Holders (MAHs) / Manufacturers to operate decentralised manufacturing in line with the current regulatory framework and provides clarifications in relation to GMP and dossier requirements. Comments on the draft Q&A were received from member states, and these will be evaluated by the Quality Innovation Group (QIG) and EMA, and the final version will be submitted to the Immunologicals Working Party (IWP), CAT and Biologics Working Party (BWP) for adoption.

### 7.3.3. Update on Quality Innovation Group (QIG) activities

CAT: Marcel Hoefnagel, Marcos Timon

Scope: Update the CAT on recent relevant activities of the QIG and inform CAT members on how to access QIG documents

**Action:** for information

An overview of the QIG role and its activities was presented, including its Listen and Learn Focus Group (LLFG) meetings.

## 7.4. Cooperation with the EU regulatory network

### 7.4.1. EU Network Training Centre: supporting capacity and capability building in the EU Medicines Regulatory Network

Scope: Advancing the ATMP training curriculum going forward

**Action:** for information

CAT noted the information on EU Network Training Centre (EU NTC) and the collaboration with the IncreaseNet project. There was a short discussion on planned trainings on the CAT guideline for investigational ATMPs.

### 7.4.2. Feedback from HTA/EMA workshop on uncertainty management

Scope: Oral report from workshop on uncertainty management

**Action:** for information

CAT noted the feedback from the workshop on uncertainty management.

### 7.4.3. Exchange of views with European Commission on Legislation Reform

**Action:** for discussion

The CAT members received an update on the reform of the pharmaceutical legislation, in particular on the proposed reform of the EMA committees, and exchanged views with the EC Representatives.

## 7.5. Cooperation with international regulators

### 7.5.1. ATMP cluster teleconference with US-FDA, Health Canada and PMDA (Japan)

CAT: Ilona Reischl

Scope: Feedback from the teleconference of 12.12.2024

**Action:** for information

A short feedback was given from the discussions at the December 2024 ATMP cluster teleconference.

### 7.5.2. US-FDA-EMA collaboration on gene therapies for (ultra) rare diseases (CoGenT)

Scope: Feedback on the CoGenT pilot project

**Action:** for information

EMA presented the pilot project with FDA to enhance collaboration on gene therapies for (ultra) rare diseases. The aim of the pilot is to exchange information (e.g. list of questions, information requests from FDA to applicant) and the participation, as observers, in each other meetings (e.g. CAT plenary, FDA mid-cycle meeting). CAT was also informed that one product was already selected for the CoGenT pilot.

## 7.6. CAT work plan

### 7.6.1. CAT work plan 2025

CAT: Ilona Reischl

Scope: CAT work plan 2025

**Action**: for adoption

Additional topic leads were appointed. The CAT work plan for 2025 was adopted.

Post meeting note: the CAT work plan for 2025 was published on the EMA website.

## 7.7. Planning and reporting

### 7.7.1. Business Pipeline Report – Q42024 Forecast report

Scope: Q4/2024 Update of the marketing authorisation applications (MAAs) expected in 2025, report for the human scientific committees

**Action:** for information

CAT noted the presentation of the forecast of ATMP MAAs expected in 2025.

## 7.8. Others

### 7.8.1. IRIS functionalities for CAT members post procedure management transition to IRIS

Scope: The IRIS team will provide a demo of IRIS Network portal functionalities useful to CAT members following the transition of post-authorisation procedures to IRIS which took place in January 2025

**Action:** for information

EMA presented the IRIS Network Portal functionalities for post-authorisation procedures. The expected changes to the CAT agenda and minutes were also presented.

# 8. Any other business

No items

Date of next CAT meeting:

19-21 February 2025

## 9. List of participants

List of participants including any restrictions with respect to involvement of members/alternates/experts following evaluation of declared interests for the 22-24 January 2025 CAT meeting, which was held in-person.

An asterisk (*) after the role, in the second column, signals that the member/alternate attended remotely. Additional experts participated in (part of) the meeting, either in person or remotely.

| Name | Role | Member State or affiliation | Outcome restriction following evaluation of e-DoI | Topics on agenda for which restrictions apply |
|---|---|---|---|---|
| Ilona Reischl | Chair | Austria | No interests declared | |
| Silke Dorner | Member | Austria | No interests declared | |
| Claire Beuneu | Member | Belgium | No interests declared | |
| Olga Kholmanskikh | Alternate | Belgium | No interests declared | |
| Rozalina Kulaksazova | Member | Bulgaria | No interests declared | |
| Azra Selimovic | Member | Croatia | No interests declared | |
| Rafaella Pontou | Member | Cyprus | No interests declared | |
| Eva Kolouchová | Member* | Czechia | No interests declared | |
| Radka Nejezchlebová | Alternate | Czechia | No interests declared | |
| Martin Oleksiewicz | Member | Denmark | No interests declared | |
| Johanne Juhl Korsbaek | Alternate | Denmark | No restrictions applicable to this meeting | |
| Toivo Maimets | Member | Estonia | No interests declared | |
| Pille Saalik | Alternate | Estonia | No interests declared | |
| Heli Suila | Member | Finland | No interests declared | |
| Violaine Closson Carella | Member | France | No interests declared | |
| Jean-Michel Race | Alternate* | France | No interests declared | |
| Jan Mueller-Berghaus | Member (CHMP co- | Germany | No interests declared | |

| | | | | |
|---|---|---|---|---|
| | opted member) | | | |
| Egbert Flory | Alternate (to CHMP representative)* | Germany | No interests declared | |
| Maria Gazouli | Member | Greece | No interests declared | |
| Angeliki Rompoti | Alternate* | Greece | No restrictions applicable to this meeting | |
| Andras Donaszi-Ivanov | Member | Hungary | No restrictions applicable to this meeting | |
| Viola Bardoczy | Alternate* | Hungary | No restrictions applicable to this meeting | |
| Joseph De Courcey | Member* | Ireland | No interests declared | |
| Richard Carroll | Alternate | Ireland | No interests declared | |
| Concetta Quintarelli | Member* | Italy | No restrictions applicable to this meeting | |
| Barbara Bonamassa | Alternate* | Italy | No interests declared | |
| Liga Kunrade | Alternate | Latvia | No interests declared | |
| Vilma Perikaite | Member (CHMP member) | Lithuania | No interests declared | |
| Nancy De Bremaeker | Alternate* | Luxembourg | No interests declared | |
| John Joseph Borg | Member (CHMP member) | Malta | No interests declared | |
| Emmely de Vries | Member | Netherlands | No interests declared | |
| Berendina Maria (Tineke) van den Hoorn | Alternate | Netherlands | No interests declared | |
| Rune Kjeken | Member | Norway | No participation in final deliberations and voting on: | |
| Ole Henrik Myrdal | Alternate | Norway | No interests declared | |
| Dariusz Sladowski | Member | Poland | No restrictions applicable to this meeting | |
| Maria Isabel Borba Vieira | Member (CHMP member) | Portugal | No interests declared | |
| Denisa Marilena Margina | Member | Romania | No interests declared | |
| Liviu Nitulescu | Alternate* | Romania | No restrictions applicable to this meeting | |
| Katarina Kollarova | Member | Slovakia | No interests declared | |

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|---|---|---|---|---|
| Margareta Fogelová | Alternate* | Slovakia | No interests declared | |
| Suzana Vidic | Member | Slovenia | No restrictions applicable to this meeting | |
| Sol Ruiz | Member (CHMP co-opted member)* | Spain | No interests declared | |
| Marcos Timón | Alternate (to CHMP representative)* | Spain | No interests declared | |
| Maria Luttgen | Member | Sweden | No restrictions applicable to this meeting | |
| Charlotte Anderberg | Alternate* | Sweden | No interests declared | |
| Bernd Gansbacher | Alternate | Clinicians' Representative | No interests declared | |
| Alessandra Renieri | Alternate | Clinicians' Representative | No restrictions applicable to this meeting | |
| Kerstin Sollerbrant Melefors | Member | Patients' Representative | No interests declared | |
| Mencia de Lemus Belmonte | Alternate | Patients' Representative | No interests declared | |
| Kieran Breen | Member (Vice-Chair) | Patients' Representative | No interests declared | |
| Federica Chiara | Alternate* | Patients' Representative | No interests declared | |
| Catherine Milne | Observer/Alternate* | EDQM | No interests declared | |
| Torbjörn Callréus | Expert | Malta | No interests declared | |
| Marjolijn Schalk | Expert | Netherlands | No interests declared | |
| Jean Luc Golnez | Expert | Belgium | No interests declared | |
| Esther Rincon | Expert | Spain | No interests declared | |
| Johanna Lahteenvuo | Expert | Finland | No interests declared | |
| Pauliina Lehtolainen-Dalkilic | Expert | Finland | No interests declared | |
| Marcel Hoefnagel | Expert | Netherlands | No interests declared | |
| Odoardo Maria Olimpieri | Expert | Italy | No interests declared | |
| Antonella Isgrò | Expert | Italy | No interests declared | |
| Federico De Angelis | Expert | Italy | No interests declared | |
| Representatives from the European Commission attended the meeting | | | | |

Meeting run with support from relevant EMA staff.

Experts' declared interests were evaluated against the agenda topics or activities they participated in.

# 10. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

For a list of acronyms and abbreviations, see:

List of abbreviations used in EMA human medicines scientific committees and CMDh documents, and in relation to EMA's regulatory activities

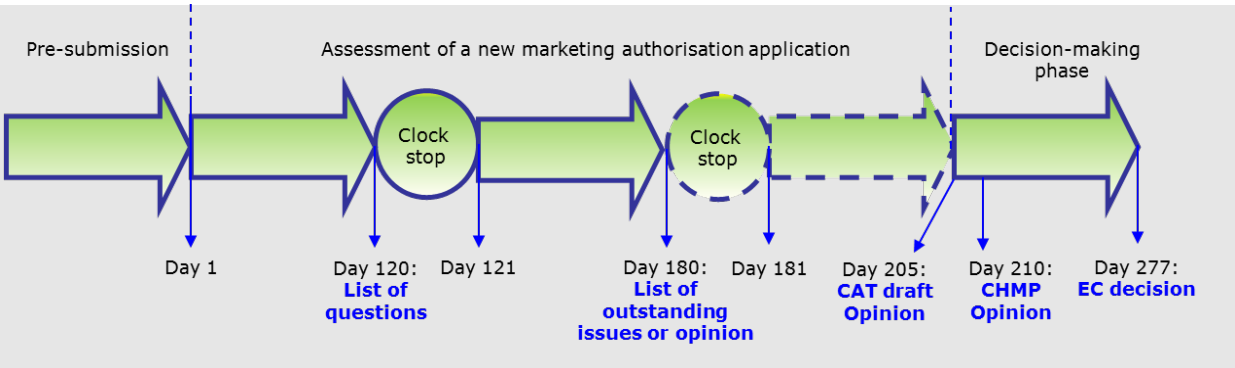
### Evaluation of ATMPs (section 2)

This section lists applications for marketing authorisations of new Advanced Therapy Medicinal Products (ATMPs) that are to be discussed by the Committee. It also lists Post-authorisation activities (section 2.11-2.13) and any ATMP related inspection requests (section 2.14).

*New applications (sections 2.1. to 2.9.)*

Section 2.1 is for ATMPs nearing the end of the evaluation and for which the CAT is expected to adopt a draft **opinion** at this meeting on whether marketing authorisation should be granted. Once adopted, the CAT opinion is transmitted to the CHMP for final adoption. The CHMP opinion will be forwarded to the European Commission for a final legally binding decision valid throughout the EU. More information on the evaluation of ATMPs can be found here.

The other items in the section are listed depending on the stage of the evaluation, which is shown graphically below:




The assessment of an application for a new medicine takes up to 210 'active' days. This active evaluation time is interrupted by at least one 'clock-stop' during which time the applicant prepares the answers to questions from the CAT. The clock stop happens after day 120 and may also happen after day 180, when the CAT has adopted respectively a **Day 120 list of questions** (section 2.4) or a List of outstanding issues to be addressed by the company, which is listed in the agenda under sections 2.7 (**Ongoing evaluation procedures** (section 2.3). Section 2.6 also includes the CAT discussions at any other timepoint of the evaluation procedure of new applications.

*Oral explanation (section 2.2.)*

Prior to adoption of the CAT opinion, marketing authorisation applicants are normally invited to the CAT plenary meeting to address questions raised by the Committee.

Oral explanations normally relate to ongoing applications, but they can also relate to any other issue for which the CAT would like to discuss with company representatives in person.

*New applications (section 2.7.)*

In this section, information is included on upcoming marketing authorisation applications for ATMPs, as well as information on appointment of Rapporteurs for new ATMP applications.

*Withdrawal of applications (section 2.8.)*

This section includes information on marketing authorisation applications that are withdrawn by the applicant. Applicants may decide to withdraw applications at any stage during the assessment and a CAT opinion will therefore not be issued. Withdrawals are included in the agenda for information or discussion, as necessary.

*Re-examination procedures (new applications) under article 9(2) of regulation no 726/2004 (section 2.9.)*

This section lists applications for new marketing authorisation for ATMPs for which the applicant has requested a re-examination of the opinion previously issued by the CHMP. Similar to the initial evaluation of a marketing authorisation of an ATMP, CAT will adopt a draft re-examination opinion, which is transmitted to the CHMP for final adoption.

*GMP and GCP Inspections Issues (section 2.14.)*

This section lists inspections that are undertaken for ATMPs. Inspections are carried out by regulatory agencies to ensure that marketing authorisation holders comply with their obligations. Inspection can relate to good manufacturing practice (GMP), good clinical practice (GCP), good laboratory practice (GLP) or good pharmacovigilance practice (GVP).

*Post-authorisation activities (section 2.11-2.13.)*

Section 2.11 lists type II variations, including extension of indication applications and re-examination procedures for type II variations for which the applicant has requested re-examination of the opinion previously issued by the CHMP. Section 2.12 list extension application according to Annex I of Reg. 1234/2008 and section 2.13 includes all other post-authorisation activities concerning authorised ATMPs that are not covered elsewhere in the agenda such as post-authorisation measures, annual reassessments, 5-year renewals, supply shortages, qualify defects. Issues that have been discussed at the previous meeting of the PRAC, the EMA's committee responsible for evaluating and monitoring safety issues for medicines, will also be included here.

*Companion diagnostics (section 2.14-2.15)*

This section lists applications for initial and follow-on consultation of companion diagnostics.

## Certification of ATMPs (section 3)

This section includes the scientific evaluation by the CAT of quality and non-clinical data that small and medium-sized enterprises have generated at any stage of the ATMP development process. More information on the ATMP certification procedure can be found here.

## Scientific Recommendation on Classification of ATMPs (Section 4)

This section includes the scientific recommendation by the CAT on whether medicines based on genes, cells or tissues meet the scientific criteria that define ATMPs. More information on the ATMP classification procedure, including the outcomes of finalised classifications, can be found here.

## Scientific Advice (section 5)

This section includes all scientific advice given to companies during the development of an ATMP. Information related to the number of ATMP related scientific advices discussed by CAT can be found in the CAT Monthly reports. Further information on SAWP can be found here.

## Pre-Authorisation (section 6)

### *Paediatric Investigation Plan (PIP)*

This section includes the discussion of an ATMP before a formal application for marketing authorisation is submitted. These cases refer for example to requests for an accelerated assessment for medicines that are of major interest for public health or can be considered a therapeutic innovation: in case of an accelerated assessment the assessment timetable is reduced from 210 to 150 days.

CAT contributes to the evaluation of a Paediatric Investigation Plan (PIPs) for ATMPs by the Paediatric Committee. These PIPs are included in this section of the Agenda.

### *ITF Briefing meeting in the field of ATMPs*

This section refers to briefing meetings of the Innovation Task Force and International co-operations activities of the CAT

The Innovation Task Force (ITF) is a body set up to encourage early dialogue with applicants developing innovative medicines. Minutes of meetings with applicants developing ATMPs and of other ITF meetings of interest to the CAT are included in this section of the agenda. Further information on the ITF can be found here.

### *Priority Medicines (PRIME)*

This section includes the new requests for eligibility to PRIME for ATMPs under development, the discussions in CAT of these eligibility requests and the final recommendations for eligibility of ATMPs adopted by CHMP.

CAT will appoint one of its members as the CAT sponsor for each new ATMP eligibility request who will lead the CAT discussion based on the recommendation from the SAWP.

## Organisational, regulatory and methodological matters (section 7)

This section includes topics related to regulatory and procedural guidance, CAT workplan, CAT meeting organisation (including CAT membership), planning and reporting, co-ordination with other committees, working parties and scientific advisory groups.

Furthermore, this section refers to the activities of the CAT drafting groups developing scientific guidelines for gene therapy medicinal products and for cell-based medicinal products, cooperation within the EU regulatory network and international regulators as well as direct interaction with interested parties. It also includes topics of scientific interest for the Committee that are not directly related to the work of the CAT drafting groups or CAT associated working parties.

## Any other business (section 8)

This section is populated with miscellaneous topics not suitable under the previous headings.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/