16 July 2025
EMA/CAT/280856/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 11-13 June 2025

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

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
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

Table of contents

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 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 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 thanked the members and alternates representing the clinicians and the patients' organisations for their active contribution to the CAT over the last three years.

1.2. Adoption of agenda

The CAT agenda for 11-13 June 2025 meeting was adopted with one addition: 2.13.15. Imlygic - EMA/PAM/0000262826.

1.3. Adoption of the minutes

The CAT minutes for 14-16 May 2025 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

2.1.1. Zemcelpro - Dorocubicel / Allogeneic umbilical cord-derived CD34- cells, non-expanded - PRIME - Orphan - EMEA/H/C/005772

Cordex Biologics International Limited; Treatment of adult patients with haematological

malignancies

Scope: Opinion

Action: for adoption

List of outstanding issues adopted on 21.03.2025. List of questions adopted on 11.10.2024.

The Rapporteur presented the outcome of the assessment of the responses to the list of outstanding issues. Feedback was provided from the BWP discussion. The final wording of the indication and the specific obligations were discussed and agreed.

CAT adopted a positive opinion recommending the granting of a conditional marketing authorisation.

2.2. Oral explanations

2.2.1. Delandistrogene moxeparvovec - Orphan - EMEA/H/C/005293

Roche Registration GmbH; Treatment of ambulatory patients aged 3 to 7 years old with Duchenne muscular dystrophy

Scope: Oral explanation

Action: Oral explanation

List of questions adopted on 11.10.2024. List of outstanding issues adopted 16.05.2025.

For this procedure, two patients joined the CAT discussion. The patients did not take part in the final deliberation and voting.

The Rapporteur presented the outcome of the assessment of the responses to the list of outstanding issues. Feedback was provided from the BWP discussion.

A trend vote was taken. The final vote and opinion will be taken at the July CAT meeting.

2.2.2. Autologous cartilage-derived articular chondrocytes, in-vitro expanded - EMEA/H/C/004594

Repair of symptomatic, localised, full-thickness cartilage defects of the knee joint grade III or IV

Scope: Oral explanation

Action: Oral explanation

List of questions adopted on 19.04.2024. List of outstanding issues adopted 21.03.2025.

The Rapporteur presented the outcome of the assessment of the responses to the list of outstanding issues. Feedback was provided from the BWP discussion.

A trend vote was taken. The final vote and opinion will be taken at the July CAT meeting.

2.3. Day 180 list of outstanding issues

2.3.1. Mozafancogene autotemcel - PRIME - Orphan - EMEA/H/C/005537

Rocket Pharmaceuticals B.V.; Treatment of paediatric patients with Fanconi anaemia type A

Scope: Day 180 list of outstanding issues

Action: for adoption

List of questions adopted on 19.07.2024.

The Rapporteurs presented the outcome of the assessment to the responses to the list of questions. Feedback was provided from the BWP discussion.

CAT adopted the list of outstanding questions.

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

2.6.1. 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: Request by the applicant for an extension to the clock stop to respond to the list of questions adopted in April 2025.

Action: for adoption

List of questions adopted on 25.04.2025.

CAT discussed the clock stop extension request.

2.7. New applications

2.7.1. In vitro diagnostic medical device - EMEA/H/D/006768

Qualitative determination of antibodies to adeno-associated virus serotype 74 (AAVrh74) in human serum and/or plasma

Scope: Timetable for assessment

Action: for adoption

The review timetable was agreed.

2.7.2. Onasemnogene abeparvovec– - Orphan - EMEA/H/C/6498

Novartis Europharm Limited; Treatment of patients with 5q spinal muscular atrophy (SMA)Scope: Timetable for assessment

Action: for adoption

The review timetable was agreed.

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. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/II/0051

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus; PRAC Rapporteur: Bianca Mulder

Scope: Clinical, PRAC led, request for supplementary information

Submission of the final study report for the non-interventional study KT-EU-472-5966 titled "Quantitative Testing of Health Care Professional Knowledge About Tecartus Risk Minimisation Measures'' listed as a category 3 study in the RMP.

Action: for adoption

Request for supplementary information adopted on 06.12.2024.

The second request for supplementary information was adopted.

2.11.2. Casgevy - Exagamglogene autotemcel - Orphan - EMA/VR/0000264854

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus, Co-rapporteur: Heli Suila; PRAC Rapporteur: Bianca Mulder

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.3. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/VR/0000264849

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.4. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/VR/0000264446

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical, request for supplementary information

Update of section 4.4 of the SmPC in order to amend an existing warning on secondary malignancies including of T-cell origin to limit the testing only to secondary malignancy of T-cell origin based on the data from clinical studies and literature.

Action: for adoption

Further clarification is sought from the MAH. The request for supplementary information was adopted.

2.11.5. Breyanzi - Lisocabtagene maraleucel - Orphan - EMA/VR/0000264124

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Clinical, request for supplementary information

A grouped application consisting of:

C.I.4: Update of section 5.1 of the SmPC in order to update efficacy information based on final results from study JCAR017-BCM-003; this is a global randomised multicenter phase 3 trial to compare the efficacy and safety of JCAR017 to standard of care in adult subjects with high-risk, transplant-eligible relapsed or refractory aggressive B-cell Non-Hodgkin lymphomas (TRANSFORM). In addition, final study reports for studies 017006 and JCAR017-BCM-001 Cohort 2 are submitted to support the main scope.

C.I.4: Update of section 4.8 of the SmPC in order to update information for the safety and immunogenicity based on pooled final data form the three follow up studies: (TRANSFORM BCM-003, PILOT 17006 and TRANSCEND WORLD, cohort 2 BCM-001). In addition, the MAH took the opportunity to remove the dose verification worksheet statement from the

Labelling.

Action: for adoption

The sections 4.4 and 4.8 of the SmPC are updated based on the final results of studies BCM-003, BCM-001 (cohort 2) and PILOT17006. Additional information has been requested prior to finalisation of this variation. The request for supplementary information was adopted.

2.11.6. Breyanzi - Lisocabtagene maraleucel - Orphan - EMA/VR/0000258227

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.7. Imlygic - Talimogene laherparepvec - Orphan - EMA/VR/0000258245

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.8. Abecma - Idecabtagene vicleucel - Orphan - EMA/VR/0000249089

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.9. Abecma - Idecabtagene vicleucel - Orphan - EMA/VR/0000248772

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Ebvallo - Tabelecleucel - Orphan - EMA/S/0000249324

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Second annual re-assessment in the frame of a marketing authorisation under exceptional circumstances, opinion

Action: for adoption

The opinion was adopted.

2.13.2. Casgevy - Exagamglogene autotemcel - Orphan - EMA/PAM/0000266956

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The outcome of the assessment was agreed. The PAM is considered resolved.

2.13.3. Casgevy - Exagamglogene autotemcel - Orphan - EMA/PAM/0000266953

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The outcome of the assessment was agreed. The PAM is considered resolved.

2.13.4. Casgevy - Exagamglogene autotemcel - Orphan - EMA/PAM/0000266952

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The outcome of the assessment was agreed. The PAM is considered resolved.

2.13.5. Casgevy - Exagamglogene autotemcel - Orphan - EMA/PAM/0000266949

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The request for supplementary information was agreed.

2.13.6. Casgevy - Exagamglogene autotemcel - Orphan - EMA/PAM/0000266947

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The outcome of the assessment was agreed. The PAM is considered resolved.

2.13.7. Casgevy - Exagamglogene autotemcel - Orphan - EMA/PAM/0000266944

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The request for supplementary information was agreed.

2.13.8. Casgevy - Exagamglogene autotemcel - Orphan - EMA/PAM/0000266941

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The request for supplementary information was agreed.

2.13.9. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/PAM/0000265655

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The outcome of the assessment was agreed. The PAM is considered resolved.

2.13.10. Breyanzi - Lisocabtagene maraleucel - Orphan - EMA/PAM/0000265380

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: PAM, quality

Action: for adoption

The outcome of the assessment was agreed. The PAM is considered resolved.

2.13.11. Hemgenix - Etranacogene dezaparvovec - Orphan - EMA/PAM/0000264977

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: PAM, quality

Action: for adoption

The outcome of the assessment was agreed. The PAM is considered resolved.

2.13.12. BEQVEZ - Fidanacogene elaparvovec - Orphan - EMA/PAM/0000262832

Pfizer Europe MA EEIG

Rapporteur: Jan Mueller-Berghaus

Scope: PAM, quality

Action: for adoption

The outcome of the assessment was agreed. The PAM is considered resolved.

2.13.13. Kymriah – Tisagenlecleucel - EMA/PAM/0000258545

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: PAM, pharmacovigilance

Action: for adoption

The request for supplementary information was agreed.

2.13.14. Libmeldy - Atidarsagene autotemcel - EMA/R/0000257479

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries; Co-rapporteur: Barbara Bonamassa

Scope: 5-year renewal, request for supplementary information

Action: for adoption

The request for supplementary information was agreed.

2.13.15. Imlygic - Talimogene laherparepvec - EMA/PAM/0000262826

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen; PRAC Rapporteur: Gabriele Maurer

Scope: PAM, PRAC led

Action: for adoption

CAT discussed the proposed change to the wording of the indication. Further justification from the MAH is requested. The request for supplementary information was agreed.

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

No items

3.2. Day 60 Evaluation Reports

No items

3.3. New Applications

No items

4. Scientific Recommendation on Classification of ATMPs

Timetable:

-Start of the procedure:	16.06.2025
-EMA Coordinator's draft report:	24.06.2025
-CAT Coordinator's comments:	02.07.2025
-Revised scientific recommendation:	11.07.2025

-CAT's discussion of scientific recommendation: 18.07.2025

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Allogeneic human midbrain dopaminergic neuron (mDA) progenitor cells derived from human pluripotent stem cells

Treatment of Parkinson's disease

Scope: Nomination of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Cell-free extracellular matrix derived from decellularized porcine skin tissue and cell-free concentrated secretome from human adipose-derived stromal cells

Treatment of perianal fistulas

Scope: Nomination of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Bone-marrow and/or adipose tissue derived mesenchymal stem cells, embedded into a 3d non-woven polymer matrix

Treatment of critical limb ischemia

Scope: Nomination of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.2. Day 30 ATMP scientific recommendation

4.2.1. Replication defective SV40 vector encoding for human proinsulin

Treatment of type 1 diabetes

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 27.06.2025.

4.2.2. Anitocabtagene autoleucel

Treatment of patients with multiple myeloma

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 27.06.2025.

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

No items

4.4. Finalisation of procedure

4.4.1. Human oocyte cytoplasma

For use in case of low human egg quality in in vitro (IVF)

Scope: ATMP scientific recommendation. European Commission raised no comments.

Action: for adoption

The classification report was adopted. The product does not fulfil the definition of advanced therapy medicinal product as provided in Article 2(1) of Regulation (EC) No. 1394/2007.

4.4.2. Selected autologous Tumour-Infiltrating Lymphocytes (TILs) isolated from tumour tissue, lymph node metastases, or peripheral blood, expanded, activated

Treatment of advanced or metastatic solid tumours

Scope: ATMP scientific recommendation. European Commission raised no comments.

Action: for adoption

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

4.4.3. Autologous T cells ex vivo transduced with LVV encoding a CAR that recognises BCMA

Treatment of relapsed or refractory form of plasmocytic myeloma (RRMM)

Scope: ATMP scientific recommendation. European Commission raised no comments.

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.

4.4.4. Genetically modified porcine heart

Intended for cardiac xenotransplantation to human patients with end-stage heart failure

Scope: ATMP scientific recommendation

Action: for adoption

The adoption of the classification report was postponed.

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:	02-05.06.2025
- Appointment of CAT Peer Reviewers:	11-13.06.2025
- SAWP first reports:	30.06.2025
- CAT Peer Reviewer comments (NC & C):	04.07.2025
- CAT Peer Reviewer comments (Q):	09.07.2025
- Discussion at SAWP:	07-10.07.2025
- Discussion at CAT and feedback to SAWP:	16-18.07.2025

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	07-10.07.2025
- Appointment of CAT Peer Reviewers:	16-18.07.2025
- SAWP first reports:	25.08.2025
- CAT Peer Reviewer comments (NC & C):	29.08.2025
- CAT Peer Reviewer comments (Q):	03.09.2025
- Discussion at SAWP:	01-04.09.2025
- Discussion at CAT and feedback to SAWP:	10-12.09.2025

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:	02-05.06.2025
SAWP recommendation:	10.07.2025
CAT recommendation:	18.07.2025
CHMP adoption of report and final recommendation:	24.07.2025

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 thanked the following members and alternates representing clinicians for their contribution: Alessandro Aiuti (member), Bernd Gansbacher (alternate), Paolo Gasparini (member) and Alessandra Renieri (alternate).

The Chair thanked the following members and alternates representing patients' organisations for their contribution: Kieran Breen (member), Federica Chiara (alternate), Kerstin Sollerbrant (member) and Mencia De Lemus Belmonte (alternate).

7.1.2. Vote by proxy

Kerstin Sollerbrant gave a proxy to Kieran Breen to vote on behalf of Patient Organisation during the entire meeting.

Eva Kolouchova gave a proxy to Margareta Fogelova to vote on behalf of Czechia during the entire meeting.

Suzana Vidic gave a proxy to Denisa Margina to vote on behalf of Slovenia on Wednesday 11 June after 16.00.

7.1.3. EMA Committee Reform

Scope: Brainstorming on the future process for the evaluation of MAAs for ATMPs

Action: for discussion

During the brainstorming session, CAT members reflected on how the ATMP specific expertise that is currently present in the CAT could be incorporated in the novel committee structure that is proposed in the reform of the pharmaceutical legislation.

7.1.4. CAT Dates (online & face-to-face) 2026

Scope: CAT Plenary 2026 dates

Action: for information

Topic postponed until the next meeting.

7.2. Coordination with EMA Scientific Committees

No items

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

7.3.1. BWP/CAT Learning on Claims for New Active Substance (NAS) status for ATMPs

Rapporteur/Coordinator: Sean Barry, Ilona Reischl

Scope: BWP/CAT learning on assessment of NAS claims for ATMPs - updated following MS comments

Action: for discussion

The BWP/CAT learning on the assessment of the NAS claim for ATMPs was presented. The learning was adopted.

7.4. Cooperation with the EU regulatory network

7.4.1. Provision of information to support Joint Clinical Assessment (JCA)

Outcome report on the discussion and development of guidance on exchange of information between EMA and the HTA secretariat in the context of Joint Clinical Assessments (JCA) for medicinal products under Regulation (EU) 2021/2282.

Action: for information

EMA presented the proposal how to exchange information between EMA and the HTA secretariat for the ongoing centralised MAA.

7.5. Cooperation with international regulators

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

CAT: Ilona Reischl

Scope: Agenda topics for the teleconference of 26.06.2025

Action: for information

The agenda for the ATMP cluster teleconference will be circulated closer to the meeting.

7.5.2. ICH Cell & Gene Therapy Discussion Group (CGTDG) 0 upcoming consultation on draft recommendations on future ATMP-related guidelines

CAT: Jan Mueller-Berghaus

Scope: Status update.

Action: for information

Jan Mueller-Berghaus provided feedback to CAT. At the ICH management group meeting in Madrid (13-14 May 2025), the proposal to develop a new Annex to the ICH guideline ICH

Q5E on comparability for ATMPs (Annex to ICH Q5E) was agreed.

7.6. CAT work plan

7.6.1. CAT work plan 2025

CAT: Ilona Reischl

Scope: Tour de table on progress of 2025 CAT workplan activities and feedback on the planned CAT workshop in genome editing.

Action: for discussion

EMA presented the progress of the different topics in the CAT workplan for 2025. CAT was informed of the data of the CAT workshop on genome editing (16.09.2025).

7.7. Planning and reporting

7.7.1. Q2-2025 Business Pipeline report for the human scientific committees

Scope: Q2-2025 Business Pipeline report for the human scientific committees

Action: for information

The information was noted.

7.8. Others

7.8.1. EMA survey: exploring the role of Patient Preference Studies in the Benefit/Risk assessment

Scope: Reminder to the committee

Action: for discussion

CAT was informed that the survey is still open for input. All CAT members were encouraged to participate. See CAT minutes of May 2025 (7.8.2) for more information.

7.8.2. FDA Roundtable on Cell and Gene Therapy

CAT: Ilona Reischl

Scope: https://www.youtube.com/live/0qDhRtnHXdU

Action: for information

The information was noted.

8. Any other business

No items

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 11-13 June 2025 CAT meeting.

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 restrictions applicable to this meeting	
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	
Petra Sokol	Alternate	Croatia	No interests declared	
Rafaella Pontou	Member	Cyprus	No interests declared	
Eva Kolouchová	Member	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 restrictions applicable to this meeting	
Pille Saalik	Alternate	Estonia	No interests declared	
Heli Suila	Member	Finland	No interests declared	
Maija Tarkkanen	Alternate	Finland	No interests declared	
Violaine Closson Carella	Member	France	No interests declared	
Jean-Michel Race	Alternate	France	No restrictions applicable to this meeting	
Jan Mueller-Berghaus	Member (CHMP	Germany	No interests declared	

	co-opted member)			
Maria Gazouli	Member	Greece	No restrictions applicable to this meeting	
Angeliki Rompoti	Alternate	Greece	No restrictions applicable to this meeting	
Viola Bardoczy	Member	Hungary	No restrictions applicable to this meeting	
Agnes Zotter	Alternate	Hungary	No restrictions applicable to this meeting	
Péter Zsolt Fekete	Member	Iceland	No interests declared	
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	
Vilma Perikaite	Member (CHMP member)	Lithuania	No restrictions applicable to this meeting	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No restrictions applicable to this meeting	
Alessia Pochesci	Member	Luxembourg	No restrictions applicable to this meeting	
Emmely de Vries	Member	Netherlands	No interests declared	
Berendina Maria (Tineke) van den Hoorn	Alternate	Netherlands	No interests declared	
Rune Kjeken	Member	Norway	No interests declared	
Ole Henrik Myrdal	Alternate	Norway	No interests declared	
Dariusz Sladowski	Member	Poland	No restrictions applicable to this meeting	
Maria Isabel Borba Vieira	Member	Portugal	No interests declared	
Denisa Marilena Margina	Member	Romania	No restrictions applicable to this meeting	
Liviu Nitulescu	Alternate	Romania	No restrictions applicable to this meeting	
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	
Maria Luttgen	Member	Sweden	No participation in discussion, final deliberations and voting on:	2.2.1. Delandistrogene moxeparvovec 2.7.1. In vitro diagnostic medical device
Charlotte Anderberg	Alternate	Sweden	No interests declared	
Bernd Gansbacher	Alternate	Clinicians' Representative	No interests declared	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No restrictions applicable to this meeting	
Catherine Milne	Observer/ Alternate	EDQM	No interests declared	
Torbjörn Callréus	Expert	Malta	No interests declared	
Christian Gartner	Expert	Austria	No interests declared	
Melanie Ramberger	Expert	Austria	No interests declared	
Brigitte Müller	Expert	Austria	No interests declared	
Tobias Fellinger	Expert	Austria	No restrictions applicable to this meeting	
Ingrid Bijsmans	Expert	Netherlands	No interests declared	
Sanne ten Dam	Expert	Netherlands	No interests declared	
Marja van de Bovenkamp	Expert	Netherlands	No interests declared	
Charlotte de Wolf	Expert	Netherlands	No interests declared	
Marc Maliepaard	Expert	Netherlands	No interests declared	
Viktoriia Starokozhko	Expert	Netherlands	No restrictions applicable to this meeting	
Sonia Roldan-Munoz	Expert	Netherlands	No restrictions applicable to this meeting	
Lies van Vlijmen	Expert	Netherlands	No interests declared	
Marleen Huigen	Expert	Netherlands	No restrictions applicable to this meeting	
Marlinda Hupkes	Expert	Netherlands	No interests declared	
Kristina Dunder	Expert	Sweden	No interests declared	
Elina Rönnemaa	Expert	Sweden	No interests declared	
Andreea Barbu	Expert	Sweden	No interests declared	

Pernilla Örtqvist	Expert	Sweden	No interests declared	
Tilo Moede	Expert	Sweden	No interests declared	
Attila Sebe	Expert	Germany	No interests declared	
Kathrin Bayanga	Expert	Germany	No interests declared	
Elizabeth Vroom	Expert	Patients' Representative	No interests declared	
Dimitrios Athanasiou	Expert	Patients' Representative	No interests declared	
Peter Mol	Expert	Netherlands	No restrictions applicable to this meeting	
Hester Peltenburg	Expert	Netherlands	No interests declared	
Marja van de Bovenkamp	Expert	Netherlands	No interests declared	
Charlotte Welsh	Expert	Sweden	No interests declared	
Macarena Gajardo Álvarez	Expert	Spain	No interests declared	
Katelijne De Swert	Expert	Belgium	No interests declared	
Filip Josephson	Expert	Sweden	No restrictions applicable to this meeting	
Odoardo Olimpieri	Expert	Italy	No interests declared	
Antonella Isgrò	Expert	Italy	No interests declared	
Federico De Angelis	Expert	Italy	No interests declared	
Jayne Crowe	Expert	Ireland	No interests declared	
A representative 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.				

Date of next CAT meeting:

16-18 July 2025

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

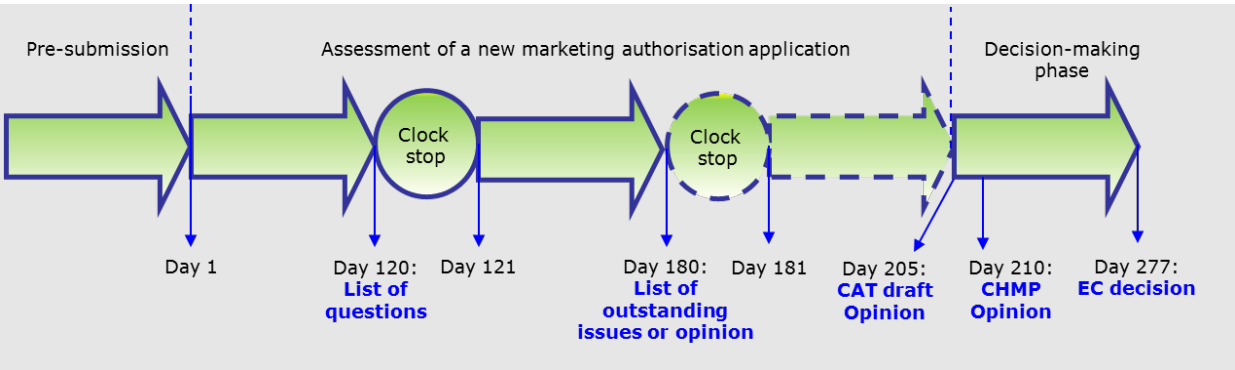
Evaluation of ATMPs (section 2)

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 2.10.)

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/