14 April 2025
EMA/CAT/141549/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 19-21 March 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).

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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 connected 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 welcome the new member.

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

1.2. Adoption of agenda

The CAT agenda for 19-21 March 2025 meeting was adopted with an addition to 6.3.4.

1.3. Adoption of the minutes

The CAT minutes for 19-21 February 2025 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

2.3.1. Obecabtagene autoleucel - PRIME - Orphan - EMEA/H/C/005907

Autolus GmbH; Treatment of patients with relapsed or refractory B cell precursor acute lymphoblastic leukaemia (ALL)

Scope: Day 180 list of outstanding issues

Action: for adoption

List of questions adopted on 19.07.2024.

The Rapporteurs presented the assessment of the responses to the list of questions.

The list of outstanding issues was adopted.

2.3.2. 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: Day 180 list of outstanding issues

Action: for adoption

List of questions adopted on 19.04.2024.

The Rapporteurs presented the assessment of the responses to the list of questions.

The list of outstanding issues was adopted. The CAT did not agree to the request from the applicant for an extension to the clock-stop to respond to the list of outstanding issues.

2.3.3. 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: Day 180 list of outstanding issues

Action: for adoption

List of questions adopted on 11.10.2024.

The Rapporteurs presented the assessment of the responses to the list of questions.

The list of outstanding issues was adopted. The CAT agreed to the request from the applicant for an extension to the clock-stop to respond to the list of outstanding issues.

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

2.5.1. 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: Day 80 assessment report

Action: for information

The information was noted.

2.5.2. 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: Day 80 assessment report

Action: for information

The information was noted.

2.6. Update on ongoing initial applications

No items

2.7. New applications

2.7.1. Onasemnogene abeparvovec - H0006498

Treatment of patients with spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene

Scope: Briefing note and the Rapporteurs' recommendation on the request for accelerated assessment

Action: for adoption

CAT did not agree to the request for accelerated assessment and adopted the briefing note and Rapporteurs' recommendation on the Request for Accelerated Assessment.

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. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0058/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 21.02.2025.

The opinion was adopted.

2.11.2. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0036

Janssen-Cilag International NV

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

Scope: Clinical, opinion

Update of sections 4.8, and 5.1 of the SmPC in order to update the list of adverse drug reactions (ADRs), and update clinical efficacy and safety information based on second interim analysis from study 68284528MMY3002 (CARTITUDE-4); this is a phase 3 randomized study comparing ciltacabtagene autoleucel, a chimeric antigen receptor T cell (CAR-T) therapy directed against BCMA, versus Pomalidomide, Bortezomib and Dexamethasone (PVd) or Daratumumab, Pomalidomide and Dexamethasone (DPd) in subjects with relapsed and lenalidomide-refractory multiple myeloma; The RMP version 5.3 has also been submitted. In addition, the MAH took the opportunity to update the list of local representatives in the Package Leaflet.

Action: for adoption

Request for supplementary information adopted on 21.02.2025.

The Rapporteur presented the outcome of the assessment of the responses to the request for supplementary information. The update of the sections 4.8 and 5.1 was agreed. The opinion was adopted.

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

Novartis Europharm Limited

Rapporteur: Rune Kjeken, PRAC Rapporteur: Gabriele Maurer

Scope: Safety, opinion

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 HCP educational programme in the Annex II in order to enhance readability.

Action: for adoption

Request for supplementary information adopted on 24.01.2025.

The Rapporteur presented the outcome of the assessment of the responses to the request for supplementary information. The opinion was adopted.

2.11.4. Luxturna - Voretigene neparvovec - Orphan - EMEA/H/C/004451/II/0054/G

Novartis Europharm Limited

Rapporteur: Sol Ruiz

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.5. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2821/G

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.6. Ebvallo - Tabelecleucel - EMA/VR/0000245074

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.7. Tecartus, Yescarta - Brexucabtagene autoleucel, Axicabtagene ciloleucel - EMA/VR/0000242383

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

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. Casgevy - Exagamglogene autotemcel - Orphan - EMEA/H/C/005763/MEA/011.1

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical & Pharmacovigilance (PRAC-led procedure)

From initial MAA

Revised Protocol Version 2.0 for PASS no. VX24-290-102

Title: Healthcare Professional Survey (HCP) to Assess the Effectiveness of the Additional Risk Minimization Measures (aRMM) for Casgevy® (exagamglogene autotemcel)

Action: for information (PRAC-led procedure)

The information was noted.

2.13.2. Imlygic - Talimogene laherparepvec - EMEA/H/C/002771/MEA/005.3

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: 6th Interim Report for PASS 20130193 (PRAC-led procedure)

A post-marketing, prospective cohort study of patients treated with talimogene laherparepvec in clinical practice to characterize the risk of herpetic illness among patients, close contacts, and healthcare providers; and long term safety in treated patients.

Annual interim reports to be included in the PSUR and DSUR.

Action: for information (PRAC-led procedure)

The information was noted.

2.13.3. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/ANX/002.6

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Response to MEA 002.5 (PRAC-led procedure):

Revised Protocol #5 for Study KTE-EU-472-6036
Title: Long-term, non-interventional study of recipients of Tecartus (brexucabtagene autoleucel) for treatment of adult patients with relapsed or refractory Mantle Cell Lymphoma (MCL).

Action: for information

The information was noted.

2.13.4. Tecartus - Brexucabtagene autoleucel - EMA/PAM/0000245367

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: ANX/0101/1 Clinical and Pharmacovigilance

Action: for adoption

The Rapporteur's assessment report was adopted.

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

3.3. New Applications

No items

4. Scientific Recommendation on Classification of ATMPs

Timetable:

-Start of the procedure:	21.03.2025
-EMA Coordinator's draft report:	01.04.2025
-CAT Coordinator's comments:	09.04.2025
-Revised scientific recommendation:	09.04.2025
-CAT's discussion of scientific recommendation:	16.04.2025

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Allogeneic T cells genetically modified ex vivo using CRISPR/Cas9 to express an anti-CD19 chimeric antigen receptor

Treatment of frontotemporal dementia (FTD) in adults who have a mutation in the GRN or chromosome 9 open reading frame 72 (C9orf72) genes

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Induced pluripotent stem cell (iPSC)-derived photoreceptor precursor cells

Treatment of primary photoreceptor disease

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Messenger mRNA encoding the human Dynein Axonemal Intermediate Chain 1 (DNAI1) protein

Treatment of primary ciliary dyskinesia caused by mutations in the DNAI1 gene

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.4. Messenger mRNA encoding the cystic fibrosis transmembrane conductance regulator (CFTCR) protein

Treatment of cystic fibrosis

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.5. Allogeneic genetically modified T-cells expressing two chimeric antigen receptors (CARs) targeting the human CD19 and CD70 proteins

Treatment of autoimmune diseases

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.2. Day 30 ATMP scientific recommendation

4.2.1. mRNA transfected macrophages cultured from autologous monocytes

Treatment of end stage liver 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 04.04.2025.

4.2.2. Genetically modified porcine heart

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

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 04.04.2025.

4.2.3. Genetically modified Escherichia coli bacteria engineered to carry genes to metabolize tryptophan and genes to use an exogenously administered sugar source

Treatment of type 2 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 04.04.2025.

4.2.4. mRNAs encoding modified C. acnes protein

Treatment of acne

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 04.04.2025.

4.2.5. Autologous tumour-derived dendritic cells

Prevention of relapse and metastasis of non-small cell lung carcinoma (NSCLC)

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT considered that additional information should be provided by the applicant.

The procedural clock is stopped awaiting the responses from the applicant.

4.2.6. Adeno-associated virus (AAV) serotype 1 (AAV1) vector containing the human granulin precursor (GRN) cDNA encoding progranulin (PGRN)

Treatment of frontotemporal dementia (FTD) in adults who have a mutation in the GRN or chromosome 9 open reading frame 72 (C9orf72) genes

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 04.04.2025.

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

No items

4.4. Finalisation of procedure

No items

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:	10-13.03.2025
- Appointment of CAT Peer Reviewers:	19-21.03.2025
- SAWP first reports:	31.03.2025
- CAT Peer Reviewer comments (NC/C):	04.04.2025
- CAT Peer Reviewer comments (Q):	09.04.2025
- Discussion at SAWP:	07-10.04.2025
- Discussion at CAT and feedback to SAWP:	14-16.04.2025

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	07-10.04.2025
- Appointment of CAT Peer Reviewers:	14-16.04.2025
- SAWP first reports:	28.04.2025
- CAT Peer Reviewer comments (NC & C):	02.05.2025
- CAT Peer Reviewer comments (Q):	07.05.2025
- Discussion at SAWP:	05-08.05.2025
- Discussion at CAT and feedback to SAWP:	14-16.05.2025

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

No items

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:

Procedure start:	10-13.03.2025
SAWP recommendation:	13.03.2025
CAT recommendation:	21.03.2025
CHMP adoption of report and final recommendation:	25.04.2025

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

6.3.4. Ongoing support

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

The Chair welcomed Péter Zsolt Fekete, as the new member for Iceland.

7.1.2. Vote by proxy

Action: for information

Maria Gazouli gave a proxy to Isavella Kyriakidou to vote on behalf of Greece during the entire meeting.

Alessia Pochesci gave a proxy to Claire Beuneu to vote on behalf of Luxembourg on Friday 21 March 2025.

Concetta Quintarelli gave a proxy to Violaine Closson-Carela to vote on behalf of Italy on Friday 21 March 2025.

Kerstin Sollerbrant gave a proxy to Kieran Breen to vote on behalf of her during 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

The agenda for the upcoming SRLM was finalised.

Members were reminded to apply for accreditation as soon as the invitation letter arrives .

7.1.4. Onboarding Programme for CAT members and alternates

CAT: Ilona Reischl

Scope: Onboarding Programme, revision 1

Action: for adoption

The revision 1 of the Onboarding Programme was adopted.

7.1.5. Revision of EMA policy 0044 on handling of competing interests

Scope: The main changes in the revision of policy 044, the updated declaration of interests form and the next steps for experts will be presented

Action: for information

The main changes in the revision of policy 0044, the updated declaration of interests form and the next steps for experts were presented to the committee. Committee members and all experts were requested to submit an updated declaration of interests by 1 May 2025.

7.2. Coordination with EMA Scientific Committees

7.2.1. CDx expert group - IVD/CDx information

CAT: Olga Kholmanskikh

Scope: IVD/CDx information in assessment report and in the MAA

Action: for endorsement

Olga Kholmanskikh presented the proposal for an update of the assessment report template to capture information on in vitro biomarker tests for patient selection for efficacy and/or safety.

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

7.3.1. Patients and Consumers Working Party (PCWP) Healthcare Professionals Working Party (HCPWP)

7.3.1.1. Updates to Rules of Procedure and mandates for PCWP and HCPWP

Scope: Updates to Rules of Procedure and mandates for PCWP and HCPWP

Action: for adoption

The updated of the PCWP and HCPWP Rule of procedures was agreed.

7.3.1.2. Patients and Consumers Working Party (PCWP) Healthcare Professionals Working Party (HCPWP) - Agenda and minutes

Scope:

- Meeting summary of the PCWP/HCPWP and all eligible organisations meeting held on 20 November 2024
- Draft Agenda of the PCWP-HCPWP meeting to be held on 1-2 April 2025

Action: for information

The information was noted.

7.4. Cooperation with the EU regulatory network

7.4.1. Revisions made to the Reflection Paper on Real-World Evidence (RWE)

Scope: Present the main changes introduced to the reflection paper on Use of real-world data (RWD) in non-interventional studies (NIS) to generate RWE for regulatory purposes after the public consultation

Action: for information

The main changes made to the draft reflection paper on RWE was presented. The reflection paper will be published in April/May 2025.

7.4.2. Engineered living materials (ELMs)

Scope: Presentation of the Horizon scanning report on ELMs

Action: for discussion

The horizon scanning report of ELMs was presented. Comments from CAT are awaited by 03.04.2025, especially on the recommendation section.

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: Feedback from the teleconference of 27.02.2025

Action: for information

A short feedback was provided from the discussion at the ATMP cluster of 27.02.2025.

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

Scope: Update on the CoGenT pilot

Action: for information

EMA provided feedback on the ongoing CoGenT pilot.

7.5.3. ICH E22 - General Considerations for Patient Preference Studies (PPS)

Scope: Presentation of the outline of the ICH E22 guideline

Action: for discussion

EMA presented the outline of the ICH E22 guideline. CAT comments are awaited by 31.03.2025

CAT was also informed of a survey that will be initiated with scientific committees and assessors on whether formal patient preference studies are useful to inform benefit risk assessment. The short survey will be sent to the CAT members and the members were asked to forward it to their assessors.

7.6. CAT work plan

7.6.1. Guideline on requirements for investigational ATMPs in clinical trials

CAT: Ilona Reischl

Scope: Organisation of training/webinar on the guideline: plan of actions

The following CAT members will be involved on a brainstorming meeting with the CAT chair and CAT secretariat on the organisation of the training and the webinar: Suzana Vidic, Viola Bardoczy, Joseph De Courcey, Heli Suila, Isabel Vieira, Clarie Beuneu.

Action: for discussion

7.6.2. Publication of clinical trial in EU

CAT: Ilona Reischl

Scope: confirmation of list of authors and call to CAT members to identify scientific publications where there is reference to the number of ATMP clinical trials in EU.

Action: for discussion

The following members from CAT and CTCG will be involved in the preparation of the manuscript: Ilona Reischl, Dariusz Sladowski, Denisa Margina, Olga Kholmanskikh. CAT members were asked to include any publication that make reference to the number of ATMP trials in the EU.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. Draft Reflection Paper on Patient Experience Data

Scope: Presentation of the draft reflection paper.

Action: for information

Note: A drafting group composed of members from EMA committees (CHMP, COMP, PDCO, PRAC and CAT) and selected WPs (SAWP, ONCWP, PCWP, HCPWP) has drafted a reflection paper (RP) on Patient Experience Data. The RP provides a framework for discussion and clarifies in particular areas where scientific knowledge is fast evolving or regulatory experience is limited. The RP is complementary to the ICH guidance work and is not intended to be a methodological guidance.

The draft reflection paper was circulated to the relevant committees and WPs for internal consultation. CAT comments are awaited by 31.03.2025. Public consultation is foreseen in Q3 2025.

7.8.2. Unauthorised Dendritic cell therapies

Scope: EMA-HMA statement: 'Unregulated advanced therapy medicinal products pose serious risks to health'

Action: for information

The information was noted.

8. Any other business

8.1. Health & Safety Video

Scope: To remind delegates of Health and Safety procedures in the EMA building

Action: for information

The information was noted.

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 19-21 March 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 restrictions applicable to this meeting	
Isavella Kyriakidou	Alternate	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-opted member)	Germany	No interests declared	
Egbert Flory*	Alternate (to CHMP representative)	Germany	No interests declared	
Angeliki Rompoti*	Alternate	Greece	No restrictions applicable to this meeting	
Viola Bardoczy	Alternate	Hungary	No restrictions applicable to this meeting	
Péter Zsolt Fekete*	Member	Iceland	No interests declared	

Joseph De Courcey	Member	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	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No interests declared	
Vilma Perikaite*	Member (CHMP member)	Lithuania	No interests declared	
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 restrictions applicable to this meeting	
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 interests declared	
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	
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	

Alessandra Renieri	Alternate	Clinicians' Representative	No restrictions applicable to this meeting	
Bernd Gansbacher	Alternate	Clinicians' Representative	No interests declared	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No interests declared	
Torbjörn Callréus	Expert	Malta	No interests declared	
Charlotte de Wolf	Expert	Netherlands	No interests declared	
Jolien de Groot	Expert	Netherlands	No interests declared	
Annemarie den Harder	Expert	Netherlands	No restrictions applicable to this meeting	
Gerlienke Geurts-Voerman	Expert	Netherlands	No interests declared	
Peter Mol	Expert	Netherlands	No interests declared	
Hester Peltenburg	Expert	Netherlands	No interests declared	
Victoria Hamelinck	Expert	Netherlands	No restrictions applicable to this meeting	
Andreea Barbu	Expert	Sweden	No interests declared	
Elina Rönnemaa	Expert	Sweden	No interests declared	
Xavier Kurz	Expert	Belgium	No interests declared	
Kristina Dunder	Expert	Sweden	No interests declared	
Hilke Zander	Expert	Germany	No interests declared	
Martina Schüßler-Lenz	Expert	Germany	No interests declared	
Juliane Rau	Expert	Germany	No interests declared	
Monique Wakelkamp	Expert	Sweden	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.				

Date of next CAT meeting:

14-16 April 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

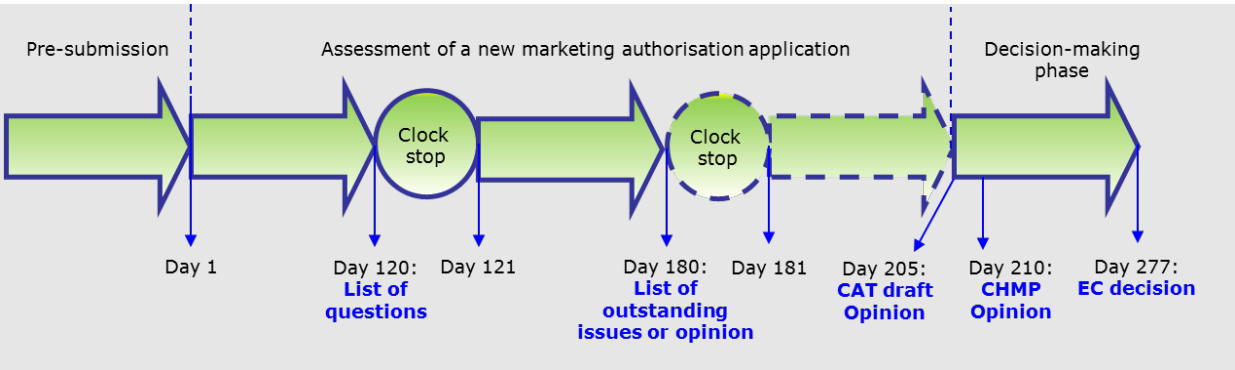
Evaluation of ATMPs (section 2)

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/