25 February 2025
EMA/CAT/115813/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 19-21 February 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 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.

1.2. Adoption of agenda

The CAT agenda for 19-21 February 2025 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for 22-24 January 2025 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

2.1.1. Beremagene geperpavec - PRIME - Orphan - EMEA/H/C/006330

Krystal Biotech Netherlands B.V.; Treatment of patients from birth with dystrophic epidermolysis bullosa (DEB) with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene

Scope: Opinion

The Rapporteur presented the outcome of the assessment of the responses to the second list of outstanding issues.

CAT recommended by majority (27 out of 28 votes) to grant of a full marketing authorisation (with an imposed PASS) to Vyjuvek. Luxembourg voted divergent on the type of marketing authorisation granted, on the basis that the clinical data in the patient population reflected in the indication were not considered comprehensive. Norway voted positive.

Action: for adoption

List of outstanding issues adopted on 06.12.2024 and 11.10.2024. List of questions adopted on 15.03.2024.

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

No items

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, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.2. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/II/0055/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

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

Janssen-Cilag International NV

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

Scope: Clinical, request for supplementary information

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

The Rapporteur presented the assessment of this variation. The request for supplementary information was adopted.

2.11.4. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0037

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.5. Casgevy - Exagamglogene autotemcel - Orphan - EMEA/H/C/005763/II/0009/G

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 06.12.2024.

The opinion was adopted.

2.11.6. Casgevy - Exagamglogene autotemcel - Orphan - EMEA/H/C/005763/II/0012/G

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.7. Kymriah - Tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0086/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Safety, opinion

A grouped application consisting of:

C.I.4: Update of section 4.4 of the SmPC in order to amend existing warnings on cytokine release syndrome based on literature.

C.I.4: Update of section 4.4 of the SmPC in order to amend existing warnings on neurological adverse reaction based on literature. The Package Leaflet is updated accordingly.

C.I.4: Update of section 4.4 of the SmPC in order to amend existing warnings on hypersensitivity reactions based on post marketing data and literature. The Package Leaflet is updated accordingly. In addition, the MAH took the opportunity to update the list of local

representatives in the Package Leaflet and to implement editorial changes to the PI.

Action: for adoption

Request for supplementary information adopted on 06.12.2024 and 11.10.2024.

The Rapporteur presented the outcome of the assessment. The opinion was adopted.

2.11.8. Libmeldy - Atidarsagene autotemcel - Orphan - EMEA/H/C/005321/II/0031/G

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 08.11.2024.

The opinion was adopted.

2.11.9. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/II/0014

BioMarin International Limited

Rapporteur: Violaine Closson Carella; PRAC Rapporteur: Bianca Mulder

Scope: Clinical, opinion

Update of the Annex II in order to propose changes to the current marketing authorisation obligations for ROCTAVIAN. The RMP version 1.3 has also been submitted.

Action: for adoption

Request for supplementary information adopted on 08.11.2024.

The Rapporteur presented the outcome of the assessment. The opinion was adopted.

2.11.10. Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0085

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical, request for supplementary information

Submission of the final report from study KT-US-482-0147 (ZUMA-26). This is a Prospective, Noninterventional, Clinical Efficacy Study Investigating and Analyzing the Impact of Tumor CD19 Antigen Expression and Density on Response to Axicabtagene Ciloleucel Treatment using a quantitative flow cytometry method.

Action: for adoption

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

2.11.11. Tecartus - Brexucabtagene autoleucel; Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/WS2736

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 06.12.2024 and 13.09.2024.

The opinion was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/REC/009

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, REC fulfilled

Action: for adoption

The outcome of the assessment was adopted.

2.13.2. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/ANX/004.3

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Clinical

From initial MAA

Annual Progress Report for Study 270-801 (EU PAS 49243; GENEr8-GTR):
A Retrospective Cohort Study of Patients Treated with ROCTAVIAN (valoctocogene roxaparvovec).

Action: for adoption

The outcome of the assessment was adopted.

2.13.3. Upstaza - Eladocagene exuparvovec - Orphan - EMEA/H/C/005352/S/0025

PTC Therapeutics International Limited

Rapporteur: Joseph DeCourcey, Co-Rapporteur: Maria Luttgen, PRAC Rapporteur: Gabriele Maurer

Scope: Annual Re-assessment

Action: for adoption

Request for supplementary information adopted on 06.12.2024.

The MAH responded adequately to the request for supplementary information. The benefit risk profile remains positive. The update to the section 4.8 of the SmPC (updated statement on dyskinesia and summary of the safety profile) is agreed. The second annual re-assessment was adopted.

2.13.4. Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/ANX/002.7

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Pharmovigilance

From initial MAA Fourth Annual Interim Report for PASS KT-EU-471-0117:
Title: Long-term, non-interventional study of recipients of Yescarta for treatment of relapsed or refractory Diffuse Large B-cell Lymphoma and Primary Mediastinal B-cell Lymphoma (EU PAS Register no.: EUPAS32539).

Action: for information

CAT noted the outcome of the PRAC discussion. No new safety issues are identified.

2.13.5. Casgevy - Exagamglogene autotemcel - EMEA/H/C/PSA/S/0113

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Blanca Mulder

Scope: Pharmacovigilance

PRAC assessment of substantial amendments to a non-interventional imposed PASS protocol - VX22-290-101 (Version 2.1): Long-term registry-based study of patients with transfusion-dependent β-thalassemia (TDT) or sickle cell disease (SCD) treated with exagamglogene autotemcel (exa-cel).

Action: for information

CAT noted the outcome of the PRAC discussion.

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:	21.02.2025
-EMA Coordinator's draft report:	07.03.2025
-CAT Coordinator's comments:	12.03.2025
-Revised scientific recommendation:	14.03.2025
-CAT's discussion of scientific recommendation:	21.03.2025

4.1. New requests – Appointment of CAT Coordinator

4.1.1. mRNA transfected macrophages cultured from autologous monocytes

Treatment of end stage liver disease

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Genetically modified porcine heart

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

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

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

Treatment of type II diabetes

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.4. mRNAs encoding modified C.acnes protein

Treatment of acne

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.5. Autologous tumour-derived dendritic cells

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

Scope: Appointment of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.6. 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: Appointment of CAT coordinator

Action: for adoption

The CAT coordinators were appointed.

4.2. Day 30 ATMP scientific recommendation

No items

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

No items

4.4. Finalisation of procedure

4.4.1. mRNAs encoding IL-12 and IL-18

Treatment of gastric cancer

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.

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

Action: for adoption

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

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

Treatment of Myasthenia Gravis

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.

4.4.4. 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: 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.

4.5. 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:	10-13.02.2025
- 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:	19-21.03.2025

5.1.2. Scientific advice procedures starting at the next SAWP meeting

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

5.2. 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

6.2.1. Overview of ITF activities in 2024

Scope: Summary of the main ITF activities and trends in 2024

Action: for information

The information was noted.

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:	
Procedure start:	10-13.02.2025
SAWP recommendation:	13.03.2025
CAT recommendation:	21.03.2025
CHMP adoption of report and final recommendation:	27.03.2025

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

No items

7.1.2. Vote by proxy

Action: for information

Maria Gazouli gave a proxy to Rafaella Pontou to vote on behalf of Greece 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

CAT was informed of the discussions between the CAT and COMP chair and the Polish CAT and COMP members on the agenda points for the joint CAT-COMP session. CAT agreed with the proposal.

CAT discussed the topics for the CAT only session of the SRLM. Additional topics were proposed.

7.1.4. Committee Meeting Dates for 2027-2028

Scope: Proposed committee meeting dates for the period of 2027-2028

Action: for information

The information was noted.

7.1.5. GIREX - Group for Internal Rules on Extensions of Clock Stops

Scope: Update

Action: for adoption

CAT noted the outcome of GIREX activities

7.1.6. Joint CHMP-CAT membership

Scope: Call for expression of interest on the appointment of CHMP members to the Committee for Advanced Therapies (CAT)

Action: for discussion

EMA presented the call for expression of interest on the appointment of CHMP members to the CAT. This call was also launched to the CHMP at the PROM meeting of 17.02.2025. Expression of interest (by CHMP members) is awaited by 12.03.2025.

7.1.7. Onboarding Programme for CAT members and alternates

CAT: Ilona Reischl

Scope: Onboarding Programme, revision 1

Action: for adoption

CAT members to review the revised document and to provide comments to the CAT secretariat by 12.03.2025. Adoption will take place at the March CAT plenary meeting.

7.2. Coordination with EMA Scientific Committees

No items

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

No items

7.4. Cooperation with the EU regulatory network

7.4.1. Update on Real-World Evidence, including DARWIN EU®

Scope: Status of CAR-T cell therapy framework study and update about DARWIN EU

Action: for information

CAT noted the update on the CAR T study and the information on the increase of the DARWIN EU network, ongoing and finalised studies and upcoming events.

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: Draft agenda of the teleconference of 27.02.2025

Action: for information

The draft agenda was presented and CAT members were identified to contribute to the agenda topics.

7.6. CAT work plan

7.6.1. Guideline on requirements for investigational ATMPs in clinical trials

CAT: Ilona Reischl

Scope: Prepare a scientific publication on the guideline and the analysis of clinical trials with ATMPs in the EU and organisation of training/webinar on the guideline: plan of actions

Action: for discussion

CAT had a discussion on the content of the scientific publication. The following CAT members will actively contribute to the preparation of the manuscript: Ilona Reischl (lead author), Olga Kholmanskikh, Dariusz Sladowski, Denisa Margina; CTCG members from the Netherlands and Austria will also contribute.

Discussion on the training and webinar on the guideline was postponed until the next meeting.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. Draft MHRA guideline on individualised mRNA cancer immunotherapies

CAT: Ilona Reischl

Scope: Draft guideline, published for consultation (deadline 31.03.2025)

Action: for information

Note: Link to the document

The draft guideline was noted.

7.8.2. Neo-antigen peptide vaccines as magistral preparation?

CAT: Ilona Reischl

Scope: Short feedback from a discussion with FDA

Action: for information

Ilona Reischl presented information on an Austrian situation where a neo-antigen based cancer immunotherapy would be made as magistral preparation (in a pharmacy). CAT members were not aware of similar situations in their countries.

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 19-21 February 2025 CAT meeting, which was virtually.

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	
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	
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	
Maija Tarkkanen	Alternate	Finland	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	
Viola Bardoczy	Alternate	Hungary	No restrictions applicable to this meeting	
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	
Alessia Pochesci	Member	Luxembourg	No restrictions applicable to this meeting	

Nancy De Bremaeker	Alternate	Luxembourg	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:	5.4.10.
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	
Katarina Kollarova	Member	Slovakia	No interests declared	
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	
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	
Kerstin Sollerbrant Melefors	Member	Patients' Representative	No interests declared	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No interests declared	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Torbjörn Callréus	Expert	Malta	No interests declared	

Finbarr Leacy	Expert	Ireland	No interests declared	
Caoimhin Concannon	Expert	Ireland	No interests declared	
Elma O'Reilly	Expert	Ireland	No interests declared	
Attila Sebe	Expert	Germany	No interests declared	
Jayne Crowe	Expert	Ireland	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:

19-21 March 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

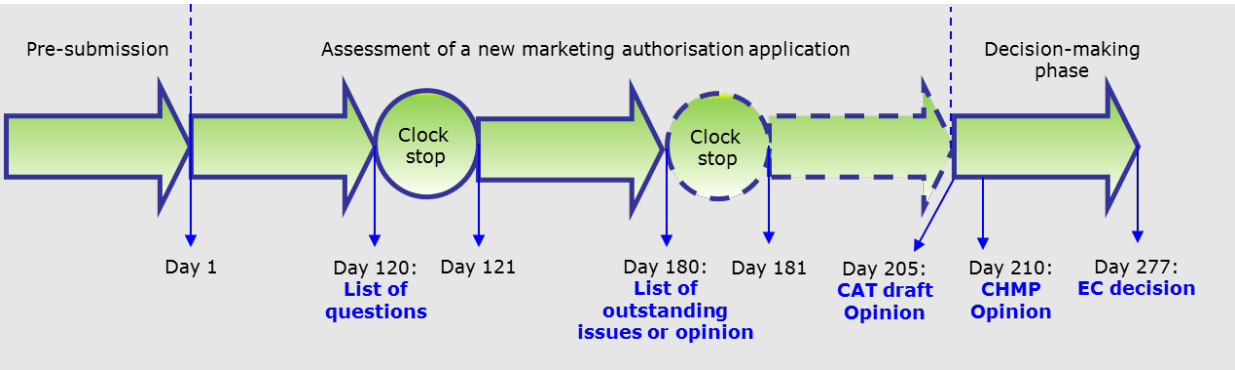
Evaluation of ATMPs (section 2)

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/