16 July 2025
EMA/CAT/232974/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 16-18 July 2025

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

16 July 2025, 14:00 – 18:30

17 July 2025, 09:00 – 18:30

18 July 2025, 09:00 – 13:00

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 this agenda 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, this agenda is a working document primarily designed for CAT members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the agenda 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

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the CAT plenary session to be held 16-18 July 2025. See July 2025 CAT minutes (to be published post September 2025 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 16-18 July 2025 meeting

1.3. Adoption of the minutes

CAT minutes for 11-13 June 2025 meeting

2. Evaluation of ATMPs

2.1. Opinions

2.1.1. Lifileucel - EMEA/H/C/004741

Treatment of unresectable or metastatic melanoma

Scope: Opinion

Action: for adoption

List of outstanding issues adopted on 16.05.2025. List of questions adopted on 06.12.2024.

2.1.2. 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: Opinion

Action: for adoption

List of outstanding issues adopted on 16.04.2025. List of questions adopted on 11.10.2024.

2.1.3. 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: Opinion

Action: for adoption

List of outstanding issues adopted on 13.06.2025, 21.03.2025. List of questions adopted on 19.04.2024.

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

2.4.1. Autologous melanoma-derived tumour infiltrating lymphocytes, ex vivo-expanded - EMEA/H/C/006563

Treatment of melanoma

Scope: Day 120 list of questions

Action: for adoption

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. Yescarta, Tecartus - Axicabtagene ciloleucel, Brexucabtagene autoleucel - Orphan - EMA/VR/0000255932

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

2.11.2. Hemgenix - Etranacogene dezaparvovec - Orphan - EMA/VR/0000270071

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, request for supplementary information

Action: for adoption

2.11.3. Breyanzi - Lisocabtagene maraleucel - Orphan - EMA/VR/0000272242

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli, PRAC rapporteur: Gabriele Maurer

Scope: Clinical, request for supplementary information

Update of sections 4.2, 4.4, 4.7 and 4.8 of the SmPC in order to update the post-treatment safety monitoring information based on clinical trials and real-world data. The Package leaflet section is updated in accordance. Version 8.0 of the RMP has also been submitted. In addition, the MAH the opportunity to update Annex II.

Action: for adoption

2.11.4. Breyanzi - Lisocabtagene maraleucel - Orphan - EMA/VR/0000249056

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, opinion

Action: for adoption

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

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical, opinion

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

2.11.6. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/VR/0000272240

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

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

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality

Action: for adoption

2.11.8. Ebvallo - Tabelecleucel - Orphan - EMA/VR/0000267364

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, opinion

Action: for adoption

2.11.9. Casgevy - Exagamglogene autotemcel - Orphan - EMA/VR/0000258214

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical, opinion

Update of section 5.3 of the SmPC in order to update non-clinical information and remove existing wording on off-target editing based on (1) Results of in silico analysis to nominate genetic variants and (2) Interim study report presenting results from genotyping of subjects and off-target editing in drug product from subjects in clinical Studies 111 and 121. In

addition, the MAH took the opportunity to make editorial changes to the PI.

Action: for adoption

2.11.10. Breyanzi - Lisocabtagene maraleucel – Orphan - EMA/VR/0000265024

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli; Co-Rapporteur: Claire Beuneu; PRAC Rappourteur: Gabriele Maurer

Scope: Clinical, request for supplementary information

A grouped application comprised of two Type II variations, as follows:

Type II (C.I.6): Extension of indication to include the treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL) after at least two lines of systemic therapy, including a Bruton's tyrosine kinase (BTK) inhibitor for BREYANZI, based on results from the pivotal Study 017001 MCL Cohort (TRANSCEND-NHL-001); this is a Phase 1, Multicenter, Open-Label Study of JCAR017, CD19-targeted Chimeric Antigen Receptor (CAR) T Cells, for Relapsed and Refractory (R/R) B-cell Non-Hodgkin Lymphoma (NHL). As a consequence, sections 4.1, 4.4, 4.8, 5.1 and 5.2 of the SmPC are updated. The Package leaflet is updated in accordance. Version 7.0 of the RMP has also been submitted. In addition, the MAH took the opportunity to introduce minor editorial changes to the PI and to update the list of local representatives in the Package Leaflet.

Action: for adoption

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Tecartus - Brexucabtagene autoleucel - Orphan - EMA/PAM/0000267756

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, PRAC rapporteur: Bianca Mulder

Scope: PAM, Clinical and Pharmocovigilance, request for supplementary information

Action: for adoption

2.13.2. Casgevy - Exagamglogene autotemcel - Orphan - EMA/PAM/0000268688

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus, PRAC rapporteur: Bianca Mulder

Scope: PAM, Pharmacovigilance

Action: for adoption

2.13.3. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMA/R/0000250212

BioMarin International Limited

Rapporteur: Violaine Closson Carella, PRAC rapporteur: Bianca Mulder

Scope: Renewal 1 year, opinion

Action: for adoption

2.13.4. Breyanzi - Lisocabtagene maraleucel – Orphan - EMA/PASS/0000269320

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Updated PASS protocol v3.0 for JCAR017-BCM-005, an imposed category 1 mandatory additional pharmacovigilance activity: Non-interventional, post-authorisation safety study (PASS) of patients treated with commercially available liso-cel (lisocabtagene maraleucel) for large B-cell lymphomas; changes consequential to EMEA/H/C/004731/II/0043/G: updates in the objectives wording (for readability), eligibility criteria, and background to add therapeutic context for follicular lymphoma patients. PRAC-only Article 107o of Directive 2001/83/EC procedure, no CAT step

Action: for information

2.14. Companion diagnostics - initial consultation

2.14.1. 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: List of questions

Action: For adoption

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

4.1. New requests – Appointment of CAT Coordinator

No items

4.2. Day 30 ATMP scientific recommendation

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

Treatment of Parkinson's disease

Scope: ATMP scientific recommendation

Action: for adoption

4.2.2. 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: ATMP scientific recommendation

Action: for adoption

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

Treatment of critical limb ischemia

Scope: ATMP scientific recommendation

Action: for adoption

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

No items

4.4. Finalisation of procedure

4.4.1. Replication defective SV40 vector encoding for human proinsulin

Treatment of type 1 diabetes

Scope: ATMP scientific recommendation. European Commission raised no comments

Action: for adoption

4.4.2. Anitocabtagene autoleucel

Treatment of patients with multiple myeloma

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

Action: for adoption

4.4.3. Genetically modified porcine heart

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

Scope: ATMP scientific recommendation. European Commission feedback.

Action: for adoption

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:	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

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	01-04.09.2025
- Appointment of CAT Peer Reviewers:	10-12.09.2025
- SAWP first reports:	22.09.2025
- CAT Peer Reviewer comments (NC & C):	26.09.2025
- CAT Peer Reviewer comments (Q):	01.10.2025
- Discussion at SAWP:	29.09-02.10.2025
- Discussion at CAT and feedback to SAWP:	08-10.10.2025

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

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

No items

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:	07-10.07.2025
SAWP recommendation:	04.09.2025
CAT recommendation:	12.09.2025
CHMP adoption of report and final recommendation:	18.09.2025

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

Action: for information

7.1.2. Vote by proxy

Action: for information

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

Scope: CAT Plenary 2026 dates

Action: for information

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

CAT: Martin Bronislaw Oleksiewicz

Scope: Preparation for the meeting

Action: for information

7.1.5. EMA Committee reform

CAT: Ilona Reischl

Scope: Feedback from the meeting with all Committee Chairs on the novel committee structures

Action: for information

7.2. Coordination with EMA Scientific Committees

7.2.1. Guideline on good pharmacovigilance practices (GVP) Module XVI Addendum I – Risk minimisation measures for medicinal products with embryo-fetal risks

Action: for information

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

7.3.1. Patients and Consumers Working Party (PCWP) and the Healthcare Professionals Working Party (HCPWP) new three-year mandate

Scope: Invitation from the Patients and Consumers Working Party (PCWP) and the Healthcare Professionals Working Party (HCPWP) to nominate new three-year candidates (June 2025-May 2028)

Action: for information

7.4. Cooperation with the EU regulatory network

7.4.1. RWE activities including projects on Duchenne Muscular Dystrophy (DMD)

Scope: To inform CAT about a project that has been launched to assess the fitness-for-purpose of real-world data (RWD) sources on Duchenne Muscular Dystrophy to support regulatory decision-making

Action: for information

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 26.06.2025

Action: for information

7.6. CAT work plan

7.6.1. CAT Workshop on Genome Editing

Scope: Update on the CAT Workshop (16.09.2025)

Action: for information

7.7. Planning and reporting

No items

7.8. Others

No items

8. Any other business

No items

Date of next CAT meeting:

12-14 August 2025

10-12 September 2025

9. Explanatory notes

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

Abbreviations in Committee CMD documents and in relation to EMA regulatory activities

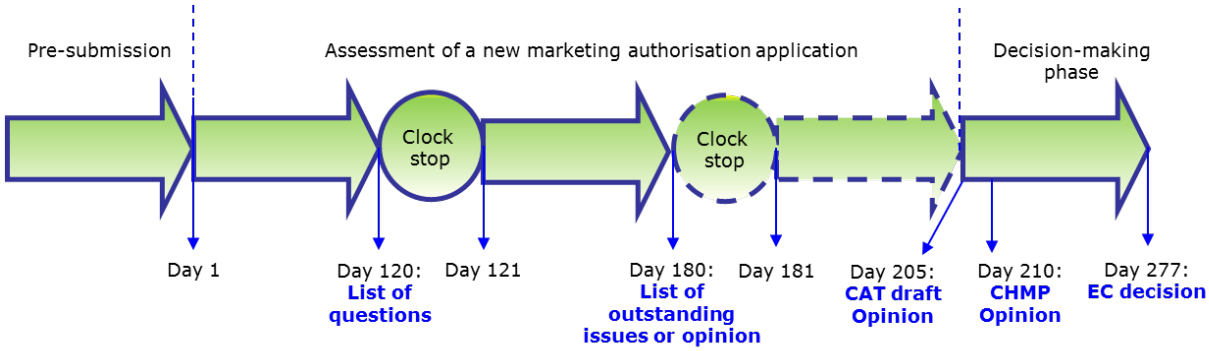
Evaluation of ATMPs (section 2)

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.

Companion diagnostics (section 2.10)

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

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.

GMP and GCP Inspections Issues (section 2.14.)

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

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/