10 September 2025
EMA/CAT/315756/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 12-14 August 2025 – *written procedure*

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

Disclaimers

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

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

Note on access to documents

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

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1. Introduction

1.1. Adoption of agenda

CAT agenda for 12-14 August 2025 meeting was adopted via a written procedure.

1.2. Adoption of the minutes

The CAT minutes for 16-18 July 2025 meeting will be adopted at the September CAT meeting.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

2.5.1. Onasemnogene abeparvovec - Orphan - EMEA/H/C/006498

Novartis Europharm Limited; Treatment of 5q spinal muscular atrophy (SMA)

Scope: Day 80 assessment report

Action: for information

The information was noted.

2.6. Update on ongoing initial applications

No item

2.7. New applications

No items

2.8. Withdrawal of initial marketing authorisation application

2.8.1. AMTAGVI - Lifileucel - EMEA/H/C/004741

Iovance Biotherapeutics B.V.; Treatment of unresectable or metastatic melanoma

Scope: Withdrawal of initial marketing authorisation application after CAT opinion

Action: for information

Negative draft CAT opinion on 18.07.2025. List of outstanding issues adopted on 16.05.2025. List of questions adopted on 06.12.2024.

The information was noted.

2.8.2. Fanskya - Mozafancogene autotemcel - PRIME - Orphan - EMEA/H/C/005537

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

Scope: Withdrawal of initial marketing authorisation application

Action: for information

List of outstanding issues adopted on 13.06.2025. List of questions adopted on 19.07.2024.

The information was noted.

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

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

TETEC Tissue Engineering Technologies AG; Repair of symptomatic, localised, full-thickness cartilage defects of the knee joint grade III or IV

Scope: Timetable for re-examination procedure

Action: for adoption

The re-examination timetable will be adopted at the September CAT meeting.

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

No items

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

No items

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:	14.08.2025
-EMA Coordinator's draft report:	25.08.2025
-CAT Coordinator's comments:	30.08.2025
-Revised scientific recommendation:	02.09.2025
-CAT's discussion of scientific recommendation:	05.09.2025

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Ex vivo genetic modification of lung grafts prior to transplantation in patients

Ex vivo genetic modification of lung grafts prior to transplantation in patients

Scope: nomination of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Lentiviral vector encoding for short hairpin RNA (shRNA) sequences down-regulating human leukocyte antigen (HLA) class I and HLA class II by targeting key messenger RNAs (mRNAs)

Treatment of deep or perforating corneal defects

Scope: nomination of CAT coordinator

Action: for adoption

The CAT coordinator was 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. Allogeneic human midbrain dopaminergic neuron (mDA) progenitor cells derived from human pluripotent stem cells

Treatment of Parkinson's disease

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

Action: for adoption

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

4.4.2. 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. European Commission raised no comments.

Action: for adoption

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

4.4.3. 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. European Commission raised no comments.

Action: for adoption

The classification report was adopted. The product does fulfil the definitions of a somatic cell therapy medicinal product, and a tissue engineered product and based on that is considered as Tissue Engineered Product as provided in Article 2(4) of Regulation (EC) No. 1394/2007. The product also fulfils the definition of a combined advanced therapy medicinal product as defined 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:	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

No items

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

No items

5.4. Final Advice Letters for procedures finalised the previous month

No items

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

No items

6.3.2. Month 1 – Discussion of eligibility

No items

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

7.2. 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

No items

7.5. Cooperation with international regulators

No items

7.6. CAT work plan

No items

7.7. Planning and reporting

No items

7.8. Others

No items

8. Any other business

No items

Date of next CAT meeting:

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

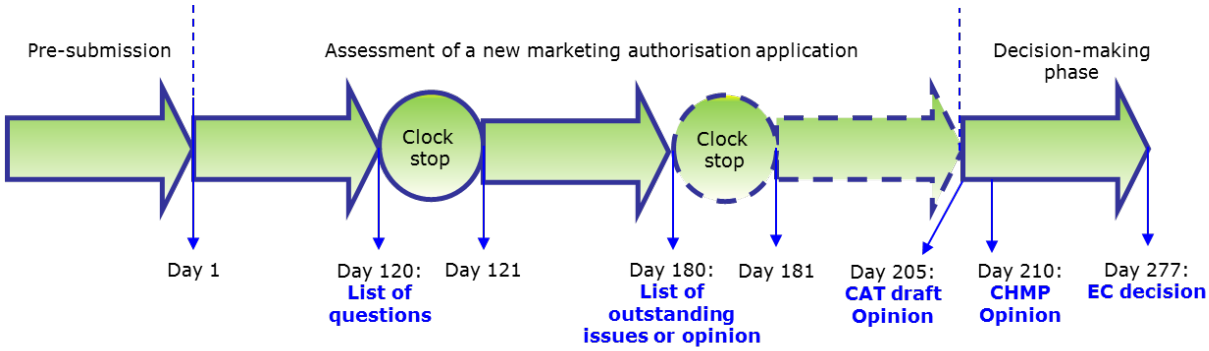
Evaluation of ATMPs (section 2)

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/