05 November 2025
EMA/CAT/352855/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 05-07 November 2025

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

05 November 2025, 14:00 – 18:00

06 November 2025, 09:00 – 18:00

07 November 2025, 09:00 – 13:00

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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

Table of contents

1. Introduction

1.1. Welcome and declarations of interest of members, alternates and experts

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 05-07 November 2025. See November 2025 CAT minutes (to be published post December 2025 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 05-07 November 2025 meeting

1.3. Adoption of the minutes

CAT minutes for 08-09 October 2025 meeting

2. Evaluation of ATMPs

2.1. Opinions

2.1.1. 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: Opinion

Action: for adoption

List of outstanding issues adopted on 12.09.2025. List of questions adopted on 16.04.2025.

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

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

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: Oral explanation, opinion

Action: for adoption

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 - EMA/VR/0000293201

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken, PRAC Rapporteur: Mari Thorn

Scope: Clinical

Update of sections 4.2, 4.4, and 4.7 of the SmPC in order to change the post-approval safety monitoring requirements after administration of Abecma; Annex II and the Package Leaflet are updated accordingly. The RMP version 5.0 has also been submitted.

Action: for adoption

2.11.2. Kymriah - Tisagenlecleucel - Orphan - EMA/VR/0000282599

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality, opinion

Action: for adoption

2.11.3. Kymriah - Tisagenlecleucel - Orphan - EMA/VR/0000290079

Novartis Europharm Limited

Rapporteur: Rune KjekenScope: Quality, opinion

Action: for adoption

2.11.4. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMA/VR/0000294531

Biomarin International Limited

Rapporteur: Violaine Closson Carella, PRAC Rapporteur: Bianca Mulder

Scope: Clinical, opinion

Submission of an updated RMP version 1.6 in order to remove the biodistribution study investigating vertical transmission of the AAV5 vector in female mice listed as category 3 study in RMP.

Action: for adoption

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

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Rune Kjeken, PRAC Rapporteur: Bianca Mulder

Scope: Clinical

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

Action: for adoption

Request for Supplementary Information adopted on 13.06.2025, 06.12.2024.

2.11.6. Yescarta, Tecartus - Axicabtagene ciloleucel, Brexucabtagene autoleucel - Orphan - EMA/VR/0000285857

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical, opinion

Update of section 4.4 of the SmPC in order to add a reference statement to current institutional / national guidelines for the monitoring and management of cytokine release syndrome (CRS), neurologic events and immune effector cell-associated neurotoxicity syndrome (ICANS). In addition, the MAH took the opportunity to introduce clarification and administrative updates to the PI, including Annex II.

Action: for adoption

Request for supplementary information adopted on 09.10.2025

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Breyanzi - Lisocabtagene maraleucel - Orphan - EMA/PAM/0000292361

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: PAM

Action: for adoption

2.13.2. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/PAM/0000291466

Janssen Cilag International

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

Scope: PAM

Action: for adoption

2.13.3. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMA/PAM/0000292813

Biomarin International Limited

Rapporteur: Violaine Closson Carella; PRAC Rapporteur: Bianca Mulder

Scope: PAM

Action: for adoption

2.13.4. Hemgenix - Etranacogene dezaparvovec - Orphan - EMA/R/0000288354

CSL Behring GmbH

Rapporteur: Silke Dorner, PRAC Rapporteur: Bianca Mulder

Scope: Renewal - 1 year

Action: for adoption

Request for supplementary information adopted on 9.10.2025

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

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. Attenuated Salmonella typhi strain Ty21a carrying plasmid pNECVAX-NEO1

Treatment of solid malignancies with or without metastases

Scope: ATMP scientific recommendation

Action: for adoption

4.2.2. iPSC-derived Retinal Pigment Epithelium (RPE) cells on a synthetic polymer membrane

Restoring vision in advanced (late-stage) retinitis pigmentosa (RP)

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. 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)

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

Scope: ATMP scientific recommendation.

Action: for adoption

4.4.2. CD4+CD25+CD127-MOG-CAR+ T regulatory cells

Treatment and prevention of progression of Myelin Oligodendrocyte Glycoprotein Antibody-Associated Disease, Amyotrophic Lateral Sclerosis (ALS), Primary Progressive Multiple Sclerosis.

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

Action: for adoption

4.4.3. Non-replicating recombinant adeno-associated viral vector serotype hu68 (AAVhu68), containing a codon-optimized human survival motor neuron 1 (SMN1) gene

Treatment of Spinal Muscular Atrophy (SMA)

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

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:	27-30.10.2025
- Appointment of CAT Peer Reviewers:	05-07.11.2025
- SAWP first reports:	17.11.2025
- CAT Peer Reviewer comments (NC & C):	21.11.2025
- CAT Peer Reviewer comments (Q):	26.11.2025
- Discussion at SAWP:	24-27.11.2025
- Discussion at CAT and feedback to SAWP:	03-05.12.2025

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	24-27.11.2025
- Appointment of CAT Peer Reviewers:	03-05.12.2025
- SAWP first reports:	05.01.2026
- CAT Peer Reviewer comments (NC & C):	09.01.2026
- CAT Peer Reviewer comments (Q):	14.01.2026
- Discussion at SAWP:	12-15.01.2026
- Discussion at CAT and feedback to SAWP:	21-23.01.2026

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 item

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:	
Procedure start:	27-30.10.2025
SAWP recommendation:	27.11.2025
CAT recommendation:	05-12.2025
CHMP adoption of report and final recommendation:	11.12.2025

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

Scope: Feedback from the meeting

CAT: Martin Bronislaw Oleksiewicz

Action: for information

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

Scope: Preparation for the meeting

CAT: Rafaella Pontou

Action: for information

7.2. Coordination with EMA Scientific Committees

7.2.1. SAWP composition – re-examination exercise

Scope: SAWP secretariat would like to present details on the requirements, procedural aspects, application forms and timelines of the SAWP composition - re-examination exercise

Action: for information

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

No items

7.4. Cooperation with the EU regulatory network

7.4.1. EU Life Science Strategy and Biotech Act

European Commission:

Scope: Update to the Committee

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 ATMP cluster of 02.10.2025

Action: for information

7.6. CAT work plan

7.6.1. CAT work plan 2026

Scope: Work plan topics for 2026

CAT: Ilona Reischl

Action: for discussion

7.7. Planning and reporting

No items

7.8. Others

7.8.1. WHO Consultation on Regulatory Aspects of Xenotransplantation

Scope: Feedback from the WHO meeting that took place on 29.09.2025

CAT: Ilona Reischl

Action: for information

7.8.2. WHO Implementation Workshop: WHO Considerations in developing a regulatory framework for human cells and tissues and for advanced therapy medicinal products

Scope: Feedback from the WHO meeting that took place on 24-26.09.2025 in Brazzaville, Congo

CAT: Ilona Reischl

Action: for information

7.8.3. Guideline on predictive biomarker assay development in the context of medicinal product lifecycle

CAT Resources: Jörg Engelbergs, Olga Kholmanskikh, Ilona Reischl

Scope: The guideline on predictive biomarker assay development covers the topic of companion diagnostics, highly relevant to ATMPS (e.g. co-development scenario). We would like written feedback on the guideline that is now undergoing internal consultation.

Action: for information

7.8.4. ATMP Support Pilot for academic developers of ATMPs

Scope: Report and learnings from the Academic support pilot

Action: for information

8. Any other business

No items

Date of next CAT meeting:

5-7 December 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

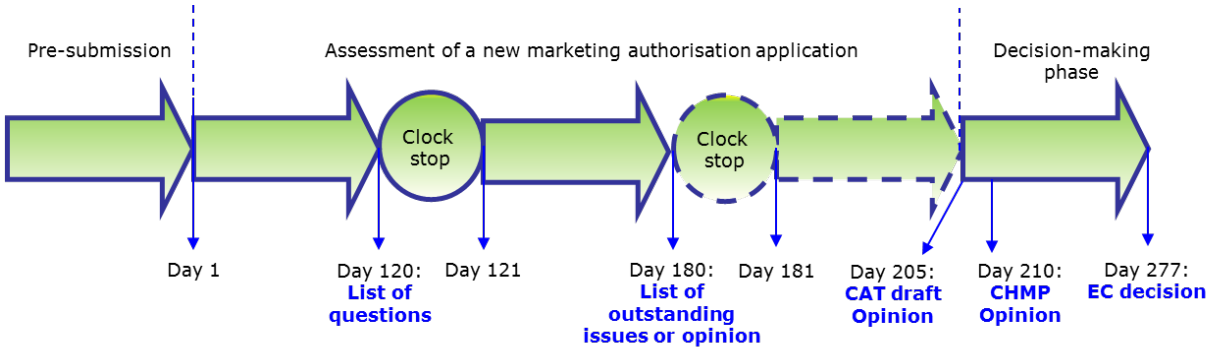
Evaluation of ATMPs (section 2)

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/