14 May 2025
EMA/CAT/163785/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 14-16 May 2025

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

14 May 2025, 14:00 – 18:30, room 1C

15 May 2025, 09:00 – 18:30, room 1C

16 May 2025, 09:00 – 13:00, room 1C

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 14-16 May 2025. See May 2025 CAT minutes (to be published post June 2025 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 14-16 May 2025 meeting

1.3. Adoption of the minutes

CAT minutes for 14-16 April 2025 meeting

2. Evaluation of ATMPs

2.1. Opinions

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

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

Scope: Opinion

Action: for adoption

List of outstanding issues adopted on 21.03.2025. List of questions adopted on 19.07.2024.

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

2.3.1. Lifileucel - EMEA/H/C/004741

Treatment of unresectable or metastatic melanoma

Scope: Day 180 list of outstanding issues

Action: for adoption

List of questions adopted on 06.12.2024.

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

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical, opinion

Submission of the final report from study KT-US-482-0147 (ZUMA-26). This is a Prospective, Noninterventional, Clinical Efficacy Study Investigating and Analysing the Impact of Tumour Cd19 Antigen Expression and Density on Response to Axicabtagene Ciloleucel Treatment Using a Quantitative Flow Cytometry Method.

Action: for adoption

Request for supplementary information adopted on 21.02.2025.

2.11.2. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2771

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Karin Erneholm

Scope: Safety, PRAC led, request for supplementary information

Submission of an updated RMP version 4.3 for Tecartus and version 11.1 for Yescarta following the PRAC recommendation for the secondary malignancy of T-cell origin signal (EPITT no: 20040), and of a PASS protocol for a framework for the sampling and testing of secondary malignancies of T-cell origin.

Action: for adoption

Request for supplementary information adopted on 24.01.2025.

2.11.3. Kymriah - Tisagenlecleucel - Orphan - EMA/VR/0000258240

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality, opinion

Action: for adoption

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

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical, request for supplementary information

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

2.11.5. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/VR/0000258113

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

2.11.6. Tecartus - Brexucabtagene autoleucel - Orphan - EMA/VR/0000257524

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical, opinion

Submission of the 57-month follow-up results from study ZUMA-3 (KTE-C19-103) listed as a Specific Obligation in the Annex II of the Product Information. This is a phase 1/2 open-label study evaluating the safety and efficacy of brexucabtagene autoleucel in adult participants with relapsed/refractory B-precursor acute lymphoblastic leukaemia (r/r ALL). The Annex II is updated accordingly.

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/0000245074

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, opinion

Action: for adoption

2.11.9. Hemgenix - etranacogene dezaparvovec - Orphan - EMA/VR/0000258413

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, opinion

Action: for adoption

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Ebvallo - Tabelecleucel - Orphan - EMA/PAM/0000258195

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: PAM, opinion

Action: for adoption

2.13.2. Ebvallo - Tabelecleucel - Orphan - EMA/S/0000249324

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Annual reassessment, request for supplementary information

Action: for adoption

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

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Renewal, request for supplementary information

Action: for adoption

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:	19.05.2025
-EMA Coordinator's draft report:	27.05.2025
-CAT Coordinator's comments:	28.05.2025
-Revised scientific recommendation:	04.06.2025
-CAT's discussion of scientific recommendation:	06.06.2025

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Replication defective SV40 vector encoding for human proinsulin

Treatment of type 1 diabetes

Scope: Nomination of CAT coordinator

Action: for adoption

4.1.2. Anitocabtagene autoleucel

Treatment of patients with multiple myeloma

Scope: Nomination of CAT coordinator

Action: for adoption

4.2. Day 30 ATMP scientific recommendation

4.2.1. Human oocyte cytoplasma

For use in case of low human egg quality in *in vitro* fertilisation (IVF)

Scope: ATMP scientific recommendation

Action: for adoption

4.2.2. Selected autologous Tumor-Infiltrating Lymphocytes (TILs) isolated from tumor tissue, lymph node metastases, or peripheral blood, expanded, activated

Treatment of advanced or metastatic solid tumours

Scope: ATMP scientific recommendation

Action: for adoption

4.2.3. Autologous T cells ex vivo transduced with LVV encoding a CAR that recognises BCMA

Treatment of relapsed or refractory form of plasmocytic myeloma (RRMM)

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

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

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

Treatment of primary photoreceptor disease

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

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

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

Treatment of cystic fibrosis

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

Treatment of autoimmune diseases

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

4.4.6. Autologous tumour-derived dendritic cells

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

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

4.4.7. Genetically modified porcine heart

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

Scope: European Commission raised comments. ATMP scientific recommendation

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:	05-08.05.2025
- Appointment of CAT Peer Reviewers:	14-16.05.2025
- SAWP first reports:	26.05.2025
- CAT Peer Reviewer comments (NC & C):	30.05.2025
- CAT Peer Reviewer comments (Q):	04.06.2025
- Discussion at SAWP:	02-05.06.2025
- Discussion at CAT and feedback to SAWP:	11-13.06.2025

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	02-05.06.2025

- Appointment of CAT Peer Reviewers:	11-13.06.2025
- SAWP first reports:	30.06.2025
- CAT Peer Reviewer comments (NC & C):	04.07.2025
- CAT Peer Reviewer comments (Q):	09.07.2025
- Discussion at SAWP:	07-10.07.2025
- Discussion at CAT and feedback to SAWP:	16-18.07.2025

No items

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

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

5.4. Final Advice Letters for procedures finalised the previous month

6. Pre-Authorisation Activities

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6.1. Paediatric investigation plans

No items

6.2. ITF briefing meetings in the field of ATMPs

No items

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:	
Procedure start:	05-08.05.2025
SAWP recommendation:	05.06.2025
CAT recommendation:	13.06.2025
CHMP adoption of report and final recommendation:	19.06.2025

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

No items

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

CAT: Martin Bronislaw Oleksiewicz

Scope: Preparation for the meeting

Action: for information

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

CAT: Dariusz Sladowski

Scope: Oral feedback from the meeting

Action: for information

7.2. Coordination with EMA Scientific Committees

No items

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

7.3.1. BWP/CAT Learning on Claims for New Active Substance (NAS) status for ATMPs

Rapporteur/Coordinator: Sean Barry, Ilona Reischl

Scope: BWP/CAT learning on assessment of NAS claims for ATMPs

Action: for discussion

7.4. Cooperation with the EU regulatory network

No items

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: Agenda topics for the teleconference of 26.06.2025

Action: for information

7.6. CAT work plan

No items

7.7. Planning and reporting

No items

7.8. Others

7.8.1. Richard Slayman Clinical Xenotransplant Workshop 31.05.2025 – 01.06.2025

Scope: Nomination of EMA/CAT representative to the workshop

Action: for action

7.8.2. EMA survey: exploring the role of Patient Preference Studies in the Benefit/Risk assessment

Scope: Presentation to the committee

Action: for discussion

7.8.3. American Society for gene and cell therapy (ASGCT) - Fireside chat: European Regulation and the future of Advanced Therapies

CAT: Ilona Reischl

Scope: Feedback from the fireside chat (14.05.2025)

Action: for information

8. Any other business

No items

Date of next CAT meeting:

9. Explanatory notes

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

Abbreviations / Acronyms

AAV: Adeno-Associated Virus

AR: Assessment Report

ATMP: Advanced Therapy Medicinal Product

BWP: Biologics Working Party

CAT: Committee for Advanced Therapies

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

CTFG: Clinical Trial Facilitation Group

DG: Drafting Group

EC: European Commission

EU NTC: European Union Network Training Centre

ERA: Environmental Risk Assessment

FDA: Food and Drug Administration

FL: Final Letter

GCG: Guideline Consistency Group

GCP: Good Clinical Practice

GLP: Good Laboratory Practice

GMO: Genetically-modified organism

GMP: Good Manufacturing Practice

GTMP: Gene Therapy Medicinal Product

HTA: Health Technology Assessment Bodies

HSPC: Hematopoietic Stem and Progenitor Cells

ITF: Innovative Task Force

JR: Joint Report

LoOI: List of outstanding issues

LoQ: List of questions

MA: Marketing Authorisation

MAA: Marketing Authorisation Application

MAH: Marketing Authorisation Holder

MNAT: Multinational assessment team

MSC: Mesenchymal stem cells

PDCO: Paediatric Committee

PMDA: Pharmaceuticals and Medical Devices Agency (Japan)

PIP: Paediatric Investigation Plan

PL: Package leaflet

PRAC: Pharmacovigilance and Risk Assessment Committee #

PRIME: Priority Medicines

QRD: Quality review of documents

RMP: Risk Management Plan

RP: Reflection paper

RSI: Request for supplementary information

SAs: Scientific Advices

SAG-O: Scientific Advisory Group Oncology

SAWP: Scientific Advice Working Party

SR: Summary Report

SME: Small and medium size enterprises

SmPC: Summary of Products Characteristics

TT: Timetable

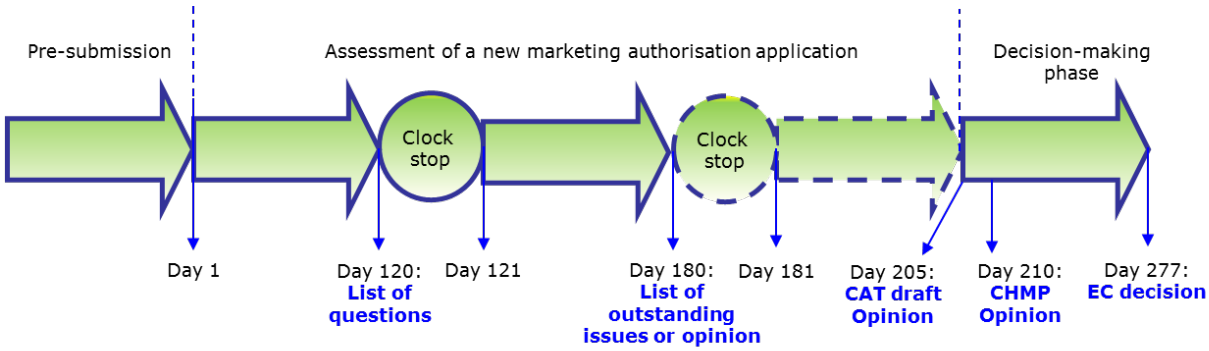
Evaluation of ATMPs (section 2)

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/