22 May 2024
EMA/CAT/180860/2024
Human Medicines Division

Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 22-24 May 2024

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

22 May 2024, 14:00 – 18:30

23 May 2024, 09:00 – 18:30

24 May 2024, 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 22-24 May 2024. See May 2024 CAT minutes (to be published post June 2024 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 22-24 May 2024 meeting

1.3. Adoption of the minutes

CAT minutes for 17-20 April 2024 meeting

2. Evaluation of ATMPs

2.1. Opinions

2.1.1. Fidanacogene elaparvovec - PRIME - EMEA/H/C/004774

Indicated for the treatment of severe and moderately severe haemophilia B

Scope: Opinion; third party intervention

Action: for adoption

List of outstanding issues adopted on 15.03.2024, list of questions adopted on 08.09.2023.

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

No items

2.10. Companion diagnostics

2.10.1. Initial consultation

No items

2.10.2. Follow-up consultation

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/0047

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, request for supplementary information

Action: for adoption

2.11.2. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/II/0036/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Safety, request for supplementary information

Grouped application comprising two variations as follows:
C.I.4 – Update of sections 4.4 and 4.8 of the SmPC in order to add immune effector cell-associated neurotoxicity syndrome (ICANS) as an adverse drug reaction (ADR) based on the cumulative review of MAH safety database and literature. The Package Leaflet is updated accordingly. In addition, the MAH took this opportunity to introduce editorial changes.
A.6 – To include the ATC Code L01XL08 in section 5.1 of the SmPC.

Action: for adoption

Request for supplementary information adopted on 16.02.2024.

2.11.3. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0027/G

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

2.11.4. Libmeldy - Atidarsagene autotemcel - Orphan - EMEA/H/C/005321/II/0025

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries

Scope: Quality, opinion

Action: for adoption

2.11.5. Upstaza - Eladocagene exuparvovec - Orphan - EMEA/H/C/005352/II/0021

PTC Therapeutics International Limited

Rapporteur: Joseph DeCourcey

Scope: Quality, request for supplementary information

Action: for adoption

2.11.6. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2500

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: 2nd Request for supplementary information for adoption

Request for supplementary information adopted on 16.02.2024.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/REC/021

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, opinion

Action: for adoption

2.13.2. Ebvallo - Tabelecleucel - Orphan - EMEA/H/C/004577/S/0008

Pierre Fabre Medicament

Rapporteur: Egbert Flory, PRAC Rapporteur: Amelia Cupelli

Scope: Annual re-assessment, opinion

Action: for adoption

2.13.3. Luxturna - Voretigene neparvovec - Orphan - EMEA/H/C/004451/REC/007.1

Novartis Europharm Limited

Rapporteur: Sol Ruiz

Scope: Quality, opinion

Action: for adoption

2.13.4. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/R/0011

BioMarin International Limited

Rapporteur: Violaine Closson Carella, Co-Rapporteur: Silke Dorner, PRAC Rapporteur: Bianca Mulder

Scope: 1 year renewal of marketing authorisation, request for supplementary information

Action: for adoption

2.13.5. Yescarta – axicabtagene ciloleucel – Orphan – EMA/H/C/004480/PSA/S/0102.4

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Protocol update for PASS study KT-EU-471-0117

Action: for information

Note: PASS study protocol updated following the CAT request to consider additional data sources for the long-term safety and efficacy follow-up study based on EBMT data.

2.14. GMP and GCP inspections requests

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:	24.05.2024
-EMA Coordinator's draft report:	07.06.2024
-CAT Coordinator's comments:	12.06.2024
-Revised scientific recommendation:	14.06.2024
-CAT's discussion of scientific recommendation:	21.06.2024

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Autologous antigen specific Cytotoxic T Lymphocytes

For treatment of cancer patients

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.2. Autologous dendritic cells against tumour peptides

For treatment of cancer patients

EMA: Jaime Oliva Penas

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.3. Autologous macrophages

For treatment of cancer patients

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.4. Autologous cytotoxic natural killer (NK) cells

For treatment of cancer patients

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.5. Autologous plasma cells producing antibodies against tumour antigen

For treatment of cancer patients

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.6. Autologous adipose-derived stromal vascular fraction cells

For chronic pain relief

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.7. Double stranded DNA targetting patient specific tumour neo-antigens

For treatment of non small cell lung cancer

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.8. Synthetic double-stranded RNA oligonucleotide conjugated to GalNAc aminosugar residues

For treatment of primary hyperoxaluria

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.2. Day 30 ATMP scientific recommendation

4.2.1. MicroRNA against BCL2 anti-apoptotic messenger RNA

For treatment of cancer patients

Scope: ATMP scientific recommendation

Action: for adoption

4.2.2. Allogeneic natural killer cells expanded in vitro and transfected to express modified Fas ligand

For treatment of haematological malignancies and glioblastoma

Scope: ATMP scientific recommendation

Action: for adoption

4.2.3. Stromal vascular fraction

For treatment of osteoarthritis

Scope: ATMP scientific recommendation

Action: for adoption

4.2.4. Implantable device 3D bioprinted with autologous microfat and hydrogel bioink

For treatment of breast reconstruction, soft tissue repair

Scope: ATMP scientific recommendation

Action: for adoption

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

4.3.1. Lymphocyte concentrate

For improvement of the pregnancy outcomes among women with unexplained repeated pregnancy loss and HLA sharing among partners

Scope: ATMP scientific recommendation

Action: for adoption

4.4. Finalisation of procedure

4.4.1. mRNA encoding ARCUS nuclease

For treatment of chronic hepatitis B (CHB) virus infection

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

4.4.2. Allogeneic human corneal endothelial cells (neltependocel) and a low molecular weight Rho kinase inhibitor (Y-27632)

For treatment of corneal oedema due to corneal endothelial dysfunction

Scope: European Commission raised no 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:	13-16.05.2024
- Appointment of CAT Peer Reviewers:	22-24.05.2024
- SAWP first reports:	03.06.2024
- CAT Peer Reviewer comments (NC/C):	07.06.2024
- CAT Peer Reviewer comments (Q):	12.06.2024
- Discussion at SAWP:	10-12.06.2024
- Discussion at CAT and feedback to SAWP:	19-21.06.2024

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	10-13.06.2024
- Appointment of CAT Peer Reviewers:	19-21.06.2024
- SAWP first reports:	01.07.2024
- CAT Peer Reviewer comments (NC/C):	05.07.2024
- CAT Peer Reviewer comments (Q):	10.07.2024
- Discussion at SAWP:	08-11.07.2024
- Discussion at CAT and feedback to SAWP:	17-19.07.2024

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:	13-16.05.2024
SAWP recommendation:	13.06.2024
CAT recommendation:	21.06.2024
CHMP adoption of report and final recommendation:	27.06.2024

No items

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 Belgian presidency – 15-17 May 2024

CAT: Claire Beuneu

Scope: Feedback from the SRLM

Action: for discussion

7.2. Coordination with EMA Scientific Committees

7.2.1. Schedule of F2F meetings for 2025

Scope: Proposed schedule of F2F meetings for 2025 was presented at the SciCoBo meeting on 6 May 2024

Action: for information

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

7.3.1. SAWP/CTCG SA pilot

Scope: Feedback from the Consolidated advice pilots: ACT EU Priority Action 7

Action: for information

7.4. Cooperation with the EU regulatory network

No items

7.5. Cooperation with international regulators

7.5.1. WHO Conference in Muskat Oman, May 14-16th

CAT: Ilona Reischl

Scope: Feedback from the WHO conference

Action: for information

7.6. CAT work plan

7.6.1. Workplan activity '1.2.3 - Interactions with Health Technology Assessment (HTA) bodies to optimise clinical evidence generation'

CAT: Ilona Reischl

Scope: Workshops on uncertainties

Action: for information

7.7. Others

No items

8. Any other business

8.1. Webinar on the "Regulation of Faecal Microbiota Transplants (FMT) and FMT-derived medicinal products in the EU"

Action: for information

Date of next CAT meeting:

17-19 June 2024

9. 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

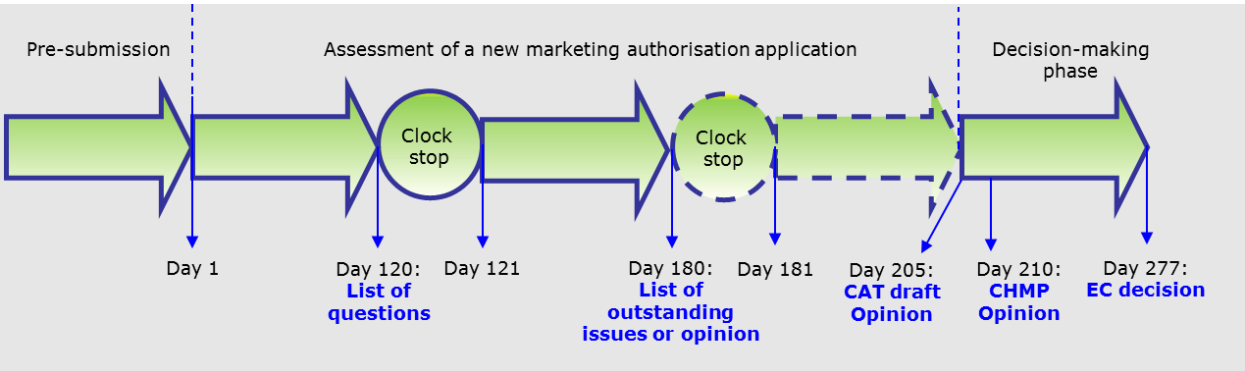
Evaluation of ATMPs (section 2)

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.

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/