11 September 2024
EMA/CAT/400955/2024
Human Medicines Division

Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 11-12 September 2024

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

11 September 2024, 14:00 – 18:30,

12 September 2024, 09:00 – 18:30,

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 11-13 September 2024. See 11-13 September 2024 CAT minutes (to be published post October 2024 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 11-13 September 2024 meeting

1.3. Adoption of the minutes

CAT minutes for 17-20 July 2024 meeting

CAT minutes for 14-16 August 2024 written procedure

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. 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: Day 80 assessment report

Action: for information

2.5.2. Dorocubicel / Allogeneic umbilical cord-derived CD34- cells, non-expanded - PRIME - Orphan - EMEA/H/C/005772

Accelerated assessment

Cordex Biologics International Limited; Treatment of adult patients with haematological malignancies

Scope: Day 80 assessment report

Action: for information

2.6. Update on ongoing initial applications

2.6.1. Mozafancogene autotemcel – PRIME – Orphan - EMEA/H/C/005537

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

Scope: Request for clock stop extension

Action: for information

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. 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, opinion

Action: for adoption

Request for Supplementary Information adopted on 19.07.2024, 24.05.2024.

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

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

Request for Supplementary Information adopted on 24.05.2024.

2.11.3. Casgevy - Exagamglogene autotemcel - Orphan - EMEA/H/C/005763/II/0003/G

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

Request for Supplementary Information adopted on 21.06.2024.

2.11.4. Ebvallo - Tabelecleucel - Orphan - EMEA/H/C/004577/II/0011/G

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, request for additional information

Action: for adoption

2.11.5. Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/II/0014/G

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, opinion

Action: for adoption

Request for Supplementary Information adopted on 19.07.2024.

2.11.6. Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/II/0015

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Clinical

Submission of the final report from study AMT-061-01/CSL222_2001 listed as a Specific Obligation in the Annex II of the Product Information. This is a Phase IIb, open-label, single-dose, single-arm, multi-center trial to confirm the factor IX activity level of the serotype 5 adeno-associated viral vector containing the Padua variant of a codon-optimized human factor IX gene (AAV5-hFIXco-Padua, AMT-061) administered to adult subjects with severe or moderately severe haemophilia B. The Annex II is updated accordingly.

Action: for adoption

2.11.7. Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/II/0016/G

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, opinion

Action: for adoption

2.11.8. Libmeldy - Atidarsagene autotemcel - Orphan - EMEA/H/C/005321/II/0027

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries

Scope: Quality, opinion

Action: for adoption

2.11.9. Libmeldy - Atidarsagene autotemcel - Orphan - EMEA/H/C/005321/II/0029

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries

Scope: Quality, opinion

Action: for adoption

2.11.10. Luxturna - Voretigene neparvovec - Orphan - EMEA/H/C/004451/II/0050/G

Novartis Europharm Limited

Rapporteur: Sol Ruiz

Scope: Quality, opinion

Action: for adoption

2.11.11. Strimvelis - Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence - Orphan - EMEA/H/C/003854/II/0040

Fondazione Telethon ETS

Rapporteur: Sol Ruiz, PRAC Rapporteur: Liana Martirosyan

Scope: Safety, request for supplementary information

Submission of an updated RMP version 7.0 in order to propose amendments to the STRIM-005 and STRIM-003 study protocols, as well as revised timelines for completion of both studies. In addition, the Annex II is updated accordingly.

Action: for adoption

2.11.12. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2689

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Beghaus

Scope: Quality, opinion

Action: for adoption

Request for Supplementary Information adopted on 21.06.2024.

2.11.13. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2736

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/REC/017

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

2.13.2. Strimvelis - Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence - Orphan - EMEA/H/C/003854/ANX/004.5

Fondazione Telethon ETS

Rapporteur: Sol Ruiz

Scope: Clinical & Pharmacovigilance, Opinion

MAH Response to ANX 004.4 [Patient Registry / STRIM-0003] as adopted in June 2023: Based on the PRAC Rapporteur review of the PASS interim study report, dated 21 March 2023, the PRAC considers that the risk-benefit balance of medicinal products containing the active substance Strimvelis concerned by the PASS interim report is subject to a request for supplementary information detailed in Section 12 in the Annex, before a recommendation can be made. The responses timetable to the Request for Supplementary Information will be 60 days.

Action: for adoption

2.13.3. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/ANX/002.4

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical & Pharmacovigilance

First Annual Interim Safety Report of Study KTE-EU-472-6036
Title: Long-term, non-interventional study of recipients of Tecartus (brexucabtagene autoleucel) for treatment of adult patients with relapsed or refractory Mantle Cell Lymphoma (MCL).

Action: for adoption

2.13.4. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/R/0047

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Annual renewal, Request for Supplementary Information

Action: for adoption

2.13.5. Ebvallo - Tabelecleucel – EMEA/H/C/004577/PSP-S-0115

Pierre Fabre Medicament

CAT Rapporteur: Egbert Flory, PRAC Rapporteur: Gabriele Maurer

Scope: Protocol update assessment for the post-marketing surveillance study, Request for supplementary information

Action: for information (PRAC led procedure)

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

4. Scientific Recommendation on Classification of ATMPs

Timetable:

-Start of the procedure:	13.09.2024
-EMA Coordinator's draft report:	27.09.2024
-CAT Coordinator's comments:	02.10.2024
-Revised scientific recommendation:	04.10.2024
-CAT's discussion of scientific recommendation:	11.10.2024

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Human allogeneic cardiosphere-derived cells

Treatment of muscular dystrophy

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.2. Allogenic fibroblasts embedded in a scaffold of hyaluronic acid and fibrinogen

Treatment of chronic and refractory ulcers

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.2. Day 30 ATMP scientific recommendation

4.2.1. Autologous cells mainly composed of CD45+CD3+ T cells and to minor extent of other cells like B cells and NK cells derived from the regional lymph node cells and enriched for neoantigen specific T cells

Treatment of cancers in adults

Scope: ATMP scientific recommendation

Action: for adoption

4.2.2. In vitro transcribed mRNA encoding the peptide VMAPRTLFL, a ligand for the activating immune receptor CD94/NKG2C

Treatment and / or prevention of leukaemia relapse, e.g. after haematopoietic stem cell transplant

Scope: ATMP scientific recommendation

Action: for adoption

4.2.3. Recombinant adeno-associated virus vector containing an expression cassette of Padua factor IX transgene

Treatment of hemophilia B

Scope: ATMP scientific recommendation

Action: for adoption

4.2.4. hiPSC derived Ovarian Support Cells (OSCs)

For ex vivo maturation of human oocytes

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. Spermatogonial stem cells, propagated in vitro

Male infertility due to gonadotoxic treatment

Scope: ATMP scientific recommendation

Action: for information

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:	02-05.09.2024
- Appointment of CAT Peer Reviewers:	11-13.09.2024
- SAWP first reports:	23.09.2024
- CAT Peer Reviewer comments (NC/C):	27.09.2024
- CAT Peer Reviewer comments (Q):	02.10.2024
- Discussion at SAWP:	30.09-03.11.2024
- Discussion at CAT and feedback to SAWP:	09-11.09.2024

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	30.09-03.10.2024
- Appointment of CAT Peer Reviewers:	09.-11.10.2024
- SAWP first reports:	21.10.2024
- CAT Peer Reviewer comments (NC/C):	25.10.2024
- CAT Peer Reviewer comments (Q):	30.10.2024
- Discussion at SAWP:	28-31.11.2024
- Discussion at CAT and feedback to SAWP:	06-08.11.2024

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

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

Timetable for assessment:

Procedure start:	02-05.09.2024
SAWP recommendation:	03.10.2024
CAT recommendation:	11.10.2024
CHMP adoption of report and final recommendation:	17.10.2024

No items

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. CAT Strategic Review & Learning meeting (SRLM) under the Hungarian presidency – 19-20 November 2024

CAT: Andras Donaszi-Ivanov, Viola Bardóczy

Scope: Draft agenda of the upcoming SRLM

Action: for discussion

7.1.3. Committee meetings in TEAMS and new tool for voting

Scope: Introduction of new online TEAMS meeting and a new voting system within TEAMS

Action: for information

7.2. Coordination with EMA Scientific Committees

7.2.1. Patients and Consumers Working Party (PCWP) and Healthcare Professionals Working Party (HCPWP)

Scope: Agenda and Minutes

Summary report of the PCWP and HCPWP joint meeting held face-to-face on 27-28 February 2024

Final agenda of the PCWP and HCPWP joint meeting held face-to-face on 02-03 July 2024

Summary report of the PCWP and HCPWP joint meeting held face-to-face on 02-03 July 2024

Action: for information

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

7.5.1. ATMP cluster teleconference with US-FDA, Health Canada and PMDA (Japan)

CAT: Ilona Reischl

Scope: Agenda of the teleconference of 19.09.2024

Action: for information

7.5.2. EU-IN -AIFA workshop: Translating Innovation to access - 3rd EU-Innovation Network multi-stakeholder meeting – 14.11.2024, Rome, Italy

CAT: Petr Soukup, Ilona Reischl

Scope: EU Innovation Network together with AIFA is organising a multi-stakeholder meeting to discuss how to translate innovation into access of effective and safe therapies in Europe. The focus of the meeting will be on ATMPs. EU-IN would like to gain feedback from CAT

Action: for information

7.6. CAT work plan

7.6.1. CAT symposium – 10.10.2024, Amsterdam, the Netherlands

Scope: Programme and practical information

Action: for information

7.7. Planning and reporting

No items

7.8. Others

7.8.1. ISCT Europe

CAT: Ilona Reischl

Scope: Feedback from the presentations and discussion at the ISCT Europe 2024 Regional meeting (Gothenburg, Sweden; 04-06.09.2024)

Action: for information

8. Any other business

No items

Date of next CAT meeting:

9-11 October 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

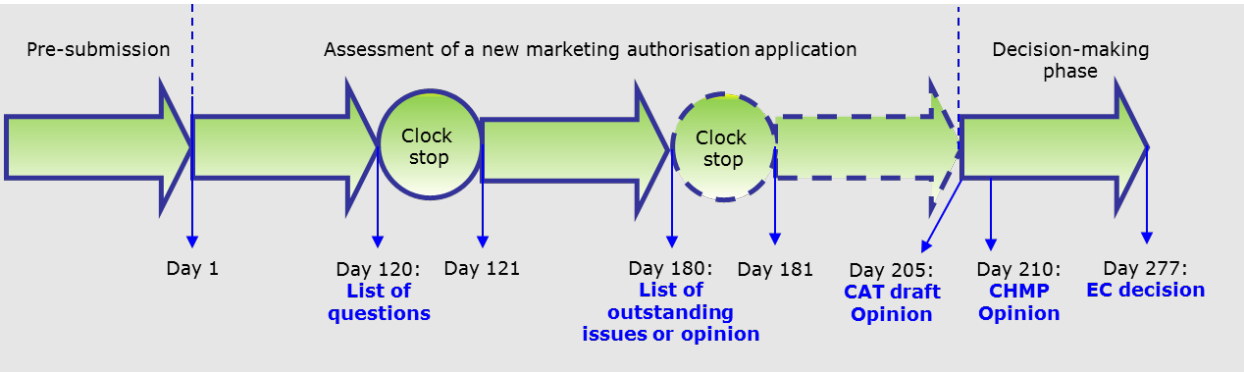
Evaluation of ATMPs (section 2)

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/