17 July 2024
EMA/CAT/490675/2024
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 19-21 June 2024

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

Disclaimers

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

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

Note on access to documents

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

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1. Introduction

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

The Chairperson opened the meeting by welcoming all participants. The meeting was held in-person.

In accordance with the Agency's policy on handling of declarations of interests of scientific committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and on the topics in the agenda of the current meeting, the Committee Secretariat announced the restricted involvement of some Committee members, alternates and experts for concerned agenda topics.

Participants were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions were declared. Restrictions applicable to this meeting are captured in the List of participants included in the minutes.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The EMA Secretariat announced the names of the Committee members who delegated their vote via proxy and the Committee members who received such proxy.

The Chair announced the start of the Hungarian presidency of the Council of the European Union (EU).

1.2. Adoption of agenda

The CAT agenda for 19-21 June 2024 meeting was adopted

1.3. Adoption of the minutes

The CAT minutes for 22-25 May 2024 meeting were adopted with one additional agenda point: 7.1.4: Strategic Review and Learning meeting under the Hungarian presidency of the Council of the European Union.

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. 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: Day 80 assessment report

Action: for information

The information was noted.

2.5.2. Mozafancogene autotemcel - PRIME - Orphan - EMEA/H/C/005537

Rocket Pharmaceuticals B.V.; Treatment of paediatric patients with Fanconi Anaemia Type A

Scope: Day 80 assessment report

Action: for information

The information was noted.

2.6. Update on ongoing initial applications

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

Repair of symptomatic, localised, full-thickness cartilage defects of the knee joint grade III or IV

Scope: Request for extension of clock stop

Action: for discussion

CAT discussed the request for clock stop extension. CAT agreed on a 3 months clock extension (6 months in total).

2.7. New applications

2.7.1. 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: Timetable for assessment

Action: for adoption

The timetable for assessment was adopted.

2.7.2. Delandistrogene moxeparovec – Orphan – EMEA/H/C/005293Roche

Registration GmbH; Treatment of ambulatory patients aged 3 to 7 years old with Duchenne muscular dystrophy

Scope: Timetable for assessment

Action: for adoption

The timetable for assessment was adopted.

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/0048

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

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

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Safety

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 24.05.2024, 16.02.2024.

All pending issues are resolved. The opinion was adopted.

2.11.3. Breyanzi - Lisocabtagene maraleucel - EMEA/H/C/004731/II/0037/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, request for supplementary information

Action: for adoption

Request for supplementary information adopted on 15.03.2024.

The second request for supplementary information was adopted.

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

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.5. Imlygic - Talimogene laherparepvec - EMEA/H/C/002771/II/0066/G

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Clinical, opinion

A grouped application consisting of two Type II variations, as follows:

C.I.13: Submission of the final report from Study 5 (added in EMEA-001251-PIP01-11-M04) titled "Exposure-Response analysis of Talimogene Laherparepvec for adult subjects with melanoma from Study 20120324 and comparison to pediatric subjects' data from Study 20110261 in support of a pediatric investigational plan"

C.I.13: Submission of the final report from Study 6 (added in EMEA-001251-PIP01-11-M04) titled "Efficacy Analysis of the Young Adult Melanoma Subgroup (from 18 to less than 36 years of age) From 4 Talimogene Laherparepvec Monotherapy Studies Using Bayesian Extrapolation With Data Collected From the Older Adult Melanoma Subgroup (from 36 years of age and older) to Support Extrapolation of Efficacy From Adult Patient With Advanced Melanoma to Adolescent Patients With Advanced Melanoma".

Action: for adoption

The opinion was adopted.

2.11.6. Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0075/G

Kite Pharma EU B.V.

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

Scope: Clinical, request for supplementary information

Grouped application comprising two type II variations as follows:

C.I.13 - Submission of the final report from study KTE-C19-101 (ZUMA-1) listed as a category 3 study in the RMP. This is a Phase 1/2 Multicenter Study Evaluating The Safety And Efficacy Of Kte-C19 In Subjects With Refractory Aggressive Non-Hodgkin Lymphoma.

C.I.13 - Submission of the final report from study KTE-C19-106 (ZUMA-6) listed as a category 3 study in the RMP. This is a Phase 1-2 Multi-Center Study Evaluating The Safety And Efficacy Of Kte-C19 In Combination With Atezolizumab In Subjects With Refractory Diffuse Large B-Cell Lymphoma (DLBCL).

The RMP version 9.2 has also been submitted.

Action: for adoption

The request for supplementary information was adopted.

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

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/021

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, opinion

Action: for adoption

The recommendation was adopted.

2.13.2. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/022

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Clinical

From II/0031:

Recommendation #19: The MAH should submit, within a time period of two months, a draft protocol and SAP for a prospective observational study assessing whether potentially suboptimal bridging therapy in high-risk patients observed in the KarMMa-3 study may be alleviated in a real-world setting. This draft should then be discussed with CAT/CHMP, so that a decision can be made on whether such a prospective study should be initiated, in its initial or amended form.

Action: for adoption

The Rapporteur presented the background of this recommendation.

The CAT agreed to the conduct of the study; some amendments were proposed. The Rapporteur's assessment report was adopted.

2.13.3. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/ANX/003.2

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Pharmacovigilance

MAH Response to ANX 003.1 [PASS Study 68284528MMY4004] RSI as adopted in January 2024.

Title: An Observational Post-authorization Safety Study to Evaluate the Safety of Multiple Myeloma Patients Treated with Ciltacabtagene Autoleucel.

Action: for adoption

CAT agreed with the outcome of the PRAC-led assessment of the PASS protocol.

2.13.4. Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/REC/003.1

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality

Action: for adoption

The recommendation was adopted.

2.13.5. Luxturna - Voretigene neparvovec - Orphan - EMEA/H/C/004451/ANX/011.1

Novartis Europharm Limited

Rapporteur: Sol Ruiz

Scope: Pharmacovigilance

Fourth interim analysis for PASS CLTW888A12401 (PERCEIVE): A Post-Authorization, Multicenter, Multinational, Longitudinal, Observational Safety Registry Study for Patients Treated with Voretigene Neparvovec.

The objective of this post-authorization observational study is to collect long-term safety information (i.e. for 5 years after treatment) associated with voretigene neparvovec (vector and/or transgene), its subretinal injection procedure, the concomitant use of corticosteroids, or a combination of these procedures and products.

Action: for adoption

CAT agreed with the outcome of the PRAC-led assessment of the PASS.

2.13.6. 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

Action: for adoption

Request for supplementary information adopted on 24.05.2024.

The Rapporteur presented the assessment of the responses to the request for supplementary information. All issues are resolved. A change to the SmPC discouraging corticosteroid prophylaxis is agreed. The specific obligations are not yet resolved. The 1-year renewal was adopted.

2.13.7. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/ANX/011.2

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical & Pharmacovigilance, request for supplementary information

REVISED PROTOCOL combining STUDY No. KTE-EU-472-6036 & KT-EU-474-6644

Joint protocol combining studies KTE-EU-472-6036 & KT-EU-474-6644 as follows:
KT-EU-472-6036: Long-term, non-interventional study of recipients of Tecartus for treatment of adult patients with relapsed or refractory (r/r) mantle cell lymphoma (MCL) or adult patients with r/r B-cell precursor acute lymphoblastic leukemia (ALL).

[From Initial MAA (ANX 002): Study No. KTE-EU-472-6036: Long-term, non-interventional study of recipients of Tecartus for treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL).
From II-008-G (ANX 011): KT-EU-474-6644: Long-term, non-interventional study of the treatment by Tecartus of adult patients with relapsed or refractory (r/r) B-cell precursor acute lymphoblastic leukemia (ALL).]

Action: for adoption

The protocol of the study is assessed by the PRAC and CAT Rapporteurs. PRAC requested an amendment to the protocol. The request for supplementary information was adopted.

2.13.8. Zolgensma - Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/P46/023

Novartis Europharm Limited

Rapporteur: Emmely de Vries

Scope: Clinical

Paediatric studies submitted in accordance with Article 46 of Regulation (EC) No1901/2006, as amended.

Clinical study report - Study No. COAV101A1IC01 (OFELIA): Phase IV Open-label, single-arm, single-dose, multicenter study to evaluate the safety, tolerability and efficacy of gene replacement therapy with intravenous OAV101(AVXS101) in paediatric patients from Latin America with spinal muscular atrophy (SMA).

Action: for adoption

Request for supplementary information adopted on 15.03.2024.

The Rapporteur presented the outcome of the assessment: no new safety signals were identified in this paediatric study and there are no changes to the product information. The report was adopted.

2.13.9. Axicabtagene ciloleucel - YESCARTA (CAP) - EMEA/H/C/004480/SDA/015.1; Brexucabtagene autoleucel - TECARTUS (CAP) - EMEA/H/C/005102/SDA/013.1; Ciltacabtagene autoleucel - CARVYKTI (CAP) - EMEA/H/C/005095/SDA/016.1; Idecabtagene vicleucel - ABECMA (CAP) - EMEA/H/C/004662/SDA/020.1; Lisocabtagene maraleucel - BREYANZI (CAP) - EMEA/H/C/004731/SDA/019.1; Tisagenlecleucel - KYMRIAH (CAP) - EMEA/H/C/004090/SDA/024.1

Bristol-Myers Squibb Pharma EEIG (Abecma, Breyanzi), Kite Pharma EU B.V. (Tecartus, Yescarta), Janssen-Cilag International NV (Carvykti), Novartis Europharm Limited (Kymriah)

CAT Rapporteurs: Rune Kjeken (Kymriah, Abecma), Jan Mueller-Berghaus (Carvykti, Tecartus, Yescarta), Concetta Quintarelli (Breyanzi)

PRAC Rapporteur: Ulla Wändel Liminga

Scope: PRAC outcome on signal of secondary malignancies of T-cell origin (EPITT 20040)

Action: for information

Feedback was provided from the PRAC conclusion on the signal of secondary malignancies of T-cell origin in patients treated with CAR-T cell products. The changes to the SmPC (sections 4.4 and 4.8), education materials, RMP and Direct Healthcare Professional Communication (DHPC) were presented. CAT agreed with the proposal for strengthening of the framework for the collection and testing of tissue samples in the post-marketing setting: this will be included as a category 3 additional pharmacovigilance activity in the RMP.

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:	21.06.2024
-EMA Coordinator's draft report:	05.07.2024
-CAT Coordinator's comments:	10.07.2024
-Revised scientific recommendation:	12.07.2024
-CAT's discussion of scientific recommendation:	19-07.2024[2]

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Autologous CD34 positive cells

For regeneration purposes, to replace damaged tissue in blood and other tissues (adipogenic, osteogenic, chondrogenic, myogenic and angiogenic)

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Autologous antigenic tumor fragments isolated from patient's circulating cancer cells

For treatment of cancer patients suffering from blood cancer

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Autologous antigenic tumor fragments isolated from patient's circulating cancer cells

For treatment of cancer patients suffering from cancer of epithelial origin

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.4. Autologous antigenic tumor fragments isolated from patient's circulating cancer cells

For treatment of cancer patients suffering from melanoma

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.5. Autologous antigenic tumor fragments isolated from patient's circulating cancer cells

For treatment of cancer patients suffering from sarcoma

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.2. Day 30 ATMP scientific recommendation

4.2.1. Autologous antigen specific Cytotoxic T Lymphocytes

For treatment of cancer patients

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 05.07.2024

4.2.2. Autologous dendritic cells against tumour peptides

For treatment of cancer patients

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 05.07.2024

4.2.3. Autologous macrophages

For treatment of cancer patients

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 05.07.2024

4.2.4. Autologous cytotoxic natural killer (NK) cells

For treatment of cancer patients

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific

recommendation to the European Commission for comments by 05.07.2024

4.2.5. Autologous plasma cells producing antibodies against tumour antigen

For treatment of cancer patients

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 05.07.2024

4.2.6. Autologous adipose-derived stromal vascular fraction cells

For chronic pain relief

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 05.07.2024

4.2.7. Double stranded DNA targeting patient specific tumour neo-antigens

For treatment of non small cell lung cancer

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 05.07.2024

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

For treatment of primary hyperoxaluria

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 05.07.2024

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

4.3.1. 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

CAT noted the postponement of the finalisation of this procedure until July 2024.

4.4. Finalisation of procedure

4.4.1. MicroRNA against BCL2 anti-apoptotic messenger RNA

For treatment of cancer patients

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

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

For treatment of haematological malignancies and glioblastoma

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

The classification report was adopted. The product does fulfil the definitions of a somatic cell therapy medicinal product and a gene therapy medicinal product and based on that is considered as a gene therapy medicinal products as provided in Article 2(5) of Regulation (EC) No. 1394/2007.

4.4.3. Stromal vascular fraction

For treatment of osteoarthritis

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

The classification report was adopted. The product does fulfil the definitions of a somatic cell therapy medicinal product and tissue engineered product and based on that is considered as tissue engineered product as provided in Article 2(4) of Regulation (EC) No 1394/2007.

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

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

4.5. Follow-up and guidance

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:	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

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	08-11.07.2024
- Appointment of CAT Peer Reviewers:	17-19.07.2024
- SAWP first reports:	26.08.2024
- CAT Peer Reviewer comments (NC/C):	30.08.2024
- CAT Peer Reviewer comments (Q):	04.09.2024
- Discussion at SAWP:	02-05.09.2024
- Discussion at CAT and feedback to SAWP:	11-13.09.2024

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

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

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:	10-13.06.2024
SAWP recommendation:	11.07.2024
CAT recommendation:	19.07.2024
CHMP adoption of report and final recommendation:	25.07.2024

6.3.2. Month 1 – Discussion of eligibility

No items

6.3.3. Month 2 – Recommendation of eligibility

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

None

7.1.2. Vote by proxy

Heli Suila gave a proxy to Silke Dorner to vote on behalf of her during the entire meeting.

Alessia Pochesci gave a proxy to Claire Beuneu to vote on behalf of her during the entire meeting.

Maria Lüttgen gave a proxy to Emmely de Vries to vote on behalf of her during the entire meeting.

Kerstin Sollerbrant gave a proxy to Kieran Breen to vote on behalf of her during the entire meeting.

Petr Soukup gave a proxy to Margareta Foldova to vote on behalf of him during the entire meeting.

7.1.3. Impact of Hopveus judgment on EMA scientific meetings

Scope: Presentation of the interim measures in advance of the revision of the Policy 0044 on the handling of competing interests of scientific committees' members and experts

Action: for information

CAT noted the presentation on the interim measures to the conflict of interest policy, following the Hopveus judgement.

7.1.4. Strategic Review and Learning meeting under the Hungarian Presidency of the Council of the European Union

CAT: Viola Bardoczy

Scope: Date for the SRLM under the Hungarian Presidency: 16-18 October 2024

Action: for information

The date of the meeting was noted. The meeting will take place in Budapest.

Post-meeting note: the originally proposed date of the meeting has been changed to: 18-20 November 2024

7.2. Coordination with EMA Scientific Committees

7.2.1. Joint resolution regarding scientific committee conduct

Action: for information

CAT noted the Joint resolution regarding scientific committee conduct. This will be incorporated in the next update of the rules of procedures of each committee.

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

7.3.1. Update to the main changes for the single-arm trials reflection paper following public consultation

CAT: Jan Mueller-Berghaus

Action: for information

Jan Mueller-Berghaus presented main changes to the reflection paper on establishing efficacy based on single-arm trials submitted as pivotal evidence in a marketing authorisation, implemented based on comments received during the public consultation.

Adoption will take place at the September PROM meeting: if CAT members have any comments, please provide these to EMA secretariat .

7.4. Cooperation with the EU regulatory network

7.4.1. CAT/RWE Quarterly Update

Scope: To present the RWE/DARWIN quarterly update

Action: for information

EMA provided the quarterly update on RWE/Darwin. The following topics were discussed: CAR-T study, Pharmacogenetics pilot in DARWIN EU; upcoming/recent events. The objectives of the CAR-T study and the timelines were presented. The appointed CAT sponsors (Rune Kjeken, Jan Müller-Berghaus, Torbjorn Callreus, Olga Kholmanskikh, Rozalina Kulaksazova) will be involved in the preparation of this study.

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 27.06.2024

Action: for discussion

The agenda of the upcoming ATMP cluster was presented.

7.5.2. International Pharmaceutical Regulators Programme (IPRP) Gene and cell therapy working group

CAT: Pille Säälik

Scope: Feedback from the in-person IPRP meeting of 29.05.2024

Action: for information

Pille Säälik provided detailed feedback from the discussion in the in-person IPRP meeting on the topic of decentralised manufacturing of cell-based ATMPs.

7.6. CAT work plan

7.6.1. Guideline on quality, non-clinical and clinical requirements for investigational ATMPs in clinical trials

CAT: Ilona Reischl

Scope: Overview of comments from the second public consultation

Action: for discussion

A discussion of the general comments received during the second public consultation took place during the plenary meeting, followed by quality and non-clinical drafting group meetings.

Following CAT members will take part in the clinical drafting group meetings: Emmely de Vries, Jan Mueller-Berghaus, Olga Kholmanskikh, Ole Myrdal.

7.7. Planning and reporting

7.7.1. Business Pipeline Report

Scope: Q2/2024 Update of the Business Pipeline report for the human scientific committees

Action: for information

The information was noted.

7.8. Others

7.8.1. International Society for cell and gene therapy (ISCT) Annual meeting

CAT: Pille Säälik

Scope: Feedback from the 2nd Annual Global Regulators Summit (28.05.2024) and the ISCT Annual meeting (29.05.2024-01.06.2024)

Action: for information

The information was noted.

7.8.2. CASSS Cell and Gene Therapy Products meeting

CAT: Ilona Reischl

Scope: Feedback from the CASSS Cell and Gene Therapy Products meeting (11-13.06.2024)

Action: for information

The information was noted.

7.8.3. EU Regulation on standards of quality and safety for substances of human origin intended for human application

Scope: Conference, 24 June 2024

Action: for information

Note: the conference will be web-streamed

The information was noted.

7.8.4. Social media guidance for chairs and members of Committees

Scope: Presentation from EMA Press Office

Action: for information

CAT noted the presentation from the EMA Press Office on the use of social media (mainly LinkedIn).

8. Any other business

No items

Date of next CAT meeting:

17-19 July 2024

9. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 19-21 June 2024 meeting.

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Ilona Reischl	Chair	Austria	No interests declared	
Silke Dorner	Member	Austria	No interests declared	
Claire Beuneu	Member	Belgium	No interests declared	
Olga Kholmanskikh	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Azra Selimovic	Member	Croatia	No interests declared	
Rafaella Pontou	Member	Cyprus	No interests declared	
Petr Soukup	Member	Czechia	No interests declared	
Martin Oleksiewicz	Member	Denmark	No interests declared	
Bibi Fatima Syed Shah	Alternate	Denmark	No interests declared	
Toivo Maimets	Member	Estonia	No interests declared	
Pille Saalik	Alternate	Estonia	No interests declared	

Heli Suila	Member	Finland	No interests declared	
Violaine Closson Carella	Member	France	No interests declared	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Maria Gazouli	Member	Greece	No interests declared	
Angeliki Rompoti	Alternate	Greece	No restrictions applicable to this meeting	
Viola Bardoczy	Alternate	Hungary	No restrictions applicable to this meeting	
Joseph De Courcey	Member	Ireland	No interests declared	
Concetta Quintarelli	Member	Italy	No participation in discussion, final deliberations and voting on:	6.3.1.1.
Barbara Bonamassa	Alternate	Italy	No interests declared	
Una Riekstina	Member	Latvia	No interests declared	
Villma Perikaite	Member (CHMP member)	Lithuania	No interests declared	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No interests declared	
Nancy De Bremaeker	Alternate	Luxembourg	No interests declared	
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	
Emmely de Vries	Member	Netherlands	No interests declared	
Berendina Maria (Tineke) van den Hoorn	Alternate	Netherlands	No interests declared	
Rune Kjeken	Member	Norway	No restrictions applicable to this meeting	
Ole Henrik Myrdal	Alternate	Norway	No interests declared	
Dariusz Sladowski	Member	Poland	No restrictions applicable to this meeting	
Maria Isabel Borba Vieira	Alternate (to CHMP	Portugal	No interests declared	

	representative)			
Denisa Marilena Margina	Member	Romania	No interests declared	
Liviu Nitulescu	Alternate	Romania	No restrictions applicable to this meeting	
Katarina Kollarova	Member	Slovakia	No interests declared	
Margareta Fogelová	Alternate	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Alternate	Slovenia	No interests declared	
Sol Ruiz	Member (CHMP co-opted member)	Spain	No interests declared	
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	
Maria Luttgen	Member	Sweden	No participation in discussion, final deliberations and voting on:	4.2.8
Charlotte Anderberg	Alternate	Sweden	No interests declared	
Bernd Gansbacher	Alternate	Clinicians' Representative	No interests declared	
Alessandra Renieri	Alternate	Clinicians' Representative	No restrictions applicable to this meeting	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No interests declared	
Federica Chiara	Alternate	Patients' Representative	No restrictions applicable to this meeting	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Torbjörn Callréus	Expert	Malta		
Ulla Wändel Liminga	Expert	Sweden	No interests declared	
Andreea Barbu	Expert	Sweden	No interests declared	
Pauliina Lehtolainen-Dalkilic	Expert	Finland	No interests declared	
Odoardo Maria Olimpieri	Expert	Italy	No interests declared	
Antonella Isgro	Expert	Italy	No interests declared	
Nina Pettersen Hessvik	Expert	Norway	No interests declared	
Fabrice Eroukhmanoff	Expert	Norway	No interests declared	

Federico De Angelis	Expert	Italy	No interests declared	
Philipp Berg	Expert	Germany	No interests declared	
A representative from the European Commission attended the meeting				
Meeting run with support from relevant EMA staff				

10. 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

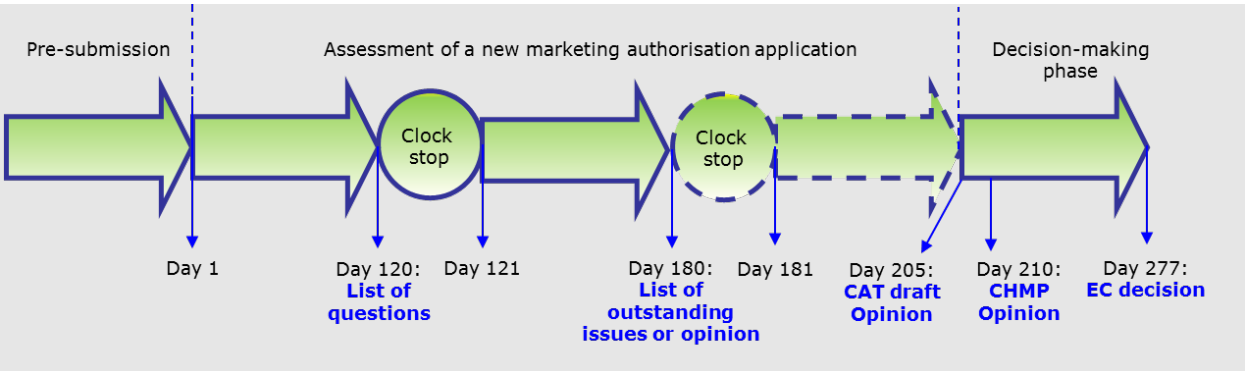
Evaluation of ATMPs (section 2)

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/