22 March 2023
EMA/CAT/79091/2023
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 15-16 February 2023

Chair: Ilona Reischl; Vice-Chair: vacant

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).

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

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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.

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 that no restriction in the involvement of meeting participants for agenda topics was identified.

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 he Committee members that received such proxy.

1.2. Adoption of agenda

CAT agenda for 15-17 February 2023 meeting was adopted.

1.3. Adoption of the minutes

CAT minutes for 18-21 January 2023 meeting were adopted.

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

No items

2.6. Update on ongoing initial applications

No items

2.7. New applications

2.7.1. Exagamglogene autotemcel - PRIME - Orphan - EMEA/H/C/005763

Vertex Pharmaceuticals (Ireland) Limited; Treatment of transfusion-dependent β-thalassemia and sickle cell disease

Scope: Timetable for assessment

Action: for adoption

The assessment timetable 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. GMP and GCP inspections requests

No items

2.11. Type II variations and variations of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008

2.11.1. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0022/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, Opinion

Action: for adoption

The opinion was adopted.

2.11.2. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0005

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli, PRAC Rapporteur: Gabriele Maurer

Scope: Clinical Pharmacology, Efficacy and Safety, Request for supplementary information

Extension of indication to include treatment of adult patients with Second-line (2L) Transplant Intended (TI) Large B-Cell Lymphoma (LBCL) for Beyanzi, based on interim analyses from pivotal study JCAR017-BCM-003; this is a global randomised multicentre Phase III trial to compare the efficacy and safety of JCAR017 to standard of care in adult subjects with high-risk, transplant-eligible relapsed or refractory aggressive B-cell Non-Hodgkin Lymphomas (TRANSFORM). As a consequence, sections 4.1, 4.4, 4.8, 5.1 and 5.2 of the SmPC are updated. The Package Leaflet is updated in accordance. Version 2.0 of the RMP has also been submitted.

Action: for adoption

Request for supplementary information adopted on 09.09.2022.

The rapporteur presented the outcome of the assessment of the responses to the request for supplementary information. The updated efficacy data from the primary analysis of study BCM-003 (longer follow-up) confirmed the efficacy of Breyanzi in the second line indication. No new safety concerns were identified in this 2L patient population studied in BCM003. The inclusion of rarer LBCL in the indication was discussed.

A second request for supplementary information was adopted.

2.11.3. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0007/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, Opinion

Action: for adoption

Request for supplementary information adopted on 09.12.2022.

2.11.4. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0009

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, Opinion

Action: for adoption

Request for supplementary information adopted on 09.12.2022.

The opinion was adopted.

2.11.5. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0013/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, Request for supplementary information

Action: for adoption

The Rapporteur presented the assessment of this variation.

The Request for supplementary information was adopted.

2.11.6. Libmeldy - atidarsagene autotemcel - Orphan - EMEA/H/C/005321/II/0011/G

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Carla Herberts, PRAC Rapporteur: Gabriele Maurer

Scope: Clinical & Quality, Opinion

Update of sections 4.2, 4.4, 4.5, 4.8, and 5.1 of the SmPC; the Package Leaflet and Labelling are updated accordingly. In addition, the MAH took the opportunity to remove ANX/002 from the Annex II and to introduce minor editorial changes to the product information (PI). The RMP version 1.3 has also been submitted.

Action: for adoption

Request for supplementary information adopted on 09.12.2022.

2.11.7. Upstaza - eladocagene exuparvovec - Orphan - EMEA/H/C/005352/II/0004/G

PTC Therapeutics International Limited

Rapporteur: Maura O'Donovan

Scope: Quality, Opinion

Action: for adoption

Request for supplementary information adopted on 09.12.2022.

The opinion was adopted.

2.11.8. Upstaza - eladocagene exuparvovec - Orphan - EMEA/H/C/005352/II/0005/G

PTC Therapeutics International Limited

Rapporteur: Maura O'Donovan

Scope: Quality, Opinion

Action: for adoption

The opinion was adopted.

2.11.9. Tecartus - brexucabtagene autoleucel; Yescarta - axicabtagene ciloleucel; - Orphan - EMEA/H/C/WS2389/G

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, Request for supplementary information

Action: for adoption

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/012.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality

Action: for adoption

The Rapporteur's assessment report of this post-authorisation measure was adopted.

2.13.2. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/013.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality

Action: for adoption

The Rapporteur's assessment report of this post-authorisation measure was adopted.

2.13.3. Carvykti - ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/REC/011

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

The Rapporteur's assessment report of this post-authorisation measure was adopted.

2.13.4. Carvykti - ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/REC/012

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

The Rapporteur's assessment report of this post-authorisation measure was adopted.

2.13.5. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/R/0068

Novartis Europharm Limited

Rapporteur: Rune Kjeken, Co-Rapporteur: Dariusz Sladowski, PRAC Rapporteur: Gabriele Maurer

Scope: 5-year Renewal of Marketing Authorisation, Opinion

Action: for adoption

The Rapporteur presented the assessment of the renewal application for Kymriah. The SmPC has been updated in line with the published core SmPC for genetically modified cells.

The renewal opinion was adopted.

2.13.6. Roctavian - valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/MEA/003

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Pharmacovigilance

To inform the impact of valoctocogene roxaparvovec (BMN 270) on fertility, general toxicity, teratology, and germline transmission in females of childbearing potential and establish an adequate waiting period after BMN 270 infusion following which female patients can become pregnant.

Action: for adoption

The Rapporteur provided feedback from the PRAC discussion: this is in line with the CAT feedback from the informal discussion at the January CAT meeting[1]. The PRAC report was adopted.

It was agreed that the CAT will assess the responses and any subsequent steps of this procedure.

[1] See CAT minutes of the January 2023 CAT meeting, agenda point 2.13.6.

2.13.7. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/R/0056

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Claire Beuneu, PRAC Rapporteur: Anette Kirstine Stark

Scope: 5-year Renewal of Marketing Authorisation, Request for supplementary information

Action: for adoption

The Rapporteur presented the assessment of the renewal application for Yescarta. The PRAC Rapporteur has identified some questions on the RMP. There was a discussion on the out-of-specification (OOS) batches for Yescarta that have been used in patients on request of the treating physician. The MAH will be requested to provide information on the total number of OOS batches (including those that are not used in patients).

The request for supplementary information was adopted.

2.13.8. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/013

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, CHMP Coordinator: Jan Mueller-Berghaus

Scope: Clinical, Request for supplementary information

Action: for adoption

The Rapporteur presented the assessment of this post-authorisation measure. CAT agreed with the Rapporteur's position.

The request for supplementary information was adopted.

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:	22.02.2023
-EMA Coordinator's draft report:	03.03.2023
-CAT Coordinator's comments:	08.03.2023
-Revised scientific recommendation:	17.03.2023
-CAT's discussion of scientific recommendation:	24.03.2023

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Lyophilized supernatant of a pathogen inactivated and gamma sterilized platelet lysate

Treatment of topical treatment of skin ulcers

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Autologous intestinal organoid derived from adult stem cells from intestinal epithelial tissue

Treatment of intractable ulcer

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Autologous chondrocytes cultured in hyaluronan-derived scaffold

Repair of cartilage defects

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. Umbilical Cord Wharton Jelly-derived mesenchymal stem cells (MSCs) cells

Treatment of spinal cord injury; drug resistant epilepsy; hypoxia ischemia encephalopathy

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 3 March 2023.

4.2.2. Bladder acellular matrix (BAM) based scaffold seeded with allogenic or autologous adipose-derived stromal cells

Treatment of urinary bladder wall augmentation in patients with small capacity high pressure urinary bladder

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 3 March 2023.

4.2.3. Fibrin gel containing autologous leucocyte- and platelet-rich plasma, autologous thrombin, and ascorbic acid

Treatment of wounds

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 3 March 2023.

4.2.4. Ex-vivo expanded allogeneic human corneal endothelial cells

Treatment of diseases of the corneal endothelium

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 3 March 2023.

4.2.5. Recombinant Adeno-Associated Viral Vector expressing a codon optimised human RPGR gene (rAAV2tYF-GRK1-RPGR)

Treatment of X-linked retinitis pigmentosa (XLRP) caused by mutations in the RPGR gene

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 3 March 2023.

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

No items

4.4. Finalisation of procedure

4.4.1. Autologous platelet concentrate, consisting of a fibrin matrix enriched with platelets, leukocytes and of cytokines and growth factors

Treatment of patients with critical limb ischaemia, in combination with mechanical lower limb revascularisation (angioplasty)

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 tissue engineered product as provided in Article 2(1) of Regulation (EC) No 1394/2007.

4.4.2. Autologous adipose tissue derived progenitor cells in biodegradable chemically crosslinked hydrogel

Treatment of subacute spinal cord injury in adults with a complete lesion (ASIA A score)

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 tissue engineered product as provided in Article 2(1) of Regulation (EC) No 1394/2007.

4.4.3. Genetically engineered *Eschericia coli* strain containing a plasmid expressing CRISPR-Cas against clbA, clbB and clbC

Prevention of disease progression in Familial Adenomatous Polyposis (FAP)

Scope: European Commission raised comments. Updated ATMP scientific recommendation

Action: for adoption

The updated 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.

4.4.4. Mitochondria isolated from allogeneic umbilical-cord mesenchymal stem cells

Treatment of Polymyositis/Dermatomyositis

Scope: European Commission raised no comments. 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.

4.4.5. Macrophage-Drug Conjugate (MDC) composed of allogenic human monocyte-derived macrophages loaded with a protein-drug conjugate

Treatment of solid tumours

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 somatic cell therapy medicinal product as provided in Article 2(1) of Regulation (EC) No 1394/2007.

4.4.6. DNA plasmid vector encoding human insulin like growth factor binding protein 2

Treatment of newly diagnosed advanced ovarian cancer after debulking surgery

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.

4.4.7. Autologous muscle precursor cells (MPCs)

Treatment of female stress urinary incontinence

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 tissue engineered product as provided in Article 2(1) of Regulation (EC) No 1394/2007.

4.4.8. Adult autologous regenerative cells

Indicated for regeneration, repair, or replacement of weakened or injured subcutaneous tissue

Scope: European Commission raised no comments. 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. As the evidence provided was indirect, CAT considered that the classification conclusion is provisional and might have to be revisited when the state of science evolves.

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:	06-09.02.2023
- Appointment of CAT Peer Reviewers:	15-17.02.2023
- SAWP first reports:	06.03.2023
- CAT Peer Reviewer comments (NC/C):	10.03.2023
- CAT Peer Reviewer comments (Q):	15.03.2023
- Discussion at SAWP:	22-24.03.2023
- Discussion at CAT and feedback to SAWP:	19-21.04.2023

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	13-16.03.2023
- Appointment of CAT Peer Reviewers:	22-24.03.2023
- SAWP first reports:	03.04.2023
- CAT Peer Reviewer comments (NC/C):	05.04.2023
- CAT Peer Reviewer comments (Q):	12.04.2023
- Discussion at SAWP:	19-21.04.2023
- Discussion at CAT and feedback to SAWP:	15-17.05.2023

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

No items

6.2. ITF briefing meetings in the field of ATMPs

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:	
Procedure start:	06-09.02.2023
SAWP recommendation:	16.03.2023
CAT recommendation:	24.03.2023
CHMP adoption of report and final recommendation:	30.03.2023

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. Election of CAT Vice-chairperson

Action: for adoption

The election of the new vice-chair took place in accordance with the CAT rules of procedure.

The nominations received were presented to the Committee.

The CAT elected Carla Herberts as CAT Vice-Chair for a three-year mandate starting on 15 March 2023.

The CAT and the Agency congratulated Carla Herberts on her election and wished her all the best in new role as Vice-Chair of the Committee.

7.1.2. CAT membership

Action: for information

The CAT Chair welcomed Marid Hystad, as the new alternate for Norway.

7.1.3. Vote by proxy

Action: for information

Silke Dorner gave a proxy to Heli Suila to vote on behalf of Silke Dorner during the election of the CAT Vice-chairperson.

Una Riekstina gave a proxy to Dariusz Sladowski to vote on behalf of Una Riekstina during the election of the CAT Vice-chairperson.

7.1.4. CAT Strategic Review & Learning meeting (SRLM) under the Sweden presidency, 4-5 May 2023, Upsala (Sweden)

CAT: Lisbeth Barkholt, Maria Lüttgen

Scope: Topics for discussion at the upcoming SRLM

Action: for discussion

The draft agenda was discussed.

7.1.5. CAT meeting dates 2025/2026

Action: for information

The information was noted.

7.2. Coordination with EMA Scientific Committees

7.2.1. CHMP Scientific Advice Working Party (SAWP): CAT–SAWP joint members

Scope: Renomination or nomination of new joint CAT-SAWP members

Action: for discussion

CAT nominated following members as the joint CAT-SAWP members: Rune Kjeken and Ebru Karakoc Madsen

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

7.3.1. PCWP-HCPWP annual meeting

Scope: Meeting summary from the PCWP-HCPWP annual meeting with all eligible organisations held on 15 November 2022 and agenda for the PCWP and HCPWP meeting to be held on 3 March 2023

Action: for information

The information was noted.

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) - POSTPONED

CAT: Ilona G. Reischl

Scope: Agenda for the teleconference to be held on 23 February 2023.

Action: for information

7.6. CAT work plan

7.6.1. Real World Evidence (RWE) update, including DARWIN EU®

Scope: To update the CAT members on the progress and finalisation of the first year studies, the prioritisation and selection of studies and data partners for the second year, the RWE experience report.

Action: for information

CAT noted the information on the experience with RWE and DARWIN EU. CAT was informed how RWE studies are selected.

7.6.2. Real World Evidence (RWE), Readers' Guidance

Scope: Template text for Real World Data / Real World Evidence in the Readers' Guidance

Action: for information

CAT was informed that the readers' guidance template has been updated to include information on Real World Data in the MAA and to include RWE questions if an evidence gap is identified.

7.6.3. Update on collaboration with Health Technology Assessment (HTA)-bodies

Action: for information

EMA provided an update on the collaboration with HTA bodies (past, present and future). A bilateral (EMA-EUnetHTA21) on ATMPs will take place on 31 March 2023: one of the recently approved ATMPs will be selected for presentation / discussion.

7.7. Planning and reporting

7.7.1. Innovation Task Force (ITF) end of the year report

Scope: ITF end of year report

Action: for information

EMA presented an overview of ITF briefing meetings and ITF activities in 2022.

7.8. Others

7.8.1. EIT EMAN Genome Editing Workshop

Scope: Nomination of 3 CAT members to attend this workshop as formal CAT representatives

Action: for discussion

This workshop is on invitation only. Invited speakers and attendees will get a reimbursed invitation. Feedback from the workshop will be provided to CAT.

CAT appointed Ilona Reischl and Alessandro Aiuti as CAT representatives.

Post-meeting note: the meeting will not be web streamed.

7.8.2. Project on outcome measures in Epidermolysis Bullosa

Scope: Call for interest for kick-off meeting

Action: for information.

CAT noted the information on the project on outcome measures in Epidermolysis bullosa. CAT members interested to take part in the kick-off meeting should contact EMA.

8. Any other business

8.1. Oncology European Specialised Expert Community (ESEC)

Scope: Presentation and updates of their activities

Action: for information

EMA presented the activities of the Oncology ESEC. Oncology experts can still be nominated for the Oncology ESEC: an e-mail with the experts' names and a brief description of their expertise should be sent to EMA.

Recently, a cardiovascular ESEC has been created: nominations of experts can also be sent to EMA.

8.2. EMA's reimbursement rules

Scope: Clarification of reimbursement rules for members and alternates following discussions at January's CAT meeting.

Action: for information

CAT noted the clarification of the reimbursement rules.

Date of next CAT meeting:

22-24 March 2023

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

SWP: Safety Working Party

SME: Small and medium size enterprises

SmPC: Summary of Products Characteristics

TT: Timetable

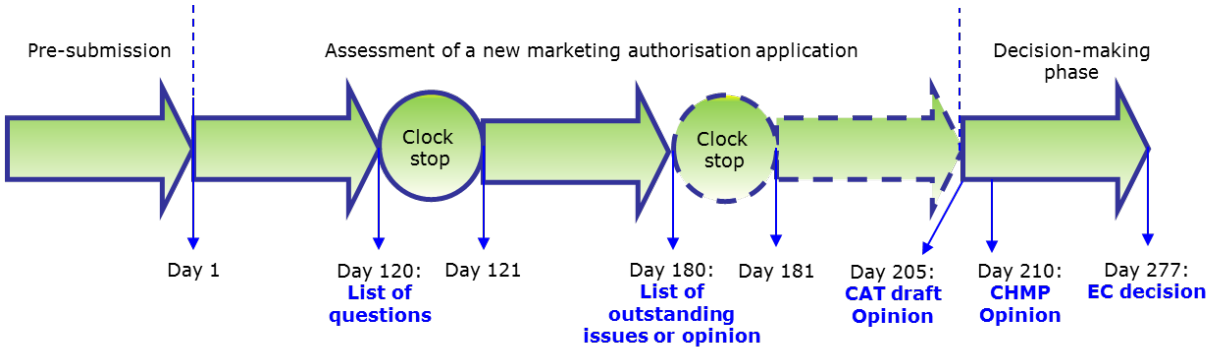
Evaluation of ATMPs (section 2)

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 any ATMP related inspection requests (section 2.9) and Post-authorisation activities (section 2.10).

New applications (sections 2.1. to 2.12.)

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 2.3) 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 2.7 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.

Re-examination procedures (new applications) under article 9(2) of regulation no 726/2004 (section 2.6.)

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.

Withdrawal of applications (section 2.7.)

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.

New applications (section 2.9.)

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.

GMP and GCP Inspections Issues (section 2.10.)

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).

Post-authorisation activities (section 2.12.)

This section lists type II variations, extension application according to Annex I of Reg. 1234/2008, re-examination procedures for type II variations (including extension of indication applications) for which the applicant has requested re-examination of the opinion previously issued by the CHMP and other issues concerning authorised medicines that are not covered elsewhere in the agenda such as 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.

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/

10. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 15-16 February 2023 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	
Claire Beuneu	Member	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	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	
Petr Soukup	Member	Czechia	No interests declared	
Kristyna Rehorova Hradilkova	Alternate	Czechia	No interests declared	
Ebru Karakoc Madsen	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	
Maija Tarkkanen	Alternate	Finland	No interests declared	
Violaine Closson	Member	France	No interests declared	
Jean-Michel Race	Alternate	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 interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Katalin Lengyel	Member	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	
Niamh Curran	Alternate	Ireland	No restrictions applicable to this meeting	
Concetta Quintarelli	Member	Italy	No interests declared	
Barbara Bonamassa	Alternate	Italy	No restrictions applicable to this meeting	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No interests declared	
Nancy De Bremaeker	Member	Luxembourg	No interests declared	
John J. Borg	Member (CHMP member)	Malta	No interests declared	
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	
Carla Herberts	Member	Netherlands	No interests declared	
Babs Fabriek	Alternate	Netherlands	No interests declared	
Rune Kjeken	Member	Norway	No restrictions applicable to this meeting	
Dariusz Śladowski	Member	Poland	No restrictions applicable to this meeting	
Maria Isabel Borba Vieira	Alternate (to CHMP representative)	Portugal	No interests declared	
Silviu Istrate	Member	Romania	No interests declared	
Katarina Vavrová	Member	Slovakia	No interests declared	
Margareta Fogelová	Alternate	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	
Lisbeth Barkholt	Member	Sweden	No interests declared	
Maria Luttgen	Alternate	Sweden	No restrictions applicable to this meeting	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	
Alessandro Aiuti	Member	Clinicians' Representative	No participation in discussions, final deliberations and voting on:	2.11.6: Libmeldy II/11
Alessandra Renieri	Alternate	Clinicians' Representative	No restrictions applicable to this meeting	
Kerstin Sollerbrant	Member	Patients' Representative	No interests declared	
Kieran Breen	Member	Patients' Representative	No interests declared	
Charlotte Anderberg	Expert - in person*	Sweden	No restrictions applicable to this meeting	
Anna Vikefors	Expert - in person*	Sweden	No interests declared	
Odoardo Olimpieri	Expert - in person*	Italy	No interests declared	
Antonella Isgrò	Expert - via telephone*	Italy	No interests declared	
Andrea Gaggioli	Expert - via telephone*	Italy	No interests declared	
Anna Kajaste	Expert - via telephone*	Italy	No restrictions applicable to this meeting	
Beate Mosl	Expert - via telephone*	Germany	No restrictions applicable to this meeting	
Attila Sebe	Expert - via telephone*	Germany	No interests declared	
Torbjorn Callreus	Expert - via telephone*	Malta	No interests declared	
A representative from the European Commission attended the meeting Meeting run with support from relevant EMA staff				