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SCIENCE MEDICINES HEALTH

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Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 17-19 July 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).



Table of contents

1.	Introduction	5
1.1.	Welcome and declarations of interest of members, alternates and experts.....	5
1.2.	Adoption of agenda	5
1.3.	Adoption of the minutes	5
2.	Evaluation of ATMPs	5
2.1.	Opinions	5
2.2.	Oral explanations	5
2.3.	Day 180 list of outstanding issues	5
2.4.	Day 120 list of questions	6
2.4.1.	Obecabtagene autoleucel - PRIME - Orphan - EMEA/H/C/005907	6
2.4.2.	Mozafancogene autotemcel - PRIME - Orphan - EMEA/H/C/005537	6
2.5.	Day 80 assessment reports	6
2.6.	Update on ongoing initial applications.....	6
2.7.	New applications	6
2.8.	Withdrawal of initial marketing authorisation application	6
2.9.	Re-examination of initial application procedures under Article 9(2) of Regulation No. 726/2004	6
2.10.	Companion diagnostics.....	7
2.10.1.	Initial consultation	7
2.10.2.	Follow-up consultation.....	7
2.11.	Type II variations and variations of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008	7
2.11.1.	Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0047	7
2.11.2.	Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0048	7
2.11.3.	Alofisel - Darvadstrocel - Orphan - EMEA/H/C/004258/II/0051/G	7
2.11.4.	Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/II/0037/G	8
2.11.5.	Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/II/0014/G	8
2.11.6.	Imlygic - Talimogene laherparepvec - EMEA/H/C/002771/II/0067/G	8
2.11.7.	Upstaza - Eladocogene exuparvovec - Orphan - EMEA/H/C/005352/II/0022/G	9
2.11.8.	Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0077.....	9
2.12.	Extension applications.....	9
2.13.	Other Post-Authorisation Activities	9
2.13.1.	Breyanzi - Lisocabtagene maraleucel - EMEA/H/C/004731/MEA/007.1.....	9
2.13.2.	Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/REC/016.1.....	10
2.13.3.	Zolgensma - Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/LEG/024.....	10

2.14.	GMP and GCP inspections requests	10
3.	Certification of ATMPs	10
3.1.	Opinion	11
3.2.	Day 60 Evaluation Reports.....	11
3.3.	New Applications	11
4.	Scientific Recommendation on Classification of ATMPs	11
4.1.	New requests – Appointment of CAT Coordinator	11
4.2.	Day 30 ATMP scientific recommendation	11
4.2.1.	Autologous CD34 positive cells	11
4.2.2.	Autologous antigenic tumor fragments isolated from patient’s circulating cancer cells	11
4.2.3.	Autologous antigenic tumor fragments isolated from patient’s circulating cancer cells	11
4.2.4.	Autologous antigenic tumor fragments isolated from patient’s circulating cancer cells	12
4.2.5.	Autologous antigenic tumor fragments isolated from patient’s circulating cancer cells	12
4.3.	Day 60 revised scientific recommendation (following list of questions)	12
4.3.1.	Implantable device 3D bioprinted with autologous microfat and hydrogel bioink	12
4.4.	Finalisation of procedure	13
4.4.1.	Autologous adipose-derived stromal vascular fraction cells	13
4.4.2.	Double stranded DNA targeting patient specific tumour neo-antigens	13
4.4.3.	Autologous antigen specific Cytotoxic T Lymphocytes	13
4.4.4.	Autologous dendritic cells against tumour peptides	13
4.4.5.	Autologous macrophages	14
4.4.6.	Autologous cytotoxic natural killer (NK) cells	14
4.4.7.	Autologous plasma cells producing antibodies against tumour antigen	14
4.4.8.	Synthetic double-stranded RNA oligonucleotide conjugated to GalNAc aminosugar residues	14
4.5.	Follow-up and guidance	14
5.	Scientific Advice	15
5.1.	New requests - appointment of CAT Rapporteurs	15
5.1.1.	Ongoing scientific advice procedures - Appointment of CAT Peer Reviewers	15
5.1.2.	Scientific advice procedures starting at the next SAWP meeting	15
5.2.	Procedures discussed at SAWP – 1st reports, D40 JRs, LoIs	15
5.3.	Finalisation of D70 procedures – feedback from the discussion meeting	15
5.4.	Final Advice Letters for procedures finalised the previous month	15
6.	Pre-Authorisation Activities	15
6.1.	Paediatric investigation plans	15
6.2.	ITF briefing meetings in the field of ATMPs	16
6.3.	Priority Medicines (PRIME) – Eligibility requests	16

6.3.1.	Month 0 - Start of the procedure	16
6.3.2.	Month 1 – Discussion of eligibility	16
6.3.3.	Month 2 – Recommendation of eligibility.....	16
6.3.4.	Ongoing support.....	16

7.	Organisational, regulatory and methodological matters	16
7.1.	Mandate and organisation of the CAT	16
7.1.1.	CAT membership	16
7.1.2.	Vote by proxy	16
7.1.3.	Update on CAT Time schedule	16
7.1.4.	Strategic Review and Learning meeting under the Hungarian Presidency of the Council of the European Union.....	17
7.2.	Coordination with EMA Scientific Committees.....	17
7.3.	Coordination with EMA Working Parties/Working Groups/Drafting Groups	17
7.3.1.	GVP Module XVI on risk minimisation measures and its Addendum II.....	17
7.3.2.	Reflection paper on criteria to be considered for the evaluation of new active substance (NAS) status of biological substances	17
7.3.3.	Questions and Answers on decentralised manufacturing	17
7.4.	Cooperation with the EU regulatory network.....	18
7.5.	Cooperation with international regulators.....	18
7.5.1.	ATMP cluster teleconference with US-FDA, Health Canada and PMDA (Japan).....	18
7.6.	CAT work plan	18
7.6.1.	CAT work plan 2025.....	18
7.6.2.	Guideline on quality, non-clinical and clinical requirements for investigational ATMPs in clinical trials	18
7.7.	Planning and reporting	19
7.8.	Others	19
8.	Any other business	19
8.1.	Update to CxMPs on IRIS for Regulatory Procedures.....	19
9.	List of participants	19
10.	Explanatory notes	22

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 remotely.

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.

1.2. Adoption of agenda

The CAT agenda for 17-19 July 2024 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for 19-22 June 2024 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

2.4.1. 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 120 list of questions

Action: for adoption

The CAT Rapporteurs presented the outcome of the assessment. Feedback was provided from the BWP discussion.

The list of questions was adopted.

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

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

Scope: Day 120 list of questions

Action: for adoption

The CAT Rapporteurs presented the outcome of the assessment. Feedback was provided from the BWP discussion.

The list of questions was adopted.

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

No items

2.7. New applications

No items

2.8. Withdrawal of initial marketing authorisation application

No items

2.9. Re-examination of initial application procedures under Article 9(2) of Regulation No. 726/2004

No items

2.10. 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

Scope: Quality, request for supplementary information

Action: for adoption

Request for supplementary information adopted on 24.05.2024.

The second request for supplementary information was adopted.

2.11.2. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0048

Bristol-Myers Squibb Pharma EEIG

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 21.06.2024.

The opinion was adopted.

2.11.3. Alofisel - Darvadstrocel - Orphan - EMEA/H/C/004258/II/0051/G

Takeda Pharma A/S

Rapporteur: Maria Luttgen, PRAC Rapporteur: Gabriele Maurer

Scope: Efficacy, request for supplementary information

A grouped application comprised of 4 Type II Variations, as follows:

(C.I.4): Update of sections 4.8 and 5.1 of the SmPC in order to update the safety information, based on pooled safety data from the two phase 3 controlled studies (ADMIRE-CD & ADMIRE-CD II) and to update efficacy information based on final results from study ADMIRE-CD II, listed as an obligation in the Annex II. ADMIRE-CD II (Cx601-0303) is a Phase III randomised double blind, placebo controlled study to assess efficacy and safety of Cx601, adult allogeneic expanded adipose-derived stem cells (eASC) for the treatment of

complex perianal fistula(s) in patients with Crohn's disease. The Annex II is updated accordingly. In addition, the MAH took the opportunity to introduce minor changes to the PI, including to section 4.2 of the SmPC and to the Package Leaflet.

3 x (C.I.13): Submission of interim results from studies Darvadstrocel-3003 and Alofisel-5003 (INSPIRE) and final results from study Darvadstrocel-3002 to support the benefit-risk assessment of darvadstrocel based on all new available clinical data.

The RMP version 8.0 has also been submitted.

Action: for adoption

The Rapporteur presented the outcome of the assessment. The request for supplementary information was adopted.

2.11.4. [Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/II/0037/G](#)

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 21.06.2024, 15.03.2024.

The opinion was adopted.

2.11.5. [Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/II/0014/G](#)

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.6. [Imlygic - Talimogene laherparepvec - EMEA/H/C/002771/II/0067/G](#)

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Clinical

A grouped application as follows:

Type II (C.I.4): Update of sections 4.2, 4.8 and 5.1 of the SmPC in order to update paediatric information based on the paediatric study 20110261, which was previously submitted in procedure II/0063. This is a phase 1, multicentre, open-label study of talimogene laherparepvec in paediatric subjects with advanced non-CNS tumours that were amenable to direct injection in the clinical setting. The Package Leaflet is updated

accordingly. In addition, the MAH took the opportunity to introduce minor editorial and formatting changes to the Product Information.

Type IA (A.6): To change the ATC Code of Antineoplastic cell and gene therapy from L01XX51 to L01XL02.

Action: for adoption

The opinion was adopted.

2.11.7. Upstaza - Eladocagene exuparvovec - Orphan - EMEA/H/C/005352/II/0022/G

PTC Therapeutics International Limited

Rapporteur: Joseph DeCoursey

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.8. Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0077

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

The Rapporteur presented the assessment of the variation. The request for supplementary information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Breyanzi - Lisocabtagene maraleucel - EMEA/H/C/004731/MEA/007.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Pharmacovigilance

MAH's response to MEA 007 [LTFU study (GC LTFU 001)] RSI as adopted in January 2024.

Long-term follow-up of safety and efficacy for all paediatric and adult subjects exposed do a GM T cell therapy in Bristol-Myers Squibb sponsored, or Bristol Myers Squibb alliance partner sponsored, clinical trials in accordance with Health Authorities' guidance for long-term (up to 15 years) follow-up of subjects treated with gene therapy products.

Action: for adoption

The Rapporteur presented the assessment.

However, the MAH should provide the next interim report as per the agreed due date including satisfactory responses to the request for supplementary information as part of this procedure. The request for supplementary information was adopted.

2.13.2. **Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/REC/016.1**

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality

Action: for adoption

The Rapporteur's assessment was adopted.

2.13.3. **Zolgensma - Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/LEG/024**

Novartis Europharm Limited

Rapporteur: Emmely de Vries

Scope: Non-clinical

From PSUSA-00010848-202305

Study reports for 2170026 & 222018:

- Study no. 2170026 (OAV101: Single Dose Intravenous or Intracerebroventricular Germline Transduction and Integration Study in Neonatal FVB/NCrI Mice with up to a 24-Week Evaluation Period).

- Study no. 2220183: AAV Vector expression in cynomolgus macaque gonadal tissue of scAAV9-CB-GFP and scAAV9-CB-mCherry 28 days after administration

Action: for adoption

The Rapporteur presented the assessment of the two studies. CAT discussed the questions to the applicant. The request for supplementary information was adopted.

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

3.3. New Applications

No items

4. Scientific Recommendation on Classification of ATMPs

4.1. New requests – Appointment of CAT Coordinator

No items

4.2. Day 30 ATMP scientific recommendation

4.2.1. Autologous CD34 positive cells

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

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 09.08.2024

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

For treatment of cancer patients suffering from blood cancer

CAT Coordinator: Emmely de Vries

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 09.08.2024.

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

For treatment of cancer patients suffering from cancer of epithelial origin

CAT Coordinator: Emmely de Vries

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 09.08.2024.

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

For treatment of cancer patients suffering from melanoma

CAT Coordinator: Emmely de Vries

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 09.08.2024.

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

For treatment of cancer patients suffering from sarcoma

CAT Coordinator: Emmely de Vries

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 09.08.2024.

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

The ATMP classification report that was updated further to the responses from the applicant on the questions raised by CAT was discussed. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 09.08.2024.

4.4. Finalisation of procedure

4.4.1. Autologous adipose-derived stromal vascular fraction cells

For chronic pain relief

Scope: European Commission raised comments. ATMP scientific recommendation

Action: for adoption

CAT discussed the comments raised by the European Commission. The ATMP classification report was updated to clarify this. 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.

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

For treatment of non small cell lung cancer

Scope: European Commission raised comments. ATMP scientific recommendation

Action: for adoption

CAT discussed the comments raised by the European Commission. The ATMP classification report was updated to strengthen this conclusion. 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.

4.4.3. Autologous antigen specific Cytotoxic T Lymphocytes

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

4.4.4. Autologous dendritic cells against tumour peptides

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 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. Autologous macrophages

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

4.4.6. Autologous cytotoxic natural killer (NK) cells

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

4.4.7. Autologous plasma cells producing antibodies against tumour antigen

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

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

For treatment of primary hyperoxaluria

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.

4.5. 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:	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

5.1.2. Scientific advice procedures starting at the next SAWP meeting

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:	30.09-02.10.2024
- Discussion at CAT and feedback to SAWP:	09-11.10.2024

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

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:	
Procedure start:	08-11.07.2024
SAWP recommendation:	05.09.2024
CAT recommendation:	13.09.2024
CHMP adoption of report and final recommendation:	19.09.2024

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

No items

7.1.2. Vote by proxy

No items

7.1.3. Update on CAT Time schedule

Action: for information

CAT agreed with the EMA proposal to try out an interactive time schedule at the September CAT meeting.

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

CAT: Viola Bardoczy

Scope: Draft agenda

Action: for discussion

The date for the SRLM under the Hungarian Presidency is changed from date announced in the June CAT meeting: 18-20 November 2024. The agenda is under development: presentation will be scheduled from the Hungary medicines agency, academia and ATMP developers (from industry); a CAT closed session will also be included.

7.2. Coordination with EMA Scientific Committees

No items

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

7.3.1. GVP Module XVI on risk minimisation measures and its Addendum II

Action: for information (presentation following written consultation of CAT concluded on 4 July 2024)

CAT noted the presentation by EMA on the GVP Module XVI.

7.3.2. Reflection paper on criteria to be considered for the evaluation of new active substance (NAS) status of biological substances

Scope: Status update from drafting group

Action: for information

Following CAT members/experts are contributing to the drafting group discussions on the NAS status for ATMPs: B Bonamassa, H Suila, I Reischl, J Scherer, M van de Bovenkamp.

CAT noted the presentation from EMA on the status of the finalisation of the reflection paper on criteria to be considered for the evaluation of new active substance (NAS) status of biological substances.

7.3.3. Questions and Answers on decentralised manufacturing

Scope: Presentation of the updated Questions and Answers (prepared by Quality Innovation Group)

Action: for discussion

EMA presented the latest version of the questions and answers document on decentralised manufacturing. The changes from the previous version were highlighted. The document will be adopted via a written procedure.

CAT noted that the questions and answers document on comparability might need to be updated to include guidance on how to demonstrate comparability between products that are manufactured in a decentralised setting. This could be included in the CAT workplan for 2025 (in conjunction with BWP).

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: Feedback from the teleconference of 27.06.2024

Action: for information

Feedback was provided from the discussion in the last ATMP cluster teleconference.

7.6. CAT work plan

7.6.1. CAT work plan 2025

CAT: Ilona Reischl, Claire Beuneu, Rune Kjekken, Jan Mueller-Berghaus, Mencia de Lemus Belmonte, Maria Lüttgen, Olga Kholmanskikh, Dariusz Sladowski

Scope: Tour de table on progress of 2024 CAT workplan activities and discuss timelines and next steps for 2025 CAT workplan

Action: for information

EMA presented the status of the different 2024 CAT workplan activities and discuss topics for inclusion in the 2025 CAT workplan.

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

CAT: Ilona Reischl

Scope: Status update: feedback from the quality and non-clinical drafting groups

Action: for information

Ilona Reischl provided an update on the discussion of the comments received during the second public consultation of the Guideline on quality, non-clinical and clinical requirements for investigational ATMPs in clinical trials. The aim of the quality, non-clinical and clinical drafting groups is to finalise their work so that a final version can be presented and agreed at the September CAT meeting. This will allow to present the final guideline at the CAT Scientific Symposium (that will take place on 10 October 2024).

7.7. Planning and reporting

No items

7.8. Others

No items

8. Any other business

8.1. Update to CxMPs on IRIS for Regulatory Procedures

Scope: To update the impacted committees on further procedures to be implemented in IRIS by the end of 2024 (PSURs, PAMs, Line extensions, Renewals, Annual Reassessments, PASS, Referrals)

Action: for information

EMA presented the status of the implementation of post-authorisation procedure in IRIS and the impact on the working of the Committee.

Date of next CAT meeting:

13-16 August 2024 (written procedure)

9. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 17-19 July 2024 meeting.

<u>Name</u>	<u>Role</u>	<u>Member State or affiliation</u>	<u>Outcome restriction following evaluation of e-DoI</u>	<u>Topics on agenda for which restrictions apply</u>
Ilona Reischl	Chair	Austria	No interests declared	
Silke Dorner	Member	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	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	

Petr Soukup	Member	Czechia	No interests declared	
Martin Oleksiewicz	Member	Denmark	No interests declared	
Toivo Maimets	Member	Estonia	No interests declared	
Pille Saalik	Alternate	Estonia	No interests declared	
Maija Tarkkanen	Alternate	Finland	No interests declared	
Violaine Closson Carella	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 restrictions applicable to this meeting	
Viola Bardoczy	Alternate	Hungary	No restrictions applicable to this meeting	
Joseph De Courcey	Member	Ireland	No interests declared	
Richard Carroll	Alternate	Ireland	No interests declared	
Concetta Quintarelli	Member	Italy	No participation in discussion, final deliberations and voting on:	
Barbara Bonamassa	Alternate	Italy	No interests declared	
Una Riekstina	Member	Latvia	No interests declared	
Villma Perikaite	Member (CHMP member)	Lithuania	No interests declared	
Nancy De Bremaeker	Alternate	Luxembourg	No interests declared	
John J. Borg	Member (CHMP member)	Malta	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 Kjeiken	Member	Norway	No participation in discussion, final deliberations and voting on:	
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 representative)	Portugal	No interests declared	
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	Member	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 restrictions applicable to this meeting	
Charlotte Anderberg	Alternate	Sweden	No interests declared	
Bernd Gansbacher	Alternate	Clinicians' Representative	No interests declared	
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	
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	No interests declared	
Andreea Barbu	Expert	Sweden	No interests declared	

Jolien de Groot	Expert	Netherlands	No interests declared	
Lucas Mevius	Expert	Netherlands	No interests declared	
Tilo Moede	Expert	Sweden	No interests declared	
Ingrid Bijsmans	Expert	Netherlands	No interests declared	
Sanne ten Dam	Expert	Netherlands	No interests declared	
Viktoriia Starokozhko	Expert	Netherlands	No restrictions applicable to this meeting	
Sonia Roldan-Munoz	Expert	Netherlands	No interests declared	
Lies van Vlijmen	Expert	Netherlands	No interests declared	
Odoardo Olimpieri	Expert	Italy	No interests declared	
Antonella Isgrò	Expert	Italy	No interests declared	
Johan Sällström	Expert	Sweden	No restrictions applicable to this meeting	
Alex Mihail Simion	Expert	Belgium	No interests declared	
Attila Sebe	Expert	Germany	No interests declared	
Dagmar Pospíšilová	Expert	Czechia	No interests declared	
Matthias Renner	Expert	Germany	No restrictions applicable to this meeting	
Andras Donaszi-Ivanov	Observer	Hungary	No restrictions applicable to this 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](#)

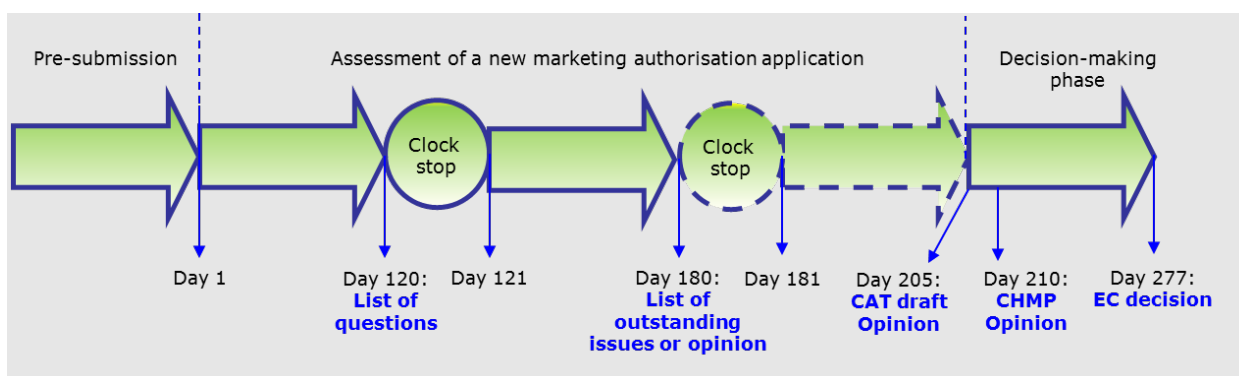
Evaluation of ATMPs (section 2)

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/