9 October 2024
EMA/CAT/533443/2024
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes for the meeting on 11-12 September 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 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.

The Chair welcomed the new member and alternate.

1.2. Adoption of agenda

CAT agenda for 11-13 September 2024 meeting was adopted.

1.3. Adoption of the minutes

CAT minutes for 17-20 July 2024 meeting were adopted.

CAT minutes for 14-16 August 2024 via written procedure 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

2.5.1. Delandistrogene moxeparvovec - Orphan - EMEA/H/C/005293

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

Scope: Day 80 assessment report

Action: for information

The information was noted.

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

Accelerated assessment

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

Scope: Day 80 assessment report

Action: for information

The information was noted.

2.6. Update on ongoing initial applications

Mozafancogene autotemcel – PRIME – Orphan - EMEA/H/C/005537Rocket Pharmaceuticals; Treatment of paediatric patients with Fanconi anaemia type A

Scope: Request for clock stop extension

Action: for information

CAT discussed the request for clock stop extension. CAT agreed on a clock-stop extension.

2.6.1. 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: Request for clock stop extension

Action: for information

CAT discussed the request for clock stop extension. CAT agreed on a clock-stop extension .

2.7. New applications

2.8. Withdrawal of initial marketing authorisation application

No items

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

No items

2.10. Companion diagnostics

2.10.1. Initial consultation

No items

2.10.2. Follow-up consultation

No items

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

2.11.1. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0047

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, opinion

Action: for adoption

The request for supplementary information adopted on 19.07.2024, 24.05.2024.

The opinion was adopted.

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

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The request for supplementary information adopted on 24.05.2024.

The opinion was adopted.

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

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The request for supplementary information adopted on 21.06.2024.

The opinion was adopted.

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

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, request for additional information

Action: for adoption

The request for supplementary information was adopted.

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

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, opinion

Action: for adoption

The request for supplementary information adopted on 19.07.2024.

The opinion was adopted.

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

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Clinical

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

severe or moderately severe haemophilia B. The Annex II is updated accordingly.

Action: for adoption

The Rapporteur presented the assessment of the variation. The opinion was adopted.

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

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

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

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

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

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

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

Novartis Europharm Limited

Rapporteur: Sol Ruiz

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

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

Fondazione Telethon ETS

Rapporteur: Sol Ruiz, PRAC Rapporteur: Liana Martirosyan

Scope: Safety, request for supplementary information

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

Action: for adoption

The Rapporteur presented the outcome of the assessment (PRAC-led procedure). The request for supplementary information was adopted.

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

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Beghaus

Scope: Quality, opinion

Action: for adoption

The request for supplementary information adopted on 21.06.2024.

The opinion was adopted.

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

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

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

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

The opinion was adopted.

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

Fondazione Telethon ETS

Rapporteur: Sol Ruiz

Scope: Clinical & pharmacovigilance, opinion

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

Action: for adoption

The Rapporteur presented the outcome of the assessment. CAT agreed with the outcome of the PRAC-led assessment of the PASS interim report.

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

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical & Pharmacovigilance

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

Action: for adoption

The Rapporteur presented the outcome of the assessment. CAT agreed with the outcome of the PRAC-led assessment of the interim safety report.

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

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Annual renewal, request for supplementary information

Action: for adoption

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

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

Pierre Fabre Medicament

CAT Rapporteur: Egbert Flory, PRAC Rapporteur: Gabriele Maurer

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

Action: for information (PRAC led procedure)

The Rapporteur presented the outcome of the assessment (PRAC-led procedure). CAT agreed with the outcome of the PRAC-led assessment of the protocol update.

2.14. GMP and GCP inspections requests

No items

3. Certification of ATMPs

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

3.1. Opinion

No items

3.2. Day 60 Evaluation Reports

No items

3.3. New Applications

4. Scientific Recommendation on Classification of ATMPs

Timetable:

-Start of the procedure: 13.09.2024

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

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Human allogeneic cardiosphere-derived cells

Treatment of muscular dystrophy

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

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

Treatment of chronic and refractory ulcers

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.2. Day 30 ATMP scientific recommendation

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

Treatment of cancers in adults

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. The CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 04.10.2024.

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

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

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. The CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 04.10.2024.

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

Treatment of haemophilia B

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. The CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 04.10.2024.

4.2.4. hiPSC derived Ovarian Support Cells (OSCs)

For ex vivo maturation of human oocytes

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. The procedural clock has been stopped, awaiting the receipt of the additional information from the applicant.

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

No items

4.4. Finalisation of procedure

4.4.1. Spermatogonial stem cells, propagated in vitro

Male infertility due to gonadotoxic treatment

Scope: ATMP scientific recommendation

Action: for information

The classification report was adopted. The product does fulfil the definition of a tissue engineered medicinal product as provided in Article 2(1) of Regulation (EC) No. 1394/2007. After consultation with the EC, a statement was added in relation to the new regulation on substances of human origin (SoHO), which covers substances for medically assisted reproduction. This recommendation of CAT is without prejudice to the decision on the scope of the pharmaceutical and SoHO legislative frameworks, which is not under the remit of CAT.

4.5. Follow-up and guidance

No items

5. Scientific Advice

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

5.1. New requests - appointment of CAT Rapporteurs

5.1.1. Ongoing scientific advice procedures - Appointment of CAT Peer Reviewers

Timetable:

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

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

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

5.2. Procedures discussed at SAWP – 1st reports, D40 JRs, LoIs

5.3. Finalisation of D70 procedures – feedback from the discussion meeting

No items

5.4. Final Advice Letters for procedures finalised the previous month

No items

6. Pre-Authorisation Activities

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6.1. Paediatric investigation plans

No items

6.2. ITF briefing meetings in the field of ATMPs

No items

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:

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

No items

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

No items

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

The Chair welcomed András Donászi-Ivanov, as new member for Hungary, and Radka Nejezchlebová, as the new alternate for Czechia.

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

CAT: Andras Donaszi-Ivanov, Viola Bardóczy

Scope: Draft agenda of the upcoming SRLM

Action: for discussion

The draft agenda of the upcoming CAT SRLM was presented. Members were invited to propose topics. It was noted that additional topics could be added following the CAT

Scientific Symposium on 10 October 2024.

7.1.3. Committee meetings in TEAMS and new tool for voting

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

Action: for information

Webex polls, used till now in Webex meetings for online voting, is to be discontinued on 30 September 2024. Decisions, an app from Microsoft Teams, was approved as the new online voting tool; the new voting tool to be used for virtual Committee meetings starting 1 October 2024.

The committee was also informed of the timelines for WebEx discontinuation and transition to Microsoft Teams as a new platform for running Committee meetings. In the following period, several training and practice run sessions will be organised by EMA Secretariat in order to make sure the CAT members and experts have the right access to the new platform before switching completely to Microsoft Teams starting 4 November 2024 (CAT November 2024 plenary meeting).

7.1.4. CAT deadlines around the end of year holiday period

CAT: Emmely de Vries, Tineke van den Hoorn

Scope: Challenges for NCAs with the timetables of centralised procedures during the end-of-year period and the consequential pressure put on assessment teams

Action: For discussion

Emmely de Vries (NL) shared with CAT the topic on the deadlines around the end-of-year holiday period. Overall, the members were in agreement with the initiative from the NL on trying to find solution to reduce the end-of-year workload. There was no specific comment from the members.

7.2. Coordination with EMA Scientific Committees

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

Scope: Agenda and Minutes

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

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

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

Action: for information

The information was noted.

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

No items

7.4. Cooperation with the EU regulatory network

7.4.1. EDQM webinar

CAT: Catherine Milne

Scope: Advancements in gene therapy: the European Pharmacopoeia's new approach webinar on 03.12.2024 from 14.00-16.00.

Catherine Milne provided information on the upcoming EDQM webinar. Participants are invited to submit their questions in advance using the online question form.

7.5. Cooperation with international regulators

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

CAT: Ilona Reischl

Scope: Agenda of the teleconference of 19.09.2024

Action: for information

The agenda for the upcoming teleconference was shared.

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

CAT: Petr Soukup, Ilona Reischl

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

Action: for information

The agenda of the upcoming multi-stakeholder meeting in Rome on 15 November 2024 was shared.

Petr Soukup presented a draft agenda for an upcoming ATMP Clinical Trial Webinar. It was considered that this webinar might be more difficult to support, as there are already a lot of CAT activities ongoing. Further discussions are needed.

7.6. CAT work plan

7.6.1. CAT symposium – 10.10.2024, Amsterdam, the Netherlands

Scope: Programme and practical information

Action: for information

The EMA Secretariat presented the plan for the upcoming CAT Scientific Symposium.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. ISCT Europe

CAT: Ilona Reischl

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

Action: for information

Feedback was provided from the ISCT Europe 2024 Regional meeting (Gothenburg, Sweden; 04-06.09.2024).

8. Any other business

No items

Date of next CAT meeting:

9-11 October 2024

9. List of participants

List of participants including any restrictions with respect to involvement of members/alternates/ experts following evaluation of declared interests for the 11-13 September meeting, which was held remotely.

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	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	
Petr Soukup	Member	Czechia	No interests declared	
Radka Nejezchlebová	Alternate	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	
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	
Andras Donaszi-Ivanov	Member	Hungary	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 restrictions applicable to this meeting	
Una Riekstina	Member	Latvia	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 Kjeken	Member	Norway	Cannot act as rapporteur, other leading/co-ordinating role or peer reviewer	5.1.1.5
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	
Margareta Fogelová	Alternate	Slovakia	No interests declared	
Suzana Vidic	Alternate	Slovenia	No restrictions applicable to this meeting	
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:	2.5.1.
Charlotte Anderberg	Alternate	Sweden	No interests declared	
Bernd Gansbacher	Alternate	Clinicians' Representative	No interests declared	
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	
Alessandra Renieri	Alternate	Clinicians' Representative	No restrictions applicable to this meeting	
Kerstin Sollerbrant Melefors	Member	Patients' Representative	No interests declared	

Mencia de Lemus Belmonte	Alternate	Patients' Representative	No interests declared	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No interests declared	
Catherine Milne	Observer/ Alternate	EDQM	No interests declared	
Beate Mosl	Expert	Germany	No interests declared	
Sabine Weisheit-Vattekar	Expert	Norway	No interests declared	
Elina Rönnemaa	Expert	Sweden	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

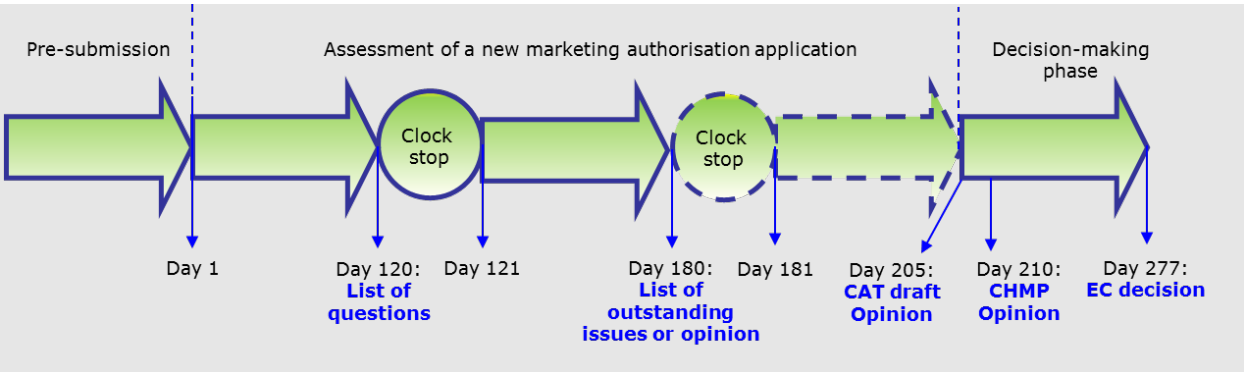
Evaluation of ATMPs (section 2)

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/