6 September 2023
EMA/CAT/421568/2023
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 12-14 July 2023

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

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

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

Note on access to documents

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

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1. Introduction

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

The Chairperson opened the meeting by welcoming all participants. The meeting was held in-person with some members connected 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 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 Chair welcomed the new members from the Netherlands and Sweden and the new alternate from Sweden.

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

1.2. Adoption of agenda

The CAT agenda for 12-14 July 2023 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for 14-17 June 2023 meeting were adopted. The Rapporteur provided an oral feedback from the discussion in the CHMP of the Abecma variation II/31 (see minutes of the June 2023 CAT meeting, agenda item 2.11.1).

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

2.8. 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/0034

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, Opinion

Action: for adoption

Request for supplementary information adopted on 16.06.2023.

The opinion was adopted.

2.11.2. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0021

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, Request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.3. Carvykti - ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0018

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, Request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.4. Carvykti - ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0019

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.5. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0069

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 15.06.2023 and 17.05.2023.

The opinion was adopted.

2.11.6. Libmeldy - atidarsagene autotemcel - Orphan - EMEA/H/C/005321/II/0017

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Netherlands

Scope: Quality, Opinion

Action: for adoption

The opinion was adopted.

2.11.7. Zolgensma - onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/II/0040

Novartis Europharm Limited

Rapporteur: Netherlands

Scope: Safety, Request for supplementary information

Update of sections 4.4 and 5.1 of the SmPC in order to add a new warning and precaution capturing the theoretical risk of tumorigenicity as a result of vector integration and to include a new statement indicating random instances of vector integration are possible, based on final results from studies 2220205 and 2220117, and literature. The Package Leaflet is updated accordingly. The RMP version 3 has also been submitted.

Action: for adoption

CAT was informed of the outcome of the PRAC discussion on this procedure. The request for supplementary information was adopted.

2.11.8. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0037/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, Opinion

Action: for adoption

The opinion was adopted on 17 July 2023 (written procedure).

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/REC/014

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality

Action: for adoption

The assessment of this quality recommendation was adopted.

2.13.2. Roctavian - valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/MEA/003.1

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Safety

MAH's response to MEA 003 [Impact of BMN 270 on fertility, general toxicity, teratology, and germline transmission in females of childbearing potential] as adopted in February 2023.

Action: for adoption

The Rapporteur presented the assessment of this recommendation. The questions to the MAH were discussed and updated in line with the discussion. The request for supplementary information was adopted.

2.13.3. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/013.1

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical

MAH’s Response to REC 013 [Study KT-US-482-0147 (ZUMA-26): to study and analyse the impact of tumour CD19 expression on response to treatment using a quantitative flow cytometry method. A phase II open label, multicentre study exploring the efficacy of axicabtagene ciloleucel and its association with the CD19 expression profile in large B-cell lymphoma in subjects whose disease has relapsed 12 or more months after first-line chemoimmunotherapy] as adopted in February 2023

Action: for adoption

The Rapporteur presented the assessment of the revised protocol for the proposed study

The revised protocol is considered acceptable. CAT agreed with the conclusion of the Rapporteur.

2.13.4. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/PSA/S/0102.2

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus; PRAC Rapporteur: Marie Louise Schougaard Christiansen

Scope: Substantial amendment to a protocol for a long-term, non-interventional study of recipients of Yescarta for treatment of relapsed or refractory Diffuse Large B-cell Lymphoma and Primary Mediastinal B-cell Lymphoma [MAH's further response to PSA/S/0102]: feedback from the PRAC discussion

Action: for information

EMA provided feedback from the PRAC discussion on the proposal from the MAH to include additional data sources to study the long-term follow-up of Yescarta. A request for supplementary information was adopted by PRAC; the updated protocol needs to be submitted by Kite Pharma.

2.14. GMP and GCP inspections requests

No items

3. Certification of ATMPs

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

3.1. Opinion

No items

3.2. Day 60 Evaluation Reports

No items

3.3. New Applications

No items

4. Scientific Recommendation on Classification of ATMPs

Deadline for submission of new requests: 27.07.2023. New requests will appear in the agenda of the August 2023 CAT meeting.

Timetable:

-Start of the procedure: 11.08.2023

-EMA Coordinator's draft report:	22.08.2023
-CAT Coordinator's comments:	30.08.2023
-Revised scientific recommendation:	01.09.2023
-CAT's discussion of scientific recommendation:	08.09.2023

4.1. New requests – Appointment of CAT Coordinator

No items

4.2. Day 30 ATMP scientific recommendation

4.2.1. Neonatal human dermal fibroblast (nHDF) cell-produced living extracellular vascular tissue

For regeneration, repair, or replacement of damaged blood vessels in patients with end-stage renal disease (ESRD), needing arterial bypass and with vascular trauma

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 31.07.2023.

4.2.2. Bovine collagen membrane seeded with allogeneic mesenchymal stem cells derived from adipose tissue (ADSC)

Treatment of patients who are undergoing a surgical procedure of coronary artery bypass grafting and have ischemic left ventricular dysfunction

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 31.07.2023.

4.2.3. Lymphocytic Choriomeningitis Virus (LCMV) reassortant strain exerting efficient anti-tumoral activity

Treatment of metastatic solid cancers

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 31.07.2023.

4.2.4. Human Cardiomyocytes (CM), Human Stromal Cells (StC)

Treatment of heart failure

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 31.07.2023.

4.2.5. Doruxapapogenum ralaplasmidum (pGX3024), DNA plasmid encoding E6 and E7 proteins of HPV6 and HPV11

Treatment of recurrent respiratory papillomatosis

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 31.07.2023.

4.2.6. TERT Ribonucleoprotein

Treatment of cancer

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 31.07.2023.

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

No items

4.4. Finalisation of procedure

No items

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:	03-06.07.2023
- Appointment of CAT Peer Reviewers:	12-14.07.2023
- SAWP first reports:	21.08.2023
- CAT Peer Reviewer comments (NC/C)	25.08.2023
- CAT Peer Reviewer comments (Q)	30.08.2023
- Discussion at SAWP:	28-31.08.2023
- Discussion at CAT and feedback to SAWP:	06-08.09.2023

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	28-31.08.2023
- Appointment of CAT Peer Reviewers:	06-08.09.2023
- SAWP first reports:	18.09.2023
- CAT Peer Reviewer comments (NC/C)	22.09.2023
- CAT Peer Reviewer comments (Q)	27.09.2023
- Discussion at SAWP:	25-28.09.2023
- Discussion at CAT and feedback to SAWP:	04-06.10.2023

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

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

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:	03-06.07.2023
SAWP recommendation:	31.08.2023
CAT recommendation:	08.09.2023
CHMP adoption of report and final recommendation:	14.09.2023

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

No items

6.3.4. Ongoing support

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

Action: for information

The CAT Chair welcomed Hans Ovelgönne as the new member for the Netherlands, Maria Lüttgen as the new member for Sweden and Charlotte Anderberg as the new alternate for Sweden.

7.1.2. Vote by proxy

Action: for information

Finland gave a proxy to Austria to vote on behalf of Finland during the entire meeting.

Latvia gave a proxy to Bulgaria to vote on behalf of Latvia during the entire meeting.

Romania gave a proxy to Ireland to vote on behalf of Ireland during the entire meeting.

Luxemburg gave a proxy to France to vote on behalf of Luxemburg during the entire meeting.

7.1.3. Election of CAT Vice-chairperson

Action: for adoption

The election took place in the presence of 25 CAT members that were eligible to vote. Four proxy votes were also received. Kieran Breen was elected as CAT vice-chair for a period of 3 years. His mandate will start on 12 July 2023.

7.1.4. CAT Strategic Review & Learning meeting (SRLM) under the Spanish presidency, 25-27 October 2023 Madrid (Spain)

CAT: Sol Ruiz, Marcos Timon

Scope: Topics for discussion at the upcoming SRLM

Action: for discussion

The draft agenda of the upcoming SRLM was presented. Topics were proposed for the CAT-only session on 26 October (afternoon).

7.1.5. Meetings schedule for 2024

Scope: Information on the dates of face to face and online only meetings for 2024

Action: for information

The information was noted.

7.1.6. Feedback from the Scientific Coordination Board (SciCoBo) meeting of 23 June 2023

CAT: Ilona Reischl

Scope: Feedback from the SciCoBo meeting of 23.06.2023

Action: for information

The CAT Chair provided a short feedback from the discussions at the SciCoBo meeting.

7.1.7. CAT members' participation in drafting group, work plan and other cross-committee activities

Scope: Nomination of CAT members replacing Carla Herberts

Action: for discussion

EMA presented the excel table with CAT members' participation to drafting groups, workplan and other activities. This table is available in IRIS and CAT members were asked to check and if needed amend the table.

Following CAT members agreed to replace Carla Herberts: Jan Mueller-Berghaus for the clinical drafting group for the guideline for investigational ATMPs; Ebru Karakoc Madsen for the focus group on submission predictabilities; Maura O'Donovan for the Real-World Data / SMA study.

7.1.8. CAT Strategic Review & Learning meeting (SRLM) under the Swedish Presidency, 4 - 5 May 2023, Uppsala (Sweden)

CAT: Maria Lüttgen

Scope: Minutes of the CAT-COMP SRLM

Action: for information

The minutes were noted.

7.1.9. CAT members participation and voting

Action: For information

The information was noted.

7.2. Coordination with EMA Scientific Committees

No items

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

No items

7.4. Cooperation with the EU regulatory network

No items

7.5. Cooperation with international regulators

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

CAT: Ilona Reischl

Scope: Update on the teleconference that took place on 22.06.2023

Action: for information

A short feedback was provided from the discussions at the ATMP cluster teleconference.

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

CAT: Ilona Reischl

Scope: Agenda of the teleconference that took place on 14.09.2023

Action: for information

CAT members were asked to provide suggested agenda items for the September ATMP cluster TC.

7.6. CAT work plan

7.6.1. CAT workplan 2023

Scope: Half-year review

Action: for discussion

The half-year review of the CAT workplan was presented and discussed.

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

CAT topic leads: Ilona Reischl, Rune Kjeken, Claire Beuneu, Alessandro Aiuti

Scope: Feedback from drafting group meeting

Action: for discussion

Feedback was provided from the drafting group meeting that was held on 13.07.2023 (afternoon) during which mainly the comments received on the quality part were discussed.

Discussion and reflections are needed to further align the quality, non-clinical and clinical parts, to revisit the introductory sections and to update the references. Depending on the level and nature of changes introduced, CAT will have to decide if a second (short) public consultation is needed.

CAT members were asked to review and comment on the revised non-clinical and clinical parts of the guideline; the quality part, revised after the drafting group of 13.07.23 and is also open for comments. Deadline for comments: 28.08.2023.

7.7. Planning and reporting

7.7.1. EMA-FDA Q&A on PRIME/Breakthrough Therapies

QIG chair: Marcel Hoefnagel

Scope: Final EMA-FDA Q&A on PRIME/Breakthrough Therapies

Action: for adoption

The Quality Innovation Group (QIG) Chair presented the final version of the EMA-FDA Q&A on PRIME/Breakthrough Therapies. This Q&A is based on the outcome of the EMA/FDA stakeholder workshop on quality development in early access approaches, such as PRIME and Breakthrough Therapies that was held in November 2018 and complements the EMA Toolbox guidance on scientific elements and regulatory tools to support quality data packages for PRIME and certain marketing authorisation applications targeting an unmet medical need - Scientific guideline | European Medicines Agency (europa.eu)

The Q&A was adopted on 18 July 2023 via a written procedure.

7.7.2. Quality Innovation Group (QIG)

QIG chair: Marcel Hoefnagel

Scope: Update from the chair of the Quality Innovation Group (QIG)

Action: for information

The QIG Chair introduced the scope of the QIG (as entry point for quality/technical innovations), its activities and the priority topics for 2023. CAT can propose topics for consideration for the priority topics for 2024.

7.8. Others

7.8.1. Webinar – EU Network awareness session on Companion Diagnostics (CDx)

Scope: CDx Awareness session – 14 July, 11.00-13.00

Action: for information

Topics:

- case studies relating to companion diagnostics (CDx), including both co-developed and follow-on cases within the context of the consultation procedure by the Notified Bodies with the medicines regulators;
- lessons learned from analytical/clinical performance to form an opinion on the suitability of CDx.

Note: Interested CAT member can join this awareness session in person. Registration is required (deadline for registration: 13 July 2023)

7.8.2. Academic ATMP Pilot

Scope: Status overview of the pilot and draft Executive Director Decision on Fee reduction for Academic ATMP Pilot

Action: for discussion

EMA presented the status of the Academic ATMP pilot and the proposal for fee reductions for the selected candidates for the pilot. CAT agreed with the proposed fee reductions for the pilot product. CAT was informed that discussions are ongoing on fee incentives for academia outside to the current ATMP pilot.

CAT will be kept informed of the products that will be selected and will flag if a product is part of the pilot whenever it comes for scientific advice (SA) or paediatric investigation plan (PIP).

More information on the Academic ATMP pilot can be found here.

7.8.3. CASSS Cell and Gene Therapy Products symposium

CAT: Ilona Reischl

Scope: Feedback from the CASSS symposium held in Bethesda (USA) on 27-29.06.2023

Action: for information

The CAT Chair gave a short feedback from the CASSS meeting.

7.8.4. 10th Industry Stakeholder platform on Research and Development support

CAT: Ilona Reischl

Scope: Feedback from the meeting topic on scientific advice for medicinal product developments comprising of drug-device combinations and drug-companion diagnostic combinations

Action: for information

The CAT Chair gave a short feedback from the 10th Industry Stakeholder platform on Research and Development support, focussing mainly on the provision of scientific advice for drug-device combinations and drug-companion diagnostic combinations.

8. Any other business

No items

Date of next CAT meeting:

09-11 Augustus 2023 (written procedure only)

06-08 September 2023

9. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 12-14 July 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	
Silke Dorner	Member	Austria	No interests declared	
Olga Kholmanskikh	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Azra Selimovic	Member	Croatia	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Rafaella Pontou	Member	Cyprus	No interests declared	
Kristyna Rehorova Hradilkova	Alternate	Czechia	No interests declared	
Ebru Karakoc Madsen	Member	Denmark	No interests declared	
Toivo Maimets	Member	Estonia	No interests declared	
Pille Saalik	Alternate	Estonia	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	
Balázs Sarkadi	Alternate	Hungary	No restrictions applicable to this meeting	
Maura O'Donovan	Member	Ireland	No interests declared	
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	
Hans Ovelgönne	Member	Netherlands	No interests declared	
Rune Kjeken	Member	Norway	No restrictions applicable to this meeting	
Dariusz Sladowski	Member	Poland	No restrictions applicable to this meeting	
Maria Isabel Borba Vieira	Alternate (to CHMP representative)	Portugal	No interests declared	
Katarina Vavrová	Member	Slovakia	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Margareta Fogelová	Alternate	Slovakia	No interests declared	
Suzana Vidic	Alternate	Slovenia	No participation in final deliberations and voting on:	2.11.5. Kymriah II/69 2.11.7. Zolgensma II/40
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	
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	
Alessandro Aiuti	Member	Clinicians' Representative	No participation in final deliberations and voting on:	2.11.6. Libmeldy II/17
Kerstin Sollerbrant Melefors	Member	Patients' Representative	No interests declared	
Mencia de Lemus Belmonte	Alternate	Patients' Representative	No restrictions applicable to this meeting	
Kieran Breen	Member	Patients' Representative	No interests declared	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Torbjorn Callreus	Expert	Malta	No interests declared	
Marcel Hoefnagel	Expert	Netherlands	No interests declared	
Ivana Haunerova	Expert	Czech Republic	No interests declared	
Andreea Barbu	Expert	Sweden	No interests declared	
Attila Sebe	Expert	Germany	No interests declared	
Gabriela Ullio-Gamboa	Expert	France	No interests declared	
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

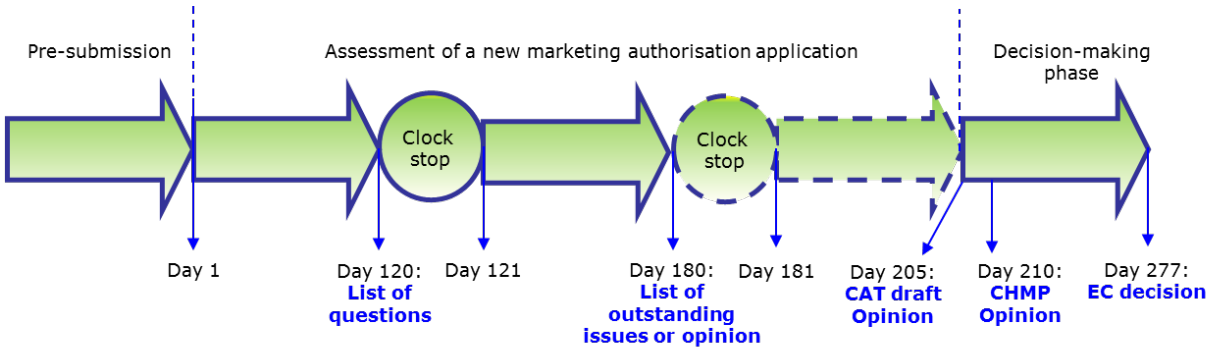
Evaluation of ATMPs (section 2)

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/