



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

13 March 2024
EMA/CAT/185591/2024
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 14-16 February 2024

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 ongoing 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 connect 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 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 14-16 February 2024 meeting was adopted with one addition to section 2.13: Abecma - Idecabtagene vicleucel - EMEA/H/C/004662/REC/019.1.

1.3. Adoption of the minutes

The CAT minutes for 17-20 January 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

No items

2.5. Day 80 assessment reports

2.5.1. Beremagene geperpavec - PRIME - Orphan - EMEA/H/C/006330

Krystal Biotech Netherlands B.V.; Treatment of patients from birth with dystrophic epidermolysis bullosa (DEB) with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene

Scope: Day 80 assessment report

Action: for information

The information was noted.

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. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/II/0032

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 08.12.2023.

The opinion was adopted.

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

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Clinical, Request for supplementary information

Grouped application comprising two variations as follows:

C.I.4 – Update of sections 4.4 and 4.8 of the SmPC in order to add immune effector cell-associated neurotoxicity syndrome (ICANS) as an adverse drug reaction (ADR) based on the cumulative review of MAH safety database and literature. The Package Leaflet is updated accordingly. In addition, the MAH took this opportunity to introduce editorial changes.

A.6 – To include the ATC Code L01XL08 in section 5.1 of the SmPC.

Action: for adoption

CAT noted the assessment from the Rapporteur on occurrence of ICANS in clinical trials or reported as ADRs. The request for supplementary information was adopted.

2.11.3. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0021

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Jo Robays

Scope: Clinical, opinion

Extension of indication to include treatment of adult patients with relapsed and refractory multiple myeloma, who have received at least 1 prior therapy, including an IMiD and a PI, have demonstrated disease progression on or after the last therapy and are refractory to lenalidomide for CARVYKTI, based on interim results from study MMY3002 listed as a specific obligation (SOB/006) in the Annex II. This is an ongoing, Phase 3, randomized, open-label, multicentre study to determine whether treatment with cilta-cel provides an efficacy benefit compared to standard therapy in participants with relapsed and lenalidomide-refractory multiple myeloma. As a consequence, sections 4.1, 4.4, 4.5, 4.8,

5.1 and 5.2 of the SmPC are updated. The Package Leaflet is updated in accordance. Version 4.1 of the RMP has also been submitted. In addition, the MAH took the opportunity to update Annex II of the PI. As part of the application the MAH is requesting a 1-year extension of the market protection.

Action: for adoption

Request for supplementary information adopted on 08.12.2023, 08.09.2023.

Lothar Bergman, the chair of the scientific advisory group (SAG) oncology, presented the responses from the SAG experts to the questions asked by the CAT. CAT noted the SAG output.

The Rapporteur presented the assessment of the responses by the MAH. Based on final assessment and responses provided by the SAG, CAT agreed that the benefit risk of Breyanzi is positive for the extension of indication. CAT discussed the wording of the sections 4.4 and 5.1 of the SmPC.

A positive opinion was adopted by CAT. CAT also concluded that the specific obligation is considered fulfilled, therefore the conditional marketing authorisation (MA) can be converted to a full Marketing Authorisation. The specific obligation is converted in a recommendation for the MAH to provide the final study report of study MMY3002.

2.11.4. Kymriah - Tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0071

Novartis Europharm Limited

Rapporteur: Rune Kjekken

Scope: Clinical, Opinion

Update of sections 5.1 and 5.2 of the SmPC in order to update efficacy and pharmacokinetic information based on final results from study CCTL019B2202 (a phase II, single arm, multicentre trial to determine the efficacy and safety of CTL019 in paediatric patients with relapsed and refractory B-cell acute lymphoblastic leukaemia). Submission of cellular kinetic report for the B-cell acute lymphoblastic leukaemia (ALL) indication based on data from pivotal study CCTL019B2202 and the supportive study CCTL019B2205J involving paediatric ALL patients (partially fulfil REC).

In addition, the MAH took this opportunity to introduce editorial changes.

Action: for adoption

Request for supplementary information adopted on 31.10.2023.

The Rapporteur presented the assessment of the responses by the MAH. The changes to the SmPC were agreed. The opinion was adopted.

2.11.5. Kymriah - Tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0075

Novartis Europharm Limited

Rapporteur: Rune Kjekken, PRAC Rapporteur: Gabriele Maurer

Scope: Clinical, opinion

Update of sections 5.1 and 5.2 of the SmPC in order to update efficacy and pharmacokinetic

information based on final results from study CCTL019C2201 PAES in the Annex II (ANX008); this is a Phase II, single arm, multicentre trial to determine the efficacy and safety of CTL019 in adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL). The RMP version 6 has also been submitted. In addition, the MAH took the opportunity to update Annex II.D of the PI.

Action: for adoption

Request for supplementary information adopted on 31.10.2023.

The Rapporteur presented the assessment of the responses by the MAH. The changes to the SmPC were agreed. The opinion was adopted.

2.11.6. Kymriah - Tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0079/G

Novartis Europharm Limited

Rapporteur: Rune Kjekken

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.7. Upstaza - Eladocagene exuparvovec - Orphan - EMEA/H/C/005352/II/0013

PTC Therapeutics International Limited

Rapporteur: Joseph DeCoursey

Scope: Quality, opinion

To submit the final report with the results of the active substance and finished product concurrent process validation batches, including hold time data for the finished product batch, in order to further assess process consistency and maintain patient's safety (Annex II condition).

Action: for adoption

Request for supplementary information adopted on 19.01.2024, 08.09.2023.

The Rapporteur presented the assessment of the responses by the MAH. The opinion was adopted.

2.11.8. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2500

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, Request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/017.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjekken

Scope: Quality, Request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.13.2. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/018.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjekken

Scope: Quality, fulfilled

Action: for adoption

The report was adopted.

2.13.3. Upstaza - Eladocagene exuparvec - Orphan - EMEA/H/C/005352/S/0017

PTC Therapeutics International Limited

Rapporteur: Joseph DeCoursey

Scope: Annual Re-assessment, opinion

Action: for adoption

Request for supplementary information adopted on 08.12.2023.

Further to the adoption of variation II/13, CAT adopted a positive opinion for the annual re-assessment.

2.13.4. Zolgensma - Onasemnogene abeparvec - Orphan - EMEA/H/C/004750/P46/022

Novartis Europharm Limited

Rapporteur: Emmely de Vries

Scope: Clinical, Request for supplementary information

Paediatric studies submitted in accordance with Article 46 of Regulation (EC) No1901/2006, as amended. Final study report COAV101A12306: Phase IIIb, open-label, single-arm, single-dose, multicentre study evaluating the safety, tolerability, and efficacy of gene

replacement therapy with intravenous OAV101 (AVXS-101) in paediatric patients with spinal muscular atrophy (SMA).

Action: for adoption

The Rapporteur presented the assessment of the P46 procedure (final results of a paediatric clinical trial in older and heavier children). The changes to the SmPC section 4.4 and 4.8 were already implemented as part of the post authorisation procedure. A request for supplementary information was adopted.

2.13.5. Luxturna - Voretigene neparvovec - EMEA/H/C/PSUSA/00010742/202307

Novartis Europharm Limited

Rapporteur: Gabriele Maurer

Scope: PRAC recommendation

Action: for adoption

The PRAC recommendation was adopted.

2.13.6. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/019.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjekken

Scope: Quality, fulfilled

Action: for adoption

The report 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

No items

3.3. New Applications

No items

4. Scientific Recommendation on Classification of ATMPs

Timetable:

-Start of the procedure:	19.02.2024
-EMA Coordinator's draft report:	01.03.2024
-CAT Coordinator's comments:	06.03.2024
-Revised scientific recommendation:	08.03.2024
-CAT's discussion of scientific recommendation:	15.03.2024

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Allogeneic human induced pluripotent stem cells-derived corneal limbal stem cells

For treatment of limbal stem cell deficiency

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Olfactory glial cells isolated from autologous human olfactory bulb, expanded in culture

For treatment of complete spinal cord injuries

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Circular RNA capable to bind to mutated regions of the messenger RNA from the DMPK gene

For treatment of myotonic dystrophy type 1

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. Modified measles vaccine virus

For the treatment of solid cancer tumours

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 01.03.2024.

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

No items

4.4. Finalisation of procedure

4.4.1. Allogeneic expanded natural killer cells

For the treatment of acute myeloid leukaemia

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

4.4.2. Autologous tissue generated in the human body (in vivo) through the foreign body reaction

For tissue augmentation

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

4.4.3. Dendritic cells activated by lysate of circulating tumour cells

For the treatment of solid tumours in metastatic stage

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

4.4.4. Autologous T Lymphocytes engineered with nanoparticles with curcumin encapsulated

For the treatment of melanoma
Scope: European Commission raised no comments. ATMP

scientific recommendation

Action: for adoption

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

4.4.5. Spermatogonial stem cells, propagated *in vitro*

Male infertility due to gonadotoxic treatment

Scope: Procedure on hold pending input/comments from the European Commission

Action: for information

The information was noted.

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:	05-08.02.2024
- Appointment of CAT Peer Reviewers:	14-16.02.2024
- SAWP first reports:	26.02.2024
- CAT Peer Reviewer comments (NC/C):	01.03.2024
- CAT Peer Reviewer comments (Q):	06.02.2024
- Discussion at SAWP:	04-07.03.2024
- Discussion at CAT and feedback to SAWP:	13-15.03.2024

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	04-07.03.2024
- Appointment of CAT Peer Reviewers:	13-15.03.2024
- SAWP first reports:	02.04.2024
- CAT Peer Reviewer comments (NC/C):	05.04.2024
- CAT Peer Reviewer comments (Q):	10.04.2024
- Discussion at SAWP:	08-11.04.2024
- Discussion at CAT and feedback to SAWP:	17-19.04.2024

No items

- 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

6.2.1. Overview of ITF activities in 2023

ITF Coordinator: Oriane Blanquie

Scope: Summary of main activities conducted by ITF in 2023

Action: for information

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:

Procedure start:	05-08.02.2024
SAWP recommendation:	07.03.2024
CAT recommendation:	15.03.2024
CHMP adoption of report and final recommendation:	21.03.2024

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

Action: for information

None

7.1.2. Vote by proxy

Action: for information

Nancy de Bremaeker (Luxembourg) gave a proxy to Claire Beuneu (Belgium) to vote on behalf of Nancy de Bremaeker during the entire meeting.

7.1.3. CAT Strategic Review & Learning meeting (SRLM) under the Belgian presidency – 15-17 May 2024

CAT: Claire Beuneu

Scope: Draft agenda of the upcoming SRLM

Action: for discussion

The draft agenda of the upcoming CAT SRLM and CAT-PDCO joint SRLM was presented.

7.1.4. CAT Strategic Review & Learning meeting (SRLM) under the Spanish presidency – 26-27 October 2023

CAT: Sol Ruiz, Marcos Timon

Scope: Final minutes of the SRLM meeting

Action: for information

The final minutes of the SRLM meeting were noted.

7.2. Coordination with EMA Scientific Committees

7.2.1. Minutes and draft agenda - PCWP and HCPWP meetings

Scope: Minutes and draft agenda for the PCWP and HCPWP meetings

Action: for information

The minutes and draft agenda for the PCWP and HCPWP meetings of 27-28 February 2024 were noted.

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

7.3.1. ATMP Quality assessment support group

Scope: To inform the CAT members on the creation of the group of ATMP Quality experts to be contacted to give informal advice and act as sounding board for member states in relation to assessment of quality aspects of ATMPs

Action: for information

EMA provided information on the ATMP quality assessment support group and on the procedure to contact the members of this group on quality related assessment questions.

7.3.2. Launch of Call for Nominations to Biological Quality European Specialised Expert Community (ESEC)

Scope: Mandate and rules of procedure

Action: for adoption

EMA presented the mandate and rules of biological quality European Specialised Expert Community (ESEC). The mandate and rules of procedure were adopted.

CAT members with quality expertise will be automatically included in this group, unless if they want to opt out.

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: Agenda of the teleconference of 22.02.2024

Action: for information

The agenda of the upcoming ATMP cluster was noted.

7.5.2. International Pharmaceutical Regulatory Programme (IPRP) Gene and Cell therapy working group

CAT: Pille Säälük

Scope: Agenda of the teleconference of 27.02.2024

Action: for information

The agenda of the upcoming IPRP Gene and Cell therapy working group was noted.

7.6. CAT work plan

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

CAT: Ilona Reischl

Scope: Draft guideline, updated following the external consultation

Action: for adoption for release for external consultation

EMA presented the status of the guideline: comments from member states and EDQM were received on the guideline, which are being discussed by the drafting group members. The pre-final guideline will now be sent to the clinical trial coordination group (CTCG) for consultation. Member states/CAT members can provide final comments by the same deadline. The guideline will be adopted by CAT members via written procedure and will be presented at CHMP PROM March 2024 meeting for adoption. This will allow publication on the external website in the March 2024, for a short second public consultation until end of May 2024.

7.6.2. CAT Scientific symposium, 8-9 October 2024

CAT Chair: Ilona Reischl

Scope: draft programme

Action: for discussion

EMA provided a short update on the activities to be performed in drafting the programme.

7.6.3. CAT - regulatory session at the European Society of Gene & Cell Therapy (ESGCT) 2024 meeting

CAT Chair: Ilona Reischl

Scope: To discuss topics on CAT and EMA activities to support developers, progress and challenges in ATMP development for CAT session on ATMP regulatory aspects at the ESGCT meeting that takes place on 22-25 October 2024 in Rome, Italy

Action: for discussion

CAT agreed to hold a regulatory session at the ESGCT annual meeting. T

7.7. Planning and reporting

No items

7.8. Others

7.8.1. Report from Group for Internal Rules on Extensions of Clock Stops

CAT: Olga Kholmanskikh

Scope: Report on outcomes and proposals to the CHMP. Comments to be sent by 29 February 2024

Action: for discussion

EMA presented the outcome and proposals that came out of the discussion in the Group for Internal Rules on Extensions of Clock Stops. The proposals were agreed. CAT indicated that it is important that the stricter application of the clock stop rules is communicated externally to the medicines' developers.

7.8.2. ATMP Support Pilot for academia

Scope: Update and information on selected candidates

Action: for information

EMA provided an update on the two additional candidate products that were selected for the pilot. Next steps were presented.

7.8.3. CoGenT (Collaboration on Gene Therapies) Pilot

Scope: Internal collaboration of regulatory authorities to facilitate the development of gene therapies for orphan diseases

Action: for information

EMA presented to CAT the CoGenT pilot that has been initiated by FDA. This would be a global collaboration of ICH founding members on scientific advice and evaluation of orphan gene therapies. The way of interactions (when and how) still needs to be defined.

CAT asked for the Rapporteurs of products to be included in the pilot to be consulted in advance.

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

CAT: Ilona Reischl

Scope: Invitation to attend the 2nd Annual Global Regulators Summit (28 May 2024) and the panel discussion on Regulatory Considerations for Platform-based Cell and Gene Therapy (29 May 2024)

Action: for agreement

CAT agreed for Pille Säälük to attend the ISCT meeting to represent the CAT.

8. Any other business

8.1.1. IRIS training

Scope: Training on the use of IRIS and the new implementation of communication process between CAT Secretariat and Committee participants

Action: for information

EMA provided a follow-up training to CAT on the use of IRIS.

Date of next CAT meeting:

13-15 March 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 February 2024 CAT meeting, which was held in-person.

<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	
Olga Kholmanskikh	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Azra Selimovic	Member	Croatia	No interests declared	
Petra Sokol	Alternate	Croatia	No interests declared	
Rafaella Pontou	Member	Cyprus	No interests declared	
Kristyna Rehorova Hradilkova	Alternate	Czechia	No interests declared	
Ebru Karakoc Madsen	Member	Denmark	No restrictions applicable to this meeting	
Bibi Fatima Syed Shah	Alternate	Denmark	No interests declared	
Toivo Maimets	Member	Estonia	No interests declared	
Pille Saalik	Alternate	Estonia	No interests declared	
Heli Suila	Member	Finland	No interests declared	
Violaine Closson Carella	Member	France	No interests declared	

<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>
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	
Anna Katalin Barane Gilicze	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	
Richard Carroll	Alternate	Ireland	No interests declared	
Concetta Quintarelli	Member	Italy	No interests declared	
Barbara Bonamassa	Alternate	Italy	No restrictions applicable to this meeting	
Una Riekstina	Member	Latvia	No interests declared	
Villma Perikaite	Member (CHMP member)	Lithuania	No restrictions applicable to this meeting	
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 Kjekken	Member	Norway	No restrictions	

<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>
			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	
Margareta Fogelová	Alternate	Slovakia	No interests declared	
Suzana Vidic	Alternate	Slovenia	No participation in final deliberations and voting on:	2.11.4; 2.11.5 Kymriah 2.13.4 Zolgensma
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	
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 restrictions applicable to this meeting	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No interests declared	
Federica Chiara	Alternate	Patients' Representative	No restrictions applicable to this meeting	
Torjorn Callreus	Expert	Malta	No interests declared	
Lothar Bergmann	Guest	Germany	No restrictions applicable to this 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>
Odoardo Maria Olimpieri	Expert	Italy	No interests declared	
Antonella Isgro	Expert	Italy	No interests declared	
Federico de Angelis	Expert	Italy	No interests declared	
Joao Manuel Lopez de Oliveira	Expert	Portugal	No interests declared with information under any other interests	
Angelo Ferreira da Silva	Expert	Portugal	No interests declared	
Attila Sebe	Expert	Germany	No interests declared	

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](#)

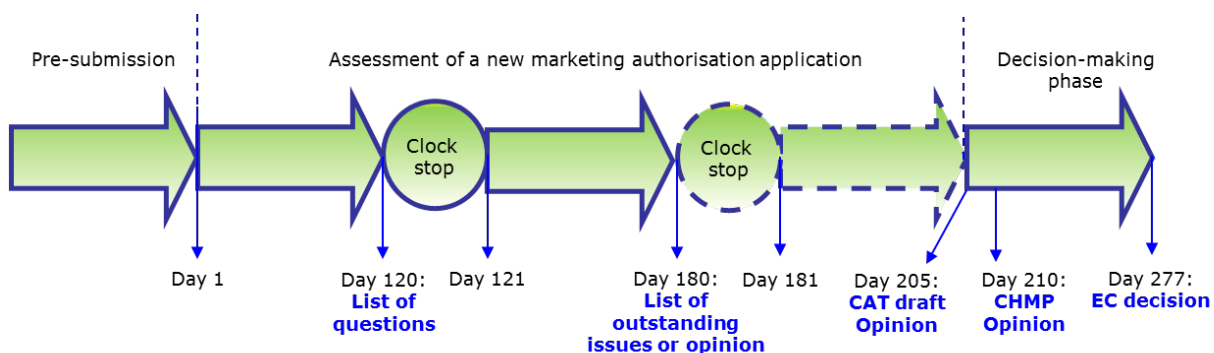
Evaluation of ATMPs (section 2)

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/