26 April 2024
EMA/CAT/279597/2024
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 17-19 April 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 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 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 members and alternates and thanked the departing members/alternates for their contributions to the Committee.

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 17-19 April 2024 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for 13-16 March 2024 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

2.4.1. Autologous cartilage-derived articular chondrocytes, in-vitro expanded - EMEA/H/C/004594

Repair of symptomatic, localised, full-thickness cartilage defects of the knee joint grade III or IV

Scope: Day 120 list of questions

Action: for adoption

The rapporteurs presented the assessment of the application. Feedback was provided from the Biologics Working Party (BWP) discussion.

The list of questions was adopted.

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

2.6.1. Fidanacogene elaparvovec - PRIME - EMEA/H/C/004774

Indicated for the treatment of severe and moderately severe haemophilia B

Scope: Third party intervention

Action: for information

The information was noted.

List of questions adopted on 08.09.2023.

2.7. New applications

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

Rocket Pharmaceuticals B.V.; Treatment of paediatric patients with Fanconi Anaemia Type A

Scope: Timetable for assessment

Action: for adoption

The timetable for assessment was adopted.

2.7.2. 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: Timetable for assessment

Action: for adoption

The timetable for assessment was adopted.

2.8. Withdrawal of initial marketing authorisation application

No items

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

No items

2.10. 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. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0023

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, Opinion

Action: for adoption

Request for supplementary information adopted on 19.01.2024 and 06.10.2023.

The opinion was adopted.

2.11.2. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/II/0010

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Safety

Submission of the final report from study BMN270-302 listed as a category 3 study in the RMP. This is a phase 3 open-label, single-arm study to evaluate the efficacy and safety of BMN 270, an adeno-associated virus vector–mediated gene transfer of human factor VIII at a dose of 4E13 vg/kg in haemophilia A patients with residual FVIII levels ≤ 1 IU/dL receiving prophylactic FVIII infusions.

Action: for adoption

The opinion was adopted.

2.11.3. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2607

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 19.01.2024.

The opinion was adopted.

2.11.4. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2632

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Karin Erneholm

Scope: Safety & Clinical, opinion

Update of section 4.2 of the SmPC in order to update the safety monitoring timelines based on data from clinical studies, post-marketing studies and literature. The Package Leaflet is updated accordingly. In addition, the MAH took the opportunity to implement editorial changes to other sections of the SmPC to align the language across both products.

Action: for adoption

The opinion was adopted. CAT noted the difference in the SmPCs of CAR T products regarding the duration of patient monitoring after treatment.

2.11.5. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0072

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality

Action: for information

Note: Withdrawal request received on 14 March 2024.

The information was noted.

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/020

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, Request for supplementary information, fulfilled

Action: for adoption

The report was adopted.

2.13.2. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/REC/014.1

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

The report was adopted.

2.13.3. Ebvallo - Tabelecleucel - Orphan - EMEA/H/C/004577/REC/007

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, opinion

Action: for adoption

The report was adopted.

2.13.4. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/SOB/006.1

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Clinical & Pharmacovigilance, opinion

MAH Response to SOB 006 as adopted in January 2024: Study 270-303 1-Year CSR - A

Phase 3b, Single Arm, Open-Label Study to Evaluate the Efficacy and Safety of BMN 270, an Adeno-Associated Virus Vector–Mediated Gene Transfer of Human Factor VIII, with Prophylactic Corticosteroids in Haemophilia A Patients.

Action: for adoption

The report was adopted. The MAH is requested to submit a variation in order to update the product information to reflect the inadequacy of the prophylactic use of corticosteroids.

2.13.5. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/ANX/002.3

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical & Pharmacovigilance

From initial MAA:
PAES Study No. KTE-EU-472-6036: First Annual Report - Long-term, non-interventional study of recipients of Tecartus for treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL)

Action: for adoption

The report was adopted.

2.13.6. Zolgensma - Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/P46/022

Novartis Europharm Limited

Rapporteur: Emmely de Vries

Scope: Clinical

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

Request for supplementary information adopted on 16.02.2024.

The report was adopted.

2.13.7. Abecma - idecabtagene vicleucel; Breyanzi - lisocabtagene maraleucel; Carvykti - ciltabtagene autoleucel; Kymriah - tisagenlecleucel; Tecartus - brexucabtagene autoleucel; Yescarta - axicabtagene ciloleucel

Bristol-Myers Squibb Pharma EEIG (Abecma, Breyanzi), Janssen-Cilag International NV (Carvykti), Novartis Europharm Limited (Kymriah), Kite Pharma EU B.V. (Tecartus, Yescarta)

CAT Rapporteurs: Rune Kjeken (Kymriah, Abecma), Jan Mueller-Berghaus (Carvykti, Tecartus, Yescarta), Concetta Quintarelli (Breyanzi)

PRAC Rapporteur (for the signal): Ulla Wändel Liminga

Scope: Feedback from PRAC discussion on signal of secondary malignancies of T-cell origin (EPITT 20040)

Action: for discussion

The PRAC rapporteur provided feedback from the PRAC discussion. The PRAC request for supplementary (RSI) was presented.

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:	22.04.2024
-EMA Coordinator's draft report:	08.05.2024
-CAT Coordinator's comments:	15.05.2024
-Revised scientific recommendation:	17.05.2024
-CAT's discussion of scientific recommendation:	24.05.2024

4.1. New requests – Appointment of CAT Coordinator

4.1.1. MicroRNA against BCL2 anti-apoptotic messenger RNA

Treatment of cancer patients

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Allogeneic natural killer cells expanded in vitro and transfected to express modified Fas ligand

Treatment of haematological malignancies and glioblastoma

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Stromal vascular fraction

Treatment of osteoarthritis

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.4. Implantable device 3D bioprinted with autologous microfat and hydrogel bioink

Treatment of Breast reconstruction, soft tissue repair

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. mRNA encoding ARCUS nuclease

Treatment of chronic hepatitis B (CHB) virus infection

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 03.05.2024.

4.2.2. Allogeneic human corneal endothelial cells (neltependocel) and a low molecular weight Rho kinase inhibitor (Y-27632)

Treatment of corneal oedema due to corneal endothelial 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 03.05.2024.

4.2.3. Lymphocyte concentrate

Improvement of the pregnancy outcomes among women with unexplained repeated pregnancy loss and HLA sharing among partners

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the classification report. CAT considered that the information provided was not sufficient to conclude on the ATMP classification: additional information is requested from the applicant.

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

No items

4.4. Finalisation of procedure

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

Treatment of limbal stem cell deficiency

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

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

Treatment of complete spinal cord injuries

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

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

Treatment of myotonic dystrophy type 1

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

4.5. 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:	08-11.04.2024
- Appointment of CAT Peer Reviewers:	17-19.04.2024
- SAWP first reports:	06.05.2024
- CAT Peer Reviewer comments (NC/C):	10.05.2024
- CAT Peer Reviewer comments (Q):	15.05.2024
- Discussion at SAWP:	13-16.05.2024
- Discussion at CAT and feedback to SAWP:	22-24.05.2024

5.1.2. 5.1.1.1. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	13-16.05.2024
- Appointment of CAT Peer Reviewers:	22-24.05.2024
- SAWP first reports:	03.06.2024
- CAT Peer Reviewer comments (NC/C):	07.06.2024
- CAT Peer Reiveiwer comments (Q):	12.06.2024
- Discussion at SAWP:	10-13.06.2024
- Discussion at CAT and feedback to SAWP:	19-21.06.2024

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

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:	08-11.04.2024
SAWP recommendation:	16.05.2024
CAT recommendation:	24.05.2024
CHMP adoption of report and final recommendation:	30.05.2024

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

No items

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

The Chair welcomed Ole Henrik Myrdal as the new alternate for Norway. The Chair thanked Marid Hystad for her contribution as alternate for Norway.

The Chair also thanked Ebru Karakoc Madsen for her contribution as member for Denmark.

7.1.2. Vote by proxy

Azra Selimovic (Croatia) gave a proxy vote to Isavella Kyriakidou (Cyprus) to vote on her behalf during the entire meeting.

Suzana Vidic (Slovenia) gave a proxy vote to Isabel Vieira (Portugal) to vote on her behalf 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 agenda of the upcoming CAT and CAT-PDCO SRLMs were presented.

CAT members were asked to make sure that they register for the meeting, also when attending remotely.

7.2. Coordination with EMA Scientific Committees

7.2.1. Paediatric procedures on IRIS platform

Scope: Inform the Committee about the progress on paediatric procedures that will be onboarded on IRIS on 4 June 2024

Action: for information

EMA informed CAT that by early June 2024 all paediatric procedures will move to IRIS. PEDRA will remain active, but new procedures will no longer be added (these can be found in IRIS).

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

7.3.1. Question and Answer on decentralised manufacturing

Scope: Presentation of the draft Question and Answers (prepared by Quality Innovation Group)

Action: for discussion

EMA presented the Question and Answer (Q&A) document on how decentralised manufacturing can be applied in the current legal framework. The Q&A is meant to apply to ATMPs or other types of products and is planned to be published after finalisation. There was a short discussion on how far this would apply to SoHO (as starting materials for ATMPs). The Q&A relates to GMP manufacturing activities only.

CAT members can provide comment to the document by 30 April 2024 .

7.3.2. Joint CAT-SAWP membership

Scope: Appointment of joint CAT-SAWP member.

Action: for agreement

Further to the call for interest, CAT nominated the following CAT member as joint CAT-SAWP member: Olga Kholmanskikh.

A call for interest for the alternate CAT-SAWP member will be launched soon (following the departure of Ebru Karakoc Madsen).

7.3.3. BWP/CAT training on Quality aspects of AAV based gene therapy medicinal products (ATMPs)

Scope: Agenda of the training to take place on 31 May 2024

Action: for awareness

The agenda of the training on AAV-based gene therapies was noted.

7.4. Cooperation with the EU regulatory network

7.4.1. European Pharmacopoeia text on cell-based preparations for human use

CAT: Violaine Closson Carella

Scope: To inform the committee of the public consultation in Pharmeuropa on the European Pharmacopoeia text (5.32) on cell-based preparations for human use

Action: for information

CAT noted the ongoing work on the European Pharmacopoeia text on cell-based preparations of human use. The document is published in Pharmeuropa and is open for comments.

CAT questioned how this text on cell-based preparations for human use is aligned to EU guidelines of cell-based ATMPs and the EDQM Tissue and Cell guide.

CAT was informed that some other documents are open for comments: pharmacopoeia text on mRNA vaccines and on high throughput sequencing.

7.4.2. European Pharmacopoeia texts on gene therapy adopted at the European Pharmacopoeia Commission session in March 2024

CAT: Catherine Milne

Scope: Adopted European Pharmacopoeia texts on gene therapy

Action: for information

CAT was informed that the Pharmacopoeia commission adopted the general monograph and the general text on gene therapy. Both documents will come into force on 01.04.2025.

There was a short exchange how CAT members could best contribute to the work of the European Pharmacopoeia Commission to develop general texts and monographs for ATMPs.

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 25.04.2024

Action: for information

The agenda of the next ATMP cluster teleconference was presented and CAT members' contribution to the different topics were identified.

7.6. CAT work plan

7.6.1. Questions and Answers on gene therapy: revision

CAT: Ilona Reischl

Scope: Feedback from the first drafting group meeting; plan of actions to identify additional questions

Action: for discussion

CAT was informed of the start of the revision of the Question and Answer (Q&A) document. CAT members were asked to provide new topics/questions to be included in the Q&A that are not yet sufficiently addressed in guidelines and reflection papers. This will allow for a discussion on the Q&A at the upcoming CAT-SRLM (see 7.1.3). Deadline for input: 13.05.2024.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. EMA lecture on 'Gene editing and its relevance for innovative treatments'

Scope: Lecture by Lluis Montoliu Ph.D., a CSIC Research Scientist and Deputy Director at the National Centre for Biotechnology in Madrid

Action: for information

The information was noted.

7.8.2. Real World Evidence, including DARWIN EU®

Scope: Quarterly update on Real World Evidence (RWE), including DARWIN EU

Action: For information

EMA provided the quarterly update including an update on DARWIN EU, potential new research questions and introduction to the Real-World Academy (a new knowledge sharing event series directed at assessors and EMA product leads), and feedback from the joint HMA/EMA workshop on registries.

8. Any other business

Date of next CAT meeting:

22-24 May 2024

9. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 17-19 April 2024 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	
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	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	
Petr Soukup	Member	Czechia	No interests declared	
Kristyna Rehorova Hradilkova	Alternate	Czechia	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	
Violaine Closson Carella	Member	France	No interests declared	
Jean-Michel Race	Alternate	France	No interests declared	

Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Maria Gazouli	Member	Greece	No interests declared	
Angeliki Rompoti	Alternate	Greece	No restrictions applicable to this meeting	
Viola Bardoczy	Alternate	Hungary	No restrictions applicable to this meeting	
Joseph De Courcey	Member	Ireland	No interests declared	
Richard Carroll	Alternate	Ireland	No interests declared	
Concetta Quintarelli	Member	Italy	No interests declared	
Barbara Bonamassa	Alternate	Italy	No interests declared	
Villma Perikaite	Member (CHMP member)	Lithuania	No interests declared	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No interests declared	
Alessia Pochesci	Member	Luxembourg	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 Kjeken	Member	Norway	Cannot act as rapporteur, other leading/co-ordinating role or peer reviewer for:	3.3.1
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 interests declared	
Katarina Kollarova	Member	Slovakia	No interests declared	
Margareta Fogelová	Alternate	Slovakia	No interests declared	
Suzana Vidic	Alternate	Slovenia	No participation in final deliberations and voting on:	2.11.5. Kymriah II/72 2.13.6. Zolgensma P46/022 2.13.7. Kymriah
Sol Ruiz	Member (CHMP co-opted member)	Spain	No interests declared	
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	
Maria Luttgen	Member	Sweden	No restrictions applicable to this meeting	
Charlotte Anderberg	Alternate	Sweden	No interests declared	
Bernd Gansbacher	Alternate	Clinicians' Representative	No interests declared	
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	
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	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Torjorn Callreus	Expert	Malta	No interests declared	
Ulla Wandel Liminga	Expert	Sweden	No interests declared	
Andreea Barbu	Expert	Sweden	No interests declared	

Peter Lönn	Expert	Sweden	No restrictions applicable to this meeting	
Alejandro Rodriguez	Expert	Sweden	No restrictions applicable to this meeting	
Karin Mathold	Expert	Sweden	No interests declared	
Karin Bolin	Expert	Sweden	No interests declared	
Adriana Ammassari	Expert	Italy	No interests declared	
Federico De Angelis	Expert	Italy	No interests declared	
Beate Mosl	Expert	Germany	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

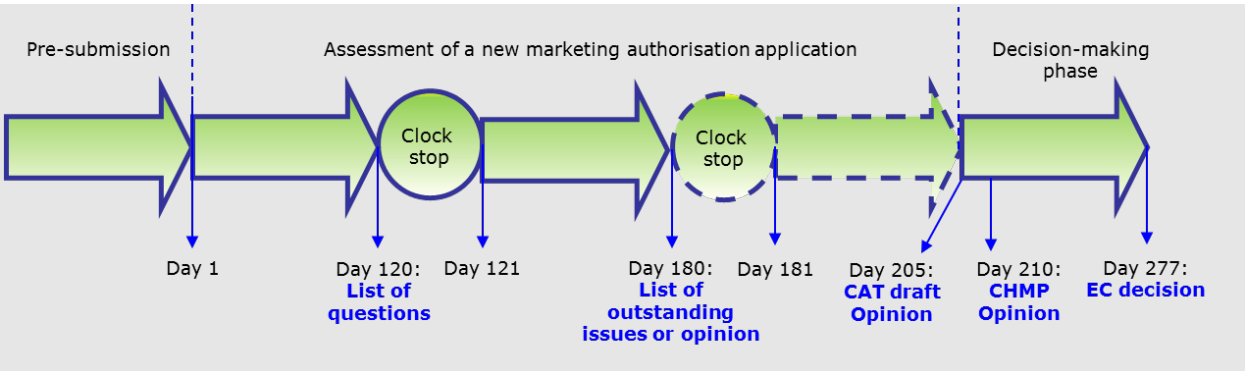
Evaluation of ATMPs (section 2)

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/