15 June 2023
EMA/CAT/368362/2023
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 15-17 May 2023

Chair: Ilona Reischl; Vice-Chair: Carla Herberts

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 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.

1.2. Adoption of agenda

The CAT agenda for 15-17 May 2023 meeting was adopted with one addition to section 7.5.

1.3. Adoption of the minutes

The CAT minutes for 19-22 April 2023 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. Exagamglogene autotemcel - PRIME - Orphan - EMEA/H/C/005763

Vertex Pharmaceuticals (Ireland) Limited; Treatment of transfusion-dependent β-thalassemia and sickle cell disease

Scope: Day 120 list of questions

Action: for adoption

The Rapporteurs presented the outcome of the initial assessment. On the quality part of the application, feedback from the BWP discussion was provided.

The list of questions was adopted.

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

No items

2.7. New applications

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

2.10.1.1. In vitro diagnostic medical device - EMEA/H/D/006255

Indicated as an aid in the selection of adult haemophilia A patients for whom valoctocogene roxaparvovec treatment is being considered

Scope: Opinion

Action: for adoption

The Rapporteur presented the assessment of the responses to the request for

supplementary information. The Rapporteur concluded that the clinical suitability of in vitro diagnostic medical device is confirmed to test for patients negative for anti-AAV5.

CAT adopted a positive opinion for this procedure.

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/0032/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, Request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.2. Alofisel - darvadstrocel - Orphan - EMEA/H/C/004258/II/0044/G

Takeda Pharma A/S

Rapporteur: Lisbeth Barkholt

Scope: Safety, Request for supplementary information

Grouped application comprising one type II variation and two type IB as follows:
- Update of section 4.8 of the SmPC in order to update the summary of the safety profile and to add anal abscess, proctalgia and anal fistula to the list of adverse drug reactions on post-marketing experience following the assessment of R/0036 based on a review of the MAH's Global Safety Database.
- Update of section 4.2 of the SmPC in order to add the term perilesional as an EDQM term, following the assessment of R/0036.
- Update of sections 1, 2.2, 3, 4.2, 6.5 and 6.6 of the SmPC in order to replace the term 'suspension for injection' for 'dispersion for injection', following the assessment of R/0036.
The Annex A, Package Leaflet and Labelling are updated in accordance.

Action: for adoption

The request for supplementary information was adopted.

2.11.3. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/II/0062/G

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Quality

Action: for adoption

The opinion was adopted.

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

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality, Request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.5. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0057

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, Opinion

Action: for adoption

Request for supplementary information adopted on 20.01.2023 and 24.03.2023.

The opinion was adopted.

2.11.6. Tecartus; Yescarta - axicabtagene ciloleucel; brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2389/G

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, Opinion

Action: for adoption

Request for supplementary information adopted on 17.02.2023.

The opinion was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. CARVYKTI - ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/REC/011.1

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

The report was adopted.

2.13.2. CARVYKTI - ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/REC/012.1

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

The report was adopted.

2.13.3. Hemgenix - etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/REC/002

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality

Action: for adoption

The report was adopted.

2.13.4. Hemgenix - etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/REC/003

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, Recommendation fulfilled

Action: for adoption

The report was adopted.

2.13.5. Hemgenix - etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/SOB/004

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Clinical

Protocol and statistical analysis plan of study CSL222_4001: An observational post-authorisation long-term follow-up study to characterise the effectiveness and safety of Hemgenix (etranacogene dezaparvovec) in patients with haemophilia B.

Action: for adoption

The report was adopted.

2.13.6. Luxturna - voretigene neparvovec - Orphan - EMEA/H/C/004451/R/0040

Novartis Europharm Limited

Rapporteur: Sol Ruiz, Co-Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Gabriele Maurer

Scope: 5-year renewal of marketing authorisation, Opinion

Action: for adoption

CAT discussed the PRAC recommendation to grant renewal for 5 years. A second renewal is considered justified . CAT discussed the need for a second renewal and agreed with PRAC. It was noted that this should not be considered as a precedent for the renewal of ATMPs/gene therapies.

The renewal of the marketing authorisation for a period of 5 years was adopted.

2.13.7. ROCTAVIAN - valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/R/0003

BioMarin International Limited

Rapporteur: Violaine Closson Carella, Co-Rapporteur: Silke Dorner, PRAC Rapporteur: Menno van der Elst

Scope: 1-year renewal of marketing authorisation

Action: for adoption

Request for supplementary information adopted on 21.04.2023.

The 1-year renewal of the marketing authorisation was adopted.

2.13.8. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/R/0056

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Claire Beuneu, PRAC Rapporteur: Anette Kirstine Stark

Scope: 5-year renewal of marketing authorisation, Opinion

Action: for adoption

Request for supplementary information adopted on 17.02.2023.

The Rapporteur presented the evaluation of the responses to the request for supplementary

information.

The 5-year renewal 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

4.1. New requests – Appointment of CAT Coordinator

No items

4.2. Day 30 ATMP scientific recommendation

4.2.1. Living human adult allogeneic immunomodulatory progenitor (iMP) cells

Treatment of myocardial scarring

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 31.05.2023.

4.2.2. Allogeneic viable natural killer (NK) cells CD56+ CD3-

Treatment of patients with acute myeloid leukaemia (AML) who are in morphologic complete remission (CR) and for whom allogeneic haematopoietic stem cell transplantation (allo-HSCT) is not a suitable or preferred option

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 31.05.2023.

4.2.3. Recombinant Adeno-associated virus serotype 9 vector containing the human-lysosome-associated membrane glycoprotein 2 isoform B transgene

Treatment of Danon disease

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 31.05.2023.

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

No items

4.4. Finalisation of procedure

4.4.1. Ixoberogene soroparvovec (Genetically engineered, replication-incompetent adeno-associated virus vector comprising the AAV.7m8 capsid proteins, carrying a version of complementary deoxyribonucleic acid for aflibercept)

Treatment of neovascular (wet) age-related macular degeneration

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.

4.4.2. Ex vivo fused allogeneic human myoblasts (MB-N) with autologous human myoblast (MB-ALS)

Treatment of amyotrophic lateral sclerosis (ALS)

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. Ex vivo fused allogeneic human myoblasts (MB-N) with autologous human bone marrow derived mesenchymal stem cells (MSC-ALS)

Treatment of amyotrophic lateral sclerosis (ALS)

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.

4.4.4. Ex vivo fused allogeneic human mesenchymal stem cell (MSC-N) with autologous human myoblast (MB-ALS)

Treatment of amyotrophic lateral sclerosis (ALS)

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.

4.4.5. Ex vivo fused allogeneic human myoblasts (MB-N1) with allogeneic human myoblasts (MB-N2)

Treatment of amyotrophic lateral sclerosis (ALS)

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.

4.4.6. Helper-dependent adenovirus vector coding for interleukin-1 receptor antagonist

Treatment of osteoarthritis of the knee

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.

4.4.7. Autologous CD34+ cells from mobilised peripheral blood

Treatment of amyotrophic lateral sclerosis (ALS)

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

4.4.8. Biotinylated cultured reticulocytes, cultured from haematopoietic stem cells

Treatment of red cell suppletion (e.g. trauma/anaemia)

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

4.4.9. Autologous chondrocytes cultured in hyaluronan-derived scaffold

Repair of cartilage defects

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

CAT discussed the updated classification report. The product does fulfil the definition of a tissue engineered 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:	8-12.05.2023
- Appointment of CAT Peer Reviewers:	15-17.05.2023
- SAWP first reports:	30.05.2023
- CAT Peer Reviewer comments (NC/C):	02.06.2023
- CAT Peer Reviewer comments (Q):	07.06.2023
- Discussion at SAWP:	05.08.2023
- Discussion at CAT and feedback to SAWP:	14-16.06.2023

5.1.2. Scientific advice procedures starting at the next SAWP meeting

No items

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

No items

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

The Chair thanked Lisbeth Barkholt, for whom this is the last CAT meeting she is attending, for her support to the work of the CAT.

7.1.2. Vote by proxy

Action: for information

7.1.3. CAT Strategic Review & Learning meeting (SRLM) under the Swedish presidency, 4 and 5 May 2023, Uppsala (Sweden)

CAT: Lisbeth Barkholt, Maria Lüttgen

Scope: Feedback from the SRLM

Action: for discussion

Lisbeth Barkholt provided a high level feedback from the discussion at the SRLM. Minutes are prepared from the meeting, which will be presented to the CAT at their June or July meeting.

7.2. Coordination with EMA Scientific Committees

7.2.1. Co-Rapporteur Day 95 Assessment

Scope: Application of the Co-Rapporteur Day 90 assessment to ATMP marketing authorisation applications (MAAs)

Action: for discussion

CAT noted the proposal to extend the procedure to develop a single overview document with the assessment of both Rapporteur and Co-Rapporteur to ATMPs: this procedure is already successfully implemented at CHMP for non-ATMP and non-Covid-19 products for 2 years. CAT agreed to implement this procedure as a pilot for the upcoming ATMP MAAs.

Feedback on CAT experience can be shared with EMA secretariat

7.2.2. Format of oral explanations (OEs) during CAT meetings

Scope: New arrangement and format of the OEs during the CAT plenary meetings in order to enhance the experience for companies to be as close as possible to the face-to-face setting

Action: for information

CAT noted the information: the new arrangement will be tested out at CHMP for OE from June 2023 onwards.

7.2.3. Minutes and draft agenda - PCWP and HCPWP meetings

Scope: Minutes and draft agenda for the patient and consumer working party (PCWP) and healthcare professional working party (HCPWP) meetings

Action: for information

The information was noted.

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

No items

7.4. Cooperation with the EU regulatory network

No items

7.5. Cooperation with international regulators

7.5.1. Workshop on ICH E6 R3 July 2023

Scope: Announcement of the public consultation of ACT EU PA04 - Multi-stakeholder Workshop on ICH E6 R3

Action: for information

The information was noted.

7.5.2. European Pharmacopoeia general monograph on gene therapy medicinal products for human use

CAT: Barbara Bonamassa

Scope: clearance of vector particle use to genetically modify cells

Action: for discussion

CAT discussed with the European Directorate for the Quality of Medicines & Health Care (EDQM) representative how to ensure that the general monograph on gene therapies that is under development is aligned to the scientific guideline and the regulatory experience with the approval of GTMP based on genetically manipulated cells. The proposed wording on clearance of viral particle used to genetically modify cells would, unintentionally, include additional stricter requirements than what is currently required for these products and would not be in line with the overarching risk-based approach principle for ATMPs.

7.6. CAT work plan

7.6.1. CAT stakeholders meeting 2023

CAT: Dariusz Sladowski, Ilona Reischl, Violaine Closson Carella, Carla Herberts

Scope: Agenda of the CAT stakeholders meeting

Action: for information

The CAT stakeholder meeting took place on 16.05.2023 from 15.00 to 18.00.

7.7. Planning and reporting

7.7.1. Business Pipeline Report – 3-year Forecast report

Action: for information

CAT noted the information in the 3-year forecast report on the ATMP marketing authorisation applications that are expected until December 2025. Members were asked to present this to their national agencies to ensure that sufficient resources will be available to evaluate these products.

7.8. Others

7.8.1. Onboarding Program for CAT members and alternates

CAT: Carla Herberts

Scope: To discuss the creation and further updating of an onboarding programme for CAT members and alternates

Action: for discussion

CAT noted the onboarding programme that is developed for CHMP members and agreed that this is also very relevant for CAT members. Some issues specific to ATMPs and CAT procedures will have to be included. The following CAT members agreed to be involved in this activity: Mencia de Lemus Belmonte, Jan Mueller-Berghaus, Ebru Karakoc Madsen and Ilona Reischl.

8. Any other business

No items

Date of next CAT meeting:

14-16 June 2023

9. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 15-17 May 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	
Corina Spreitzer	Alternate	Austria	No restrictions applicable to this meeting	
Claire Beuneu	Member	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Petra Sokol	Alternate	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	
Ebru Karakoc Madsen	Member	Denmark	No interests declared	
Bibi Fatima Syed Shah	Alternate	Denmark	No interests declared	
Toivo Maimets	Member	Estonia	No interests declared	
Pille Saalik	Alternate	Estonia	No interests declared	
Heli Suila	Member	Finland	No interests declared	
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	
Balázs Sarkadi	Alternate	Hungary	No restrictions applicable to this meeting	
Maura O'Donovan	Member	Ireland	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Niamh Curran	Alternate	Ireland	No restrictions applicable to this meeting	
Concetta Quintarelli	Member	Italy	No interests declared	
Barbara Bonamassa	Alternate	Italy	No restrictions applicable to this meeting	
Una Riekstina	Member	Latvia	No interests declared	
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	
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	
Carla Herberts	Member (Vice-Chair)	Netherlands	No interests declared	
Babs Fabriek	Alternate	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	
Margareta Fogelová	Alternate	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	
Sol Ruiz	Member (CHMP co-opted member)	Spain	No interests declared	
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	
Lisbeth Barkholt	Member	Sweden	No interests declared	
Maria Luttgen	Alternate	Sweden	No restrictions applicable to this meeting	
Alessandro Aiuti	Member	Clinicians' Representative	No restrictions applicable to this meeting	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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	Patients' Representative	No interests declared	
Federica Chiara	Alternate	Patients' Representative	No restrictions applicable to this meeting	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Hans Ovelgönne	Expert - via telephone*	Netherlands	No interests declared	
Torbjorn Callreus	Expert - via telephone*	Malta	No interests declared	
Antonella Isgrò	Expert - via telephone*	Italy	No interests declared	
Federico de Angelis	Expert - via telephone*	Italy	No interests declared	
Juliane Rau	Expert - via telephone*	Germany	No interests declared	
Atilla Sebe	Expert - via telephone*	Germany	No interests declared	
Brigitte Anliker	Expert - via telephone*	Germany	No interests declared	
Silke Schüle	Expert - via telephone*	Germany	No interests declared	
Matthias Renner	Expert - via telephone*	Germany	No restrictions applicable to this meeting	
Liam Childs	Expert - via telephone*	Germany	No interests declared	
Hanna Kankkonen	Expert - via telephone*	Finland	No interests declared	
Tia Hirvonen	Expert - via telephone*	Finland	No interests declared	
Pauliina Lehtolainen-Dalkilic	Expert - via telephone*	Finland	No interests declared	
Paula Grönroos	Expert - via telephone*	Finland	No interests declared	
Karri Penttilä	Expert - via telephone*	Finland	No interests declared	
John Aspegren	Expert - via telephone*	Finland	No restrictions applicable to this meeting	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Johanna Lähteenvuo	Expert - via telephone*	Finland	No interests declared	
Kristine Moltu	Expert - via telephone*	Norway	No interests declared	
Andreea Barbu	Expert - via telephone*	Sweden	No interests declared	
Beate Mosl	Expert - via telephone*	Germany	No restrictions applicable to this meeting	
Martina Schüßler-Lenz	Expert - via telephone*	Germany	No interests declared	
Gabriele Maurer	Expert - via telephone*	Germany	No restrictions applicable to this meeting	
Susanne Mueller	Expert - via telephone*	Germany	No interests declared	
Gabriele Ruppert-Seipp	Expert - via telephone*	Germany	No interests declared	
A representative from the European Commission attended the meeting Meeting run with support from relevant EMA staff				

10. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Abbreviations / Acronyms

AAV: Adeno-Associated Virus

AR: Assessment Report

ATMP: Advanced Therapy Medicinal Product

BWP: Biologics Working Party

CAT: Committee for Advanced Therapies

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

CTFG: Clinical Trial Facilitation Group

DG: Drafting Group

EC: European Commission

EU NTC: European Union Network Training Centre

ERA: Environmental Risk Assessment

FDA: Food and Drug Administration

FL: Final Letter

GCG: Guideline Consistency Group

GCP: Good Clinical Practice

GLP: Good Laboratory Practice

GMO: Genetically-modified organism

GMP: Good Manufacturing Practice

GTMP: Gene Therapy Medicinal Product

HTA: Health Technology Assessment Bodies

HSPC: Hematopoietic Stem and Progenitor Cells

ITF: Innovative Task Force

JR: Joint Report

LoOI: List of outstanding issues

LoQ: List of questions

MA: Marketing Authorisation

MAA: Marketing Authorisation Application

MAH: Marketing Authorisation Holder

MNAT: Multinational assessment team

MSC: Mesenchymal stem cells

PDCO: Paediatric Committee

PMDA: Pharmaceuticals and Medical Devices Agency (Japan)

PIP: Paediatric Investigation Plan

PL: Package leaflet

PRAC: Pharmacovigilance and Risk Assessment Committee #

PRIME: Priority Medicines

QRD: Quality review of documents

RMP: Risk Management Plan

RP: Reflection paper

RSI: Request for supplementary information

SAs: Scientific Advices

SAG-O: Scientific Advisory Group Oncology

SAWP: Scientific Advice Working Party

SR: Summary Report

SME: Small and medium size enterprises

SmPC: Summary of Products Characteristics

TT: Timetable

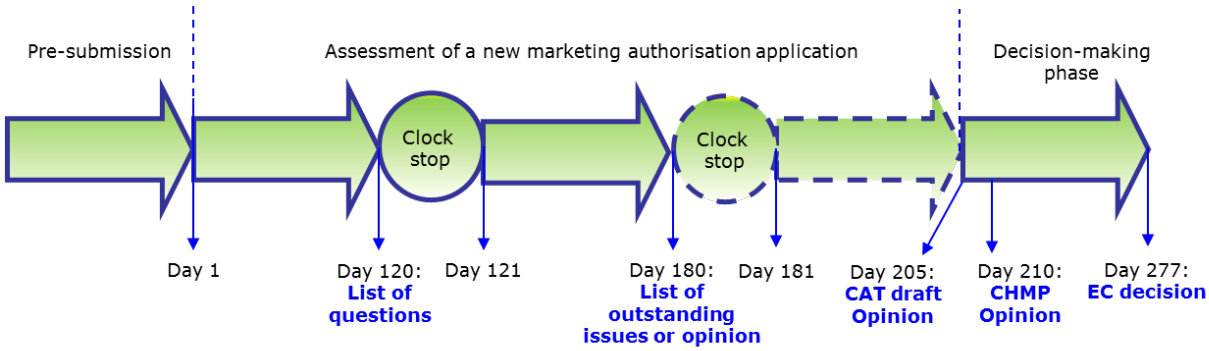
Evaluation of ATMPs (section 2)

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/