12 July 2023
EMA/CAT/367824/2023
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 15 June 2023

Chair: Ilona Reischl

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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

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1. Introduction

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

The Chairperson opened the meeting by welcoming all participants. The meeting was held remotely.

In accordance with the Agency's policy on handling of declarations of interests of scientific committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and on the topics in the agenda of the current meeting, the Committee Secretariat announced that no restriction in the involvement of meeting participants for agenda topics was identified.

Participants were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions were declared. Restrictions applicable to this meeting are captured in the List of participants included in the minutes.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The Chair welcomed the new alternate from Belgium and thanked the departing member from Netherlands for her contribution 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

CAT agenda for 14-15 June 2023 meeting was adopted with an amendment to the assessment timetable for fidanacogene elaparvovec (2.7.1) .

1.3. Adoption of the minutes

CAT minutes for 15-18 May 2023 meeting

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

No items

2.7. New applications

2.7.1. Fidanacogene elaparvovec - PRIME - Orphan - EMEA/H/C/004774

Pfizer Europe MA EEIG; Indicated for the treatment of severe and moderately severe haemophilia B

Scope: Timetable for assessment

Action: for adoption

2.8. Withdrawal of initial marketing authorisation application

No items

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

No items

2.10. Companion diagnostics

2.10.1. Initial consultation

No items

2.10.2. Follow-up consultation

No items

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

2.11.1. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0031

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken, Co-Rapporteur: Heli Suila, PRAC Rapporteur: Ulla Wändel Liminga

Scope: Clinical, request for supplementary information

Extension of indication to include treatment of adult patients with relapsed and refractory multiple myeloma (RRMM) who have received at least two prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD-38 antibody and have demonstrated disease progression on the last therapy for Abecma (idecabtagene vicleucel, ide-cel), based on results from study BB2121-MM-003 (MM-003, KarMMa-3). This is a Phase 3, multicentre, randomised, open-label study to compare the efficacy and safety of ide-cel versus standard regimens in subjects with RRMM. As a consequence, sections 2.1, 2.2, 4.1, 4.2, 4.4, 4.5, 4.8, 5.1, 5.2, 6.3, 6.4 and 6.6 of the SmPC are updated. The Package Leaflet and Labelling are updated in accordance. Version 3.0 of the RMP has also been submitted. Furthermore, the PI is brought in line with the Guideline on core SmPC, Labelling and Package Leaflet for advanced therapy medicinal products (ATMPs) containing genetically modified cells.

Action: for adoption

The rapporteur presented the assessment of the variation to extend the indication of Abecma.

The request for supplementary information was adopted on 19.06.2023 via a written procedure.

2.11.2. 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

Request for supplementary information adopted on 17.05.2023.

The opinion was adopted.

2.11.3. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0034

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.4. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0013/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, request for supplementary information

Action: for adoption

Request for supplementary information adopted on 17.02.2023.

The opinion was adopted.

2.11.5. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0018/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, request for supplementary information

Action: for adoption

The outcome of the BWP discussion was presented. The request for supplementary information was adopted.

2.11.6. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0019

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, request for supplementary information

Action: for adoption

The outcome of the BWP discussion was presented. The request for supplementary information was adopted.

2.11.7. CARVYKTI - ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/II/0016

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

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

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Quality, opinion

Action: for adoption

Request for supplementary information adopted on 17.05.2023.

The opinion was adopted.

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

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality, request for supplementary information

Action: for adoption

Request for supplementary information adopted on 17.05.2023.

The outcome of the BWP discussion was presented.

A second request for supplementary information was adopted.

2.11.10. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0070/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.11. Libmeldy - atidarsagene autotemcel - Orphan - EMEA/H/C/005321/II/0015

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Babs Fabriek

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.12. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0003

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, Clock stop extension

Action: for adoption

CAT agreed with the requested clock stop extension.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. CARVYKTI - ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/REC/013

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Quality

Action: for adoption

The report was adopted.

2.13.2. Hemgenix - etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/REC/005

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality

Action: for adoption

The report was adopted.

2.13.3. ROCTAVIAN - valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/ANX/002.1

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Clinical

MAH response to ANX 002 [Study protocol 270-601] as adopted in January 2023:

To fulfil the request of PAM, the MAH opted for a prospective longitudinal study that is observational. Most of the concerns raised in this report are related to the observational

nature of the study with regard to the examinations and tests required to achieve the objectives of the study.

Action: for adoption

The report was adopted.

2.13.4. ROCTAVIAN - valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/ANX/004.1

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Clinical

MAH response to ANX 004 [Study protocol 270-801] as adopted in January 2023:

This study is being undertaken to better characterise the long-term safety and effectiveness of Roctavian in patients in a real-world setting using periodic data extractions from registries databases to further substantiate the risk-benefit of Roctavian and to provide information on the long-term impact of treatment with Roctavian for approximately 15 years (risk of malignancy on the safety side and uncertainties on the durability on the efficacy side). This protocol is not the final version as the MAH plans to develop registry specific protocol to be added as appendices 2, 3, and 4 of the study protocol.

Action: for adoption

The report was adopted.

2.13.5. ROCTAVIAN - valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/MEA/005

BioMarin International Limited

Rapporteur: Violaine Closson Carella

Scope: Pharmacovigilance

Survey of haematologists to assess the effectiveness of the additional risk minimisation measures (aRMMs) for Roctavian (valoctocogene roxaparvovec) (Version 1.0).

Action: for adoption

This is a PRAC led procedure. The PRAC report was agreed.

2.13.6. Strimvelis - autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence - Orphan - EMEA/H/C/003854/ANX/004.4

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Sol Ruiz

Scope: Clinical and Pharmacovigilance

Third interim patient registry / STRIM-0003. Title: Adenosine deaminase severe combined immunodeficiency (ADA-SCID) registry for patients treated with Strimvelis™ (or

GSK2696273) gene therapy: long-term prospective, non-interventional follow-up of safety and effectiveness. [Interim reports submitted every 2 years.]

Action: for adoption

The report was adopted.

2.13.7. Upstaza - eladocagene exuparvovec - Orphan - EMEA/H/C/005352/REC/009

PTC Therapeutics International Limited

Rapporteur: Maura O'Donovan

Scope: Quality

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

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:	16.06.2023
-EMA Coordinator's draft report:	27.06.2023
-CAT Coordinator's comments:	30.06.2023
-Revised scientific recommendation:	05.07.2023
-CAT's discussion of scientific recommendation:	13.07.2023

4.1. New requests – Appointment of CAT Coordinator

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

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

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

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

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

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

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

Treatment of metastatic solid cancers

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

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

Treatment of heart failure

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

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

Treatment of recurrent respiratory papillomatosis

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.6. TERT Ribonucleoprotein

Treatment of cancer

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.2. Day 30 ATMP scientific recommendation

No items

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

No items

4.4. Finalisation of procedure

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

Treatment of myocardial scarring

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

The classification report was adopted. The product does fulfil the definitions of a somatic cell therapy medicinal product and a tissue engineered product and based on that is considered as a tissue engineered product as provided in Article 2(4) of Regulation (EC) No 1394/2007.

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

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

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.

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

Treatment of Danon disease

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:	05–08.06.2023
- Appointment of CAT Peer Reviewers:	14-16.06.2023
- SAWP first reports:	26.06.2023
- CAT Peer Reviewer comments (NC/C)	30.06.2023
- CAT Peer Reviewer comments (Q)	05.07.2023
- Discussion at SAWP:	03–06.07.2023
- Discussion at CAT and feedback to SAWP:	12-14.07.2023

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

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

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

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

5.4. Final Advice Letters for procedures finalised the previous month

6. Pre-Authorisation Activities

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

6.1. Paediatric investigation plans

No items

6.2. ITF briefing meetings in the field of ATMPs

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:	
Procedure start:	31.05.2023
EMA scientific officer draft report sent to SAWP reviewer:	16.06.2023
Report circulated to SAWP:	26.06.2023
SAWP recommendation:	06.07.2023
CAT recommendation (for ATMP):	14.07.2023
CHMP adoption of report and final recommendation:	20.07.2023

6.3.2. Month 1 – Discussion of eligibility

No items

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

The chair welcomed Olga Kholmanskikh as the new alternate for Belgium and thanked Carla Herberts for her contribution as the member for the Netherlands and as CAT vice-chair.

7.1.2. Vote by proxy

None

7.1.3. Election of CAT Vice-chairperson

CAT members were reminded to send in their candidatures for Vice-chairperson by 30.06.2023. Voting for Vice-chair will take place at the July CAT meeting.

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

CAT: Sol Ruiz, Marcos Timon

Sol Ruiz introduced the SRLM under the Spanish presidency and presented the draft agenda. CAT members were asked to identify additional topics for inclusion in the agenda.

The second day of the SRLM will be a join meeting with the Clinical Trial Coordination Group (CTCG).

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

CAT: Maria Lüttgen, Lisbeth Barkholt

7.2. Coordination with EMA Scientific Committees

No items

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

No items

7.4. Cooperation with the EU regulatory network

No items

7.5. Cooperation with international regulators

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

CAT: Ilona Reischl

The topics on the agenda of the upcoming ATMP cluster TC were presented.

7.6. CAT work plan

7.6.1. Report on experience with RWE studies to support EMA scientific committees

EMA presented the experience with RWE studies to support EMA scientific committees.

7.6.2. European Rare Disease Registries: a collaborative effort to assess the quality and suitability of registries to describe the natural history of disease and treatment landscape of spinal muscular atrophy

CAT: Kieran Breen, Mencia de Lemus, Lisbeth Barkholt

CAT noted the information provided on the acceptance of an abstract for the spinal muscular dystrophy (SMA) study at ICPE 2023; the objective of this study is the conduct of a fit-for-purpose (FFP) assessment of European SMA registries from the TREAT-NMD network and assess their suitability to participate in the first ever EMA-funded registry study in collaboration with CAT

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

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

A short discussion took place in preparation of the face-to-face drafting group that will be held in the margins of the July 2023 CAT meeting.

7.7. Planning and reporting

7.7.1. Business Pipeline Report – 3 year Forecast report

The information was noted.

7.8. Others

7.8.1. International Society for Gene and Cell therapy (ISCT) Paris 2023

CAT: Ilona Reischl

The CAT chair and CAT secretariat gave short feedback from the discussions and

presentations in the ISCT Global Regulators Summit and the Annual Meeting.

8. Any other business

8.1. Accessibility of PRIME reports in IRIS

EMA secretariat presented the information on the inclusion of PRIME reports in IRIS. This will be for all procedures starting in July 2023. The presentation included a short tutorial on how to access IRIS and retrieve the documents for PRIME and Scientific Advice.

Date of next CAT meeting:

12-14 July 2023

9. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 15 June 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	
Olga Kholmanskikh	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Petra Sokol	Alternate	Croatia	No interests declared	
Rafaella Pontou	Member	Cyprus	No interests declared	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	
Petr Soukup	Member	Czechia	No interests declared	
Kristyna Rehorova Hradilkova	Alternate	Czechia	No interests declared	
Ebru Karakoc Madsen	Member	Denmark	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Bibi Fatima Syed Shah	Alternate	Denmark	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	
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	
Katalin Lengyel	Member	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	
Concetta Quintarelli	Member	Italy	No interests declared	
Barbara Bonamassa	Alternate	Italy	No restrictions applicable to this meeting	
Una Riekstina	Member	Latvia	No interests declared	
Nancy De Bremaeker	Member	Luxembourg	No interests declared	
Rune Kjeken	Member	Norway	No restrictions applicable to this meeting	
Dariusz Sladowski	Member	Poland	No restrictions applicable to this meeting	
Silviu Istrate	Member	Romania	No interests declared	
Katarina Vavrová	Member	Slovakia	No interests declared	
Margareta Fogelová	Alternate	Slovakia	No interests declared	
Suzana Vidic	Alternate	Slovenia	No restrictions applicable to this meeting	
Sol Ruiz	Member (CHMP co-opted member)	Spain	No interests declared	
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Maria Luttgen	Alternate	Sweden	No restrictions applicable to this meeting	
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	
Alessandro Aiuti	Member	Clinicians' Representative	No participation in final deliberations and voting on:	Limeldy (2.11.11)
Alessandra Renieri	Alternate	Clinicians' Representative	No restrictions applicable to this meeting	
Kerstin Sollerbrant Melefors	Member	Patients' Representative	No interests declared	
Kieran Breen	Member	Patients' Representative	No interests declared	
Hans Ovelgönne	Expert	Netherlands	No interests declared	
Torbjorn Callreus	Expert	Malta	No interests declared	
Fatima Ventura	Expert	Portugal	No restrictions applicable to this meeting	
Charlotte Anderberg	Expert	Sweden	No interests declared	
Kristine Moltu	Expert	Norway	No interests declared	
Fabrice Eroukhmanoff	Expert	Norway	No interests declared	
Nina Pettersen Hessvik	Expert	Norway	No interests declared	
Karri Penttilä	Expert	Finland	No interests declared	
John Aspegren	Expert	Finland	No restrictions applicable to this meeting	
Johanna Lähteenvuo	Expert	Finland	No interests declared	
Atilla Sebe	Expert	Germany	No interests declared	
Matthias Renner	Expert	Germany	No restrictions applicable to this meeting	
Ingrid Wang	Expert	Norway	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

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

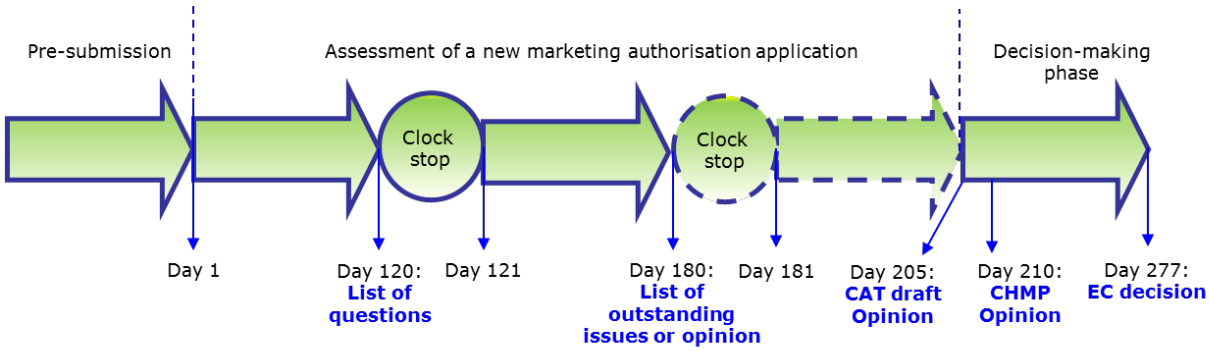
Evaluation of ATMPs (section 2)

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/