8 October 2025
EMA/CAT/387156/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 10-12 September 2025

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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Table of contents

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 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 alternate.

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 10-12 September 2025 meeting was adopted

1.3. Adoption of the minutes

The CAT minutes for 12-14 August 2025 meeting were adopted

The CAT minutes for 10-12 September 2025 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

2.3.1. Waskyra - Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding human Wiskott-Aldrich syndrome protein - Orphan - EMEA/H/C/006525

Fondazione Telethon Ets; Treatment of patients with Wiskott-Aldrich syndrome (WAS)

Scope: Day 180 list of outstanding issues

Action: for adoption

List of questions adopted on 16.04.2025.

The Rapporteurs presented the assessment of the responses to the list of questions. The revised list of outstanding issues was adopted.

The response timetable was adopted.

2.4. Day 120 list of questions

2.4.1. Onasemnogene abeparvovec - Orphan - EMEA/H/C/006498

Novartis Europharm Limited; Treatment of 5q spinal muscular atrophy (SMA)

Scope: Day 120 list of questions

Action: for adoption

The Rapporteurs presented the outcome of the assessment.

The list of questions and the response timetable was adopted.

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

2.6.1. Autologous melanoma-derived tumour infiltrating lymphocytes, ex vivo-expanded - EMEA/H/C/006563

Treatment of melanoma

Scope: 2nd Request for clock stop extension

Action: for discussion

List of questions adopted on 18.07.2025

CAT discussed the clock stop extension request and noted the recommendation from GIREX.

2.7. New applications

No items

2.8. Withdrawal of initial marketing authorisation application

No items

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

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

TETEC Tissue Engineering Technologies AG; Repair of symptomatic, localised, full-thickness cartilage defects of the knee joint grade III or IV

Scope: for appointment of re-examination Rapporteurs and re-examination timetable

Action: for discussion

The re-examination timetable was discussed and adopted.

2.10. GMP and GCP inspections requests

No items

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

2.11.1. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2771

Kite Pharma EU B.V.

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

Scope: Safety, opinion

Submission of an updated RMP version 4.3 for Tecartus and version 11.1 for Yescarta following the PRAC recommendation for the secondary malignancy of T-cell origin signal (EPITT no: 20040), and of a PASS protocol for a framework for the sampling and testing of secondary malignancies of T-cell origin.

Action: for adoption

Request for supplementary information adopted on 16.05.2025, 24.01.2025.

The opinion was adopted.

2.11.2. Ebvallo - Tabelecleucel - Orphan - EMA/VR/0000284818

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.3. Hemgenix - Etranacogene dezaparvovec - Orphan - EMA/VR/0000270071

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.4. Kymriah - Tisagenlecleucel - Orphan - EMA/VR/0000282599

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. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/VR/0000281226

Janssen Cilag International

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.6. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/VR/0000285085

Janssen Cilag International

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.7. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMA/VR/0000272240

Janssen Cilag International

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.8. Kymriah - Tisagenlecleucel - Orphan - EMA/VR/0000284307

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.9. Strimvelis - Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence - Orphan - EMA/VR/0000282021

Fondazione Telethon Ets

Rapporteur: Sol Ruiz, PRAC Rapporteur: Liana Martirosyan

Scope: Clinical, opinion

Submission of the final report from study STRIM-005. This is a non-interventional methodology study to investigate the utility of retroviral insertion site analysis in samples from subjects treated with Strimvelis gene therapy. The RMP version 8.0 has also been submitted.

Action: for adoption

The opinion was adopted.

2.11.10. Casgevy - Exagamglogene autotemcel - Orphan - EMA/VR/0000264854

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.11. Vyjuvek - Beremagene geperpavec - Orphan - EMA/VR/0000284864

Krystal Biotech Netherlands B.V.

Rapporteur: Joseph De Courcey

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.12. Imlygic - Talimogene laherparepvec - Orphan - EMA/VR/0000258245

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Quality, opinion

Action: for adoption

The opinion was adopted.

2.11.13. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMA/VR/0000280312

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Breyanzi - Lisocabtagene maraleucel - Orphan - EMA/PAM/0000285196

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: PAM

Action: for adoption

The outcome of the assessment was agreed

2.13.2. Breyanzi - Lisocabtagene maraleucel - Orphan - EMA/PAM/0000280380

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: PAM

Action: for adoption

The outcome of the assessment was agreed

2.13.3. Hemgenix - Etranacogene dezaparvovec - Orphan - EMA/PAM/0000248926

CSL Behring GmbH

Rapporteur: Silke Dorner, PRAC Rapporteur: Bianca Mulder

Scope: PAM, HCP survey, request for supplementary information

The MAH submitted a PASS protocol (version 1.0), which covers a Healthcare Professional survey (HCP) to assess the effectiveness of the additional Risk Minimisation Measures (aRMM) for Hemgenix (etranacogene dezaparvovec).

Action: for adoption

The outcome of the assessment was agreed

2.13.4. Tecartus - Brexucabtagene autoleucel - Orphan - EMA/PAM/0000273165

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Bianca Mulder

Scope: PAM, Protocol update (mainly editorial and mantle cell lymphoma milestones) for study KT-EU-472-6036.

Action: for adoption

The outcome of the assessment was agreed

2.13.5. Libmeldy - Atidarsagene autotemcel - Orphan - EMA/PAM/0000286209

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries

Scope: PAM

Action: for adoption

The outcome of the assessment was agreed

2.13.6. Tecartus - Brexucabtagene autoleucel - Orphan - EMA/R/0000278705

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Bianca Mulder

Scope: Renewal - 1 year

Action: for adoption

The 1-year renewal was agreed. The opinion was adopted.

2.13.7. Libmeldy - Atidarsagene autotemcel - Orphan - EMA/R/0000257479

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries, Co-Rapporteur: Barbara Bonamassa, PRAC Rapporteur: Gabriele Maurer

Scope: Renewal - 5 year

Action: for adoption

The 5-year renewal was agreed. The opinion was adopted.

2.14. Companion diagnostics - initial consultation

No items

2.15. Companion diagnostics – Follow-up consultation

No items

3. Certification of ATMPs

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

3.1. Opinion

No items

3.2. Day 60 Evaluation Reports

No items

3.3. New Applications

No items

4. Scientific Recommendation on Classification of ATMPs

Timetable:

-Start of the procedure: 12.09.2025

-EMA Coordinator's draft report:	23.09.2025
-CAT Coordinator's comments:	01.10.2025
-Revised scientific recommendation:	03.10.2025
-CAT's discussion of scientific recommendation:	10.10.2025

4.1. New requests – Appointment of CAT Coordinator

4.1.1. CD4+CD25+CD127-MOG-CAR+ T regulatory cells

Treatment and prevention of progression of Myelin Oligodendrocyte Glycoprotein Antibody-Associated Disease, Amyotrophic Lateral Sclerosis (ALS), Primary Progressive Multiple Sclerosis.

Scope: for nomination of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Non-replicating recombinant adeno-associated viral vector serotype hu68 (AAVhu68), containing a codon-optimized human survival motor neuron 1 (SMN1) gene

Treatment of Spinal Muscular Atrophy (SMA)

Scope: for nomination of CAT coordinator

Action: for adoption

The CAT coordinator was appointed.

4.2. Day 30 ATMP scientific recommendation

4.2.1. Lentiviral vector encoding for short hairpin RNA (shRNA) sequences down-regulating human leukocyte antigen (HLA) class I and HLA class II by targeting key messenger RNAs (mRNAs)

Ex vivo genetic modification of lung grafts prior to transplantation in patients

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 29.09.2025.

4.2.2. Viable, allogeneic, in vitro expanded human corneal keratocytes on an adhesive scaffold matrix

Treatment of deep or perforating corneal defects

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 29.09.2025.

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

No items

4.4. Finalisation of procedure

No items

4.5. Follow-up and guidance

No items

5. Scientific Advice

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

5.1. New requests - appointment of CAT Rapporteurs

5.1.1. Ongoing scientific advice procedures - Appointment of CAT Peer Reviewers

Timetable:

- Start of procedure at SAWP:	01-04.09.2025
- Appointment of CAT Peer Reviewers:	10-12.09.2025
- SAWP first reports:	22.09.2025
- CAT Peer Reviewer comments (NC & C):	26.09.2025
- CAT Peer Reviewer comments (Q):	01.10.2025
- Discussion at SAWP:	29.09-02.10.2025
- Discussion at CAT and feedback to SAWP:	08-10.10.2025

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	29.09–02.10.2025
- Appointment of CAT Peer Reviewers:	08-10.10.2025
- SAWP first reports:	20.10.2025
- CAT Peer Reviewer comments (NC & C):	24.10.2025
- CAT Peer Reviewer comments (Q):	29.10.2025
- Discussion at SAWP:	27-30.10.2025
- Discussion at CAT and feedback to SAWP:	05-07.11.2025

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

No items

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:	01-04.09.2025
SAWP recommendation:	02.10.2025
CAT recommendation:	10.10.2025
CHMP adoption of report and final recommendation:	16.10.2025

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 Denisa Partelova, as the new alternate for Slovakia.

7.1.2. Vote by proxy

Rozalina Kulaksazova gave a proxy to Pille Saalik to vote on behalf of Bulgaria for the whole meeting.

Rafaella Pontou gave a proxy vote to Maria Gazouli to vote on behalf of Cyprus for the whole meeting.

Martin Oleksiewicz gave a proxy to Emmely de Vries to vote on behalf of Denmark on Friday 12.09.2025 from 11.15 onwards.

7.1.3. Onboarding document for new CAT members/alternates

Scope: Welcoming document to assist new members/alternates

Action: for information

EMA presented the new onboarding project for new members and alternates

7.1.4. CAT Strategic Review & Learning meeting (SRLM) under the Danish presidency

Scope: Preparation for the meeting

CAT: Martin Bronislaw Oleksiewicz

Action: for information

The final programme of the upcoming SRLM and some practical information were presented.

7.2. Coordination with EMA Scientific Committees

7.2.1. GIREX report

Scope: Analysis of requests for clock-stop extensions and feedback from GIREX

Action: for discussion

EMA provide feedback from GIREX and some recommendation coming from the GIREX discussions.

7.2.2. Business Pipeline Report - Forecast for Q3-2025

Scope: Update the committee

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. Feedback from IPRP (International Pharmaceutical Regulators Programme) cell and gene therapy working group

CAT: Pille Saalik

Scope: Teleconference of 22 July 2025

Action: for information

The feedback from the IPRP teleconference were noted.

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

CAT: Ilona Reischl

Scope: Change of date to 02.10.2025 and call for topics

Action: for information

CAT members are asked to provide additional topics for inclusion in the agenda of the next ATMP cluster teleconference.

7.5.3. Opening procedures at EMA to non-EU authorities (OPEN) Framework

CAT: Ilona Reischl

Scope: proposal to broaden the scope of the OPEN framework to include ATMPs

Action: for discussion

For more information of the OPEN framework, see here

EMA presented the proposal to expand the scope of OPEN to include ATMPs. CAT was informed of the principles of the OPEN framework and the practical experience so far.

CAT was in favour of including ATMPs in OPEN.

7.6. CAT work plan

7.6.1. CAT workshop on gene editing

Scope: Informing the members of the upcoming event

Action: for information

EMA informed CAT on this upcoming workshop and how to attend.

7.6.2. State-of-play on AI activities

Scope: Stock-take on AI section of CAT workplan, update on EMRN initiatives around knowledge mining, AI literacy and change management

Action: for information

EMA provided detailed information on the AI workplan agreed under the Network Data Steering Group, the AI trainings available in EU NTC LMS and the ongoing and planned AI experimentation activities.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. REVAMP – Survey on first-hand experience

A survey has been circulated to collect feedback from users (members, assessors) on their initial experience with the new REVAMP template / process. This is a call for support in disseminating to all relevant assessors/contributors and filling the survey if involved in one of those procedures

Action: for information

CAT members we asked to complete the survey on the content and clarity of the template / process.

7.8.2. Pre-Submission Interactions Group – PreSIG

Update to the CAT on the activities of the Pre-submission Interaction Group, which is focused on enhancing pre-submission interactions to optimise submission readiness, anticipate assessment challenges, reduce premature applications, and promote alignment on submission timelines.

Action: for information

EMA provided an updated on the activities of PreSIG. The proposals were highlighted.

8. Any other business

No items

Date of next CAT meeting:

08-10 October 2025

9. List of participants

List of participants including any restrictions with respect to involvement of members/alternates/experts following evaluation of declared interests for the 10-12 September 2025 CAT 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 restrictions applicable to this meeting	
Silke Dorner	Member	Austria	No interests declared	
Claire Beuneu	Member	Belgium	No interests declared	
Olga Kholmanskikh	Alternate	Belgium	No interests declared	
Azra Selimovic	Member	Croatia	No restrictions applicable to this meeting	
Eva Kolouchová	Member	Czechia	No interests declared	
Radka Nejezchlebová	Alternate	Czechia	No interests declared	
Martin Oleksiewicz	Member	Denmark	No interests declared	
Johanne Juhl Korsbaek	Alternate	Denmark	No restrictions applicable to this meeting	
Pille Saalik	Alternate	Estonia	No interests declared	
Heli Suila	Member	Finland	No interests declared	
Maija Tarkkanen	Alternate	Finland	No interests declared	
Violaine Closson Carella	Member	France	No interests declared	
Jean-Michel Race	Alternate	France	No restrictions applicable to this meeting	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	

Maria Gazouli	Member	Greece	No restrictions applicable to this meeting	
Viola Bardoczy	Member	Hungary	No restrictions applicable to this meeting	
Agnes Zotter	Alternate	Hungary	No restrictions applicable to this meeting	
Péter Zsolt Fekete	Member	Iceland	No interests declared	
Joseph De Courcey	Member	Ireland	No interests declared	
Richard Carroll	Alternate	Ireland	No interests declared	
Concetta Quintarelli	Member	Italy	No restrictions applicable to this meeting	
Barbara Bonamassa	Alternate	Italy	No interests declared	
Una Riekstina	Member	Latvia	No restrictions applicable to this meeting	
Liga Kunrade	Alternate	Latvia	No restrictions applicable to this meeting	
Vilma Perikaite	Member (CHMP member)	Lithuania	No restrictions applicable to this meeting	
Alessia Pochesci	Member	Luxembourg	No restrictions applicable to this meeting	
Nancy De Bremaeker	Alternate	Luxembourg	No restrictions applicable to this meeting	
Emmely de Vries	Member	Netherlands	No interests declared	
Tineke van den Hoorn	Alternate	Netherlands	No interests declared	
Rune Kjeken	Member	Norway	No interests declared	
Dariusz Sladowski	Member	Poland	No restrictions applicable to this meeting	
Maria Isabel Borba Vieira	Member	Portugal	No interests declared	
Denisa Marilena Margina	Member	Romania	No restrictions applicable to this meeting	
Liviu Nitulescu	Alternate	Romania	No restrictions applicable to this meeting	
Margareta Fogelová	Member	Slovakia	No interests declared	
Denisa Partelova	Alternate	Slovakia	No interests declared	
Suzana Vidic	Member	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	
Maria Luttgen	Member	Sweden	No restrictions applicable to this meeting	
Charlotte Anderberg	Alternate	Sweden	No interests declared	
Julio Delgado Gonzalez	Member	Clinicians' Representative	No restrictions applicable to this meeting	
Alessandra Renieri	Alternate	Clinicians' Representative	No restrictions applicable to this meeting	
Federica Chiara	Member	Patients' Representative	No restrictions applicable to this meeting	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No restrictions applicable to this meeting	
Donatella Capone	Alternate	Patients' Representative	No interests declared	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Torbjörn Callréus	Expert	Malta	No interests declared	
Paolo Petracci	Expert	France	No interests declared	
Solène Maitenaz	Expert	France	No interests declared	
Gabriela Ullio Gamboa	Expert	France	No interests declared	
Simona Teodosiu	Expert	France	No interests declared	
Coralie Deligny	Expert	France	No interests declared	
Marianne Delville	Expert	France	No restrictions applicable to this meeting	
Nathalie Morgensztejn	Expert	France	No interests declared	
Nicolas Beix	Expert	France	No interests declared	
Myra Langendonk	Expert	Netherlands	No restrictions applicable to this meeting	
Loes den Otter	Expert	Netherlands	No restrictions applicable to this meeting	
Sara Ambrosino	Expert	Netherlands	No restrictions applicable to this meeting	

Charlotte de Wolf	Expert	Netherlands	No interests declared	
Beata Maria Jakli-Ullrich	Expert	Hungary	No interests declared	
Jayne Crowe	Expert	Ireland	No interests declared	
Sarah Brophy	Expert	Ireland	No restrictions applicable to this meeting	
Gavin McGauran	Expert	Ireland	No restrictions applicable to this meeting	
Ailise Carleton	Expert	Ireland	No interests declared	
Attila Sebe	Expert	Germany	No interests declared	
Finbarr Leacy	Expert	Ireland	No interests declared	
Filip Josephson	Expert	Sweden	No interests declared	
A representative from the European Commission attended the meeting Observers from Swissmedic attended the meeting Representatives from the FDA attended the meeting				
Meeting run with support from relevant EMA staff. Experts' declared interests were evaluated against the agenda topics or activities they participated in.				

10. Explanatory notes

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

Abbreviations in Committee CMD documents and in relation to EMA regulatory activities

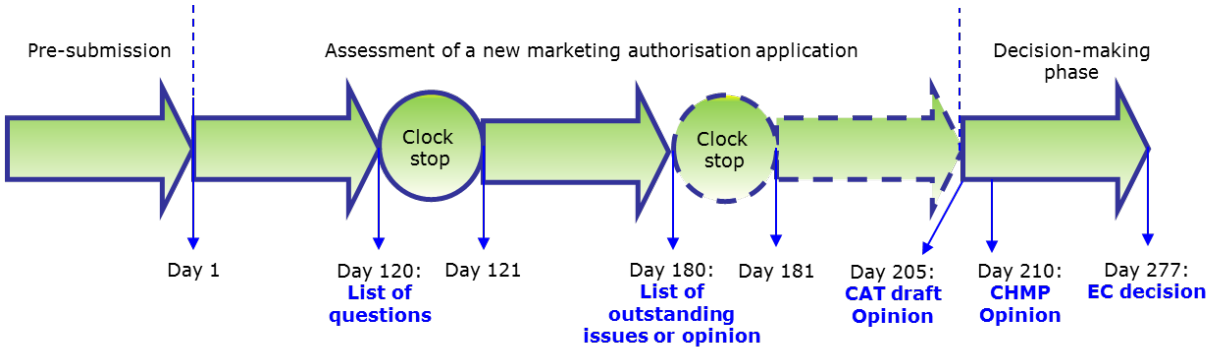
Evaluation of ATMPs (section 2)

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/