4 December 2024
EMA/CAT/8204/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 06-08 November 2024

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

Disclaimers

Some of the information contained in these minutes is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the CAT meeting reports once the procedures are finalised.

Of note, these minutes are a working document primarily designed for CAT members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the minutes cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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

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 and thanked the departing alternate for her contribution to the Committee.

1.2. Adoption of agenda

The CAT agenda for 06-08 November 2024 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for 09-12 October 2024 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

2.5.1. Lifileucel - EMEA/H/C/004741

Treatment of unresectable or metastatic melanoma

Scope: Day 80 assessment report

Action: for information

The information was noted.

2.6. Update on ongoing initial applications

No items

2.7. New applications

No items

2.8. Withdrawal of initial marketing authorisation application

No items

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

No items

2.10. 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. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/II/0043/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli, PRAC Rapporteur: Gabriele Maurer

Scope: Safety & Quality, request for supplementary information

A grouped application consisting of:

C.I.6 (Type II): Extension of indication for Breyanzi to include treatment of adult patients with 3rd line + follicular lymphoma (FL) based on final results from the pivotal study JCAR017-FOL-001 (FOL-001, TRANSCEND-FL). This is a phase 2, open-label, single-arm, multicohort, multicenter study to evaluate efficacy and safety of JCAR017 in adult subjects with relapsed or refractory (r/r) follicular Lymphoma (FL) or marginal zone lymphoma (MZL). As a consequence, sections 4.1, 4.4, 4.8, 5.1 and 5.2 of the SmPC are updated. The Package Leaflet is updated in accordance. Version 5.0 of the RMP is being submitted. Furthermore, as part of the application the MAH is requesting a 1-year extension of the market protection.

Quality variations:

B.II.d.1.e (Type II)B.II.d.1.a (Type IB)B.II.d.1.a (Type IB)

Action: for adoption

The Rapporteur presented the assessment of this variation. Further to the discussion, CAT adopted the request for supplementary information.

2.11.2. Ebvallo - Tabelecleucel - Orphan - EMEA/H/C/004577/II/0011/G

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, request for supplementary information

Type II - B.I.a.1.e

Type II - B.II.b.1.c

Type II - B.II.b.2.b

Action: for adoption

Request for Supplementary Information adopted on 13.09.2024.

CAT adopted the second request for supplementary information.

2.11.3. Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/II/0018

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Clinical

Update of sections 4.4 and 5.1 of the SmPC in order to reflect a modified 9-point anti-AAV5 Neutralising Antibody (NAb) assay. In addition, the MAH took the opportunity to introduce minor editorial changes to the PI and update the list of local representatives in the Package Leaflet and bring the PI in line with the QRD version 10.4.

Action: for adoption

The Rapporteur presented the assessment of this variation. Based on comments raised, it was agreed that additional information should be included in the SmPC. CAT adopted the request for supplementary information.

2.11.4. Libmeldy - Atidarsagene autotemcel - Orphan - EMEA/H/C/005321/II/0031/G

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries

Scope: Quality, request for supplementary information

Type II (B.I.a.1.eType IB (B.I.b.2.e) Type IB (B.I.a.1.z)

Action: for adoption

CAT adopted the request for supplementary information.

2.11.5. ROCTAVIAN - Valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830/II/0014

BioMarin International Limited

Rapporteur: Violaine Closson Carella, PRAC Rapporteur: Bianca Mulder

Scope: Clinical, request for supplementary information

Update of the Annex II in order to propose changes to the current marketing authorisation obligations for ROCTAVIAN. The RMP version 1.3 has also been submitted.

Action: for adoption

The Rapporteur presented the assessment of this variation. CAT adopted the request for supplementary information.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/022.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Clinical

Amended Protocol
Recommendation #19: The MAH should submit, within a time period of two months, a draft protocol and SAP for a prospective observational study assessing whether potentially suboptimal bridging therapy in high-risk patients observed in the KarMMa-3 study may be

alleviated in a real-world setting. This draft should then be discussed with CAT/CHMP, so that a decision can be made on whether such a prospective study should be initiated, in its initial or amended form.

Action: for adoption

The Rapporteur presented the assessment of this recommendation. CAT agreed with the protocol for the real world (RW) study and concluded that the study can be initiated. The post-authorisation measure is not considered completed, awaiting the submission of the result of the RW study.

2.13.2. Casgevy - Exagamglogene autotemcel - Orphan - EMEA/H/C/005763/MEA/011

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical & Pharmacovigilance, request for supplementary information

Protocol v. 1.0 / Study no VX24-290-102
Title: Healthcare Professional Survey (HCP) to Assess the Effectiveness of the Additional Risk Minimization Measures (aRMM) for Casgevy® (exagamglogene autotemcel).

Action: for adoption

CAT agreed with the PRAC recommendation.

2.13.3. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/ANX/002.5

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Clinical & Pharmacovigilance, request for supplementary information

MAH's response to ANX 002.2 [PAES Statistical Analysis and PASS Study KTE-EU-472-6036] RSI as adopted in February 2022. amendment #4. Title: Long-term, non-interventional study of recipients of Tecartus (brexucabtagene autoleucel) for treatment of adult patients with relapsed or refractory Mantle Cell Lymphoma (MCL).

In order to further characterise the long-term efficacy and safety of Tecartus in adult patients with relapsed or refractory Mantle cell Lymphoma (MCL) the MAH shall conduct and submit the results of a prospective study based on data from a registry, according to an agreed protocol.

Action: for adoption

CAT agreed with the PRAC assessment of the protocol of the PASS study. The request for supplementary information was agreed.

2.13.4. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/R/0047

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Rune Kjeken, PRAC Rapporteur: Bianca

Mulder

Scope: 1 year Renewal of Marketing Authorisation, Request for supplementary information

Action: for adoption

Request for supplementary information adopted on 13.09.2024.

The Rapporteur presented the assessment of the responses to the questions raised. The second request for supplementary information 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

3.3. New Applications

4. Scientific Recommendation on Classification of ATMPs

Deadline for submission of new requests: 24.10.2024.

Timetable:

-Start of the procedure:	25.11.2024
-EMA Coordinator's draft report:	19.11.2024
-CAT Coordinator's comments:	27.11.2024
-Revised scientific recommendation:	29.11.2024
-CAT's discussion of scientific recommendation:	06.12.2024

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Autologous primary urothelial cells expanded

Cystoplasty/orthotopic neobladder

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Adeno-associated virus serotype 5 containing the human NR2E3 gene (AAV5-hNR2E3)

Treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy, specifically retinitis pigmentosa or Leber congenital amaurosis, and who have sufficient viable retinal cells

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. CD70 CAR+, TCRαβ− viable cells

Treatment of renal cell carcinoma

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.2. Day 30 ATMP scientific recommendation

4.2.1. Allogeneic CD19(4G7)CAR+_TCRαβ−_CD52+/− cells

Treatment of CD19-expressing hematologic malignancies

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 22.11.2024.

4.2.2. Autologous adult bone marrow-derived, non-expanded CD133+ haematopoietic stem cells

Treatment of Asherman's syndrome

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 22.11.2024.

4.2.3. Chimeric group B adenovirus from parental wildtype viruses Ad3 and Ad7 with attenuation in E3 region and no inserted sequences

Treatment of ovarian cancer

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 22.11.24.

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

No items

4.4. Finalisation of procedure

4.4.1. Human allogeneic cardiosphere-derived cells

Treatment of muscular dystrophy

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

4.4.2. Allogenic fibroblasts embedded in a scaffold of hyaluronic acid and fibrinogen

Treatment of chronic and refractory ulcers

Scope: European Commission raised comments. ATMP scientific recommendation

Action: for discussion

The comments from the European Commission were discussed. A reference to the CAT Reflection paper on ATMP classification was included to clarify why this product should not be considered a combined ATMP. The revised classification report was adopted. The product does fulfil the definition of a tissue engineered product, not combined ATMP as provided in Article 2(1) of Regulation (EC) No. 1394/2007.

4.4.3. hiPSC derived Ovarian Support Cells (OSCs)

For ex vivo maturation of human oocytes

Scope: European Commission raised comments. ATMP scientific recommendation

Action: for discussion

The comments from the European Commission were discussed. The revised classification report was updated. Provided that the product is considered by EU National Competent

Authorities as falling under the scope of the medicinal product framework, the EMA/CAT is of the view that the product would qualify as an advanced therapy medicinal product, and more specifically of a somatic cell 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:	28-31.10.2024
- Appointment of CAT Peer Reviewers:	06-08.11.2024
- SAWP first reports:	18.11.2024
- CAT Peer Reviewer comments (NC/C):	22.11.2024
- CAT Peer Reviewer comments (Q):	27.11.2024
- Discussion at SAWP:	25-28.11.2024
- Discussion at CAT and feedback to SAWP:	04-06.12.2024

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	25-28.11.2024
- Appointment of CAT Peer Reviewers:	04-06.12.2024
- SAWP first reports:	06.01.2025
- CAT Peer Reviewer comments (NC/C):	10.01.2025
- CAT Peer Reviewer comments (Q):	15.01.2025
- Discussion at SAWP:	13-16.01.2025
- Discussion at CAT and feedback to SAWP:	22-24.01.2025

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

No items

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:	
Procedure start:	28-31.10.2024
SAWP recommendation:	28.11.2024
CAT recommendation:	06.12.24
CHMP adoption of report and final recommendation:	12.12.2024

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

No items

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

The Chair welcomed Johanne Juhl Korsbæk as the new alternate for Denmark and thanked Bibi Fatima Syed Shah for her contribution as alternate for Denmark.

7.1.2. CAT Strategic Review & Learning meeting (SRLM) under the Hungarian presidency – 19-20 November 2024

CAT: Andras Donaszi-Ivanov, Viola Bardóczy

Scope: Final agenda of the upcoming SRLM

Action: for discussion

The final agenda was presented and the topics for the CAT only session were confirmed.

CAT members were asked to register, also in case they are attending the meeting remotely.

7.1.3. Vote by proxy

Joseph de Courcey gave a proxy to Silke Dorner to vote on behalf of Joseph de Courcey during part of the meeting.

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. CoGenT Pilot

Scope: To provide an update and seek feedback on proposed touchpoints / exchange of information for the selected procedures.

Action: for discussion

Topic postponed until a next CAT meeting.

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

CAT: Ilona Reischl

Scope: Verbal report from the teleconference of 19.09.2024

Action: for information

A short oral feedback was provided.

7.6. CAT work plan

7.6.1. CAT symposium – 10.10.2024, Amsterdam, the Netherlands

Scope: Feedback from the symposium

Action: for information

CAT members were informed that all presentations and the recording are now available on the EMA website: Scientific Symposium on Advanced Therapy Medicinal Products - 'Contribution, evolution, revolution' | European Medicines Agency (EMA)

7.6.2. CAT work plan 2025

CAT: Ilona Reischl

Scope: Work plan topics in the draft 2025 CAT workplan

Action: for discussion

CAT members discussed and agreed on the topics for the 2025 CAT workplan. CAT members were encouraged to actively take part in the workplan topics. The workplan topics will be presented by the CAT chair at the Scientific Coordination Group (meeting of all chairs of the Committees). Finalisation of the workplan and adoption will take place at the December 2024 or January 2025 meeting.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. CAT regulatory session at the ESGCT Annual meeting (25.10.2024)

CAT: Ilona Reischl, Kieran Breen, Emmely de Vries

Scope: Oral feedback from the CAT Regulatory session

Action: for information

A short feedback from the CAT session at the ESGCT annual meeting was provided.

7.8.2. Unauthorised Dendritic cell therapies

CAT: Joseph De Courcey, Ilona Reischl

Scope: Exchange on national investigations of the involved companies

Action: for discussion

Joseph De Courcey presented the investigations led by the Health Products Regulatory Authority of the advertisement and/or manufacture of unauthorised dendritic cell therapies.

CAT members presented the outcome of similar investigations led by their agencies.

CAT discussed the possibility to prepare a public statement to warn vulnerable patients on these practices: the CAT statement on unproven cell-based therapies (https://www.ema.europa.eu/en/documents/public-statement/ema-warns-against-using-unproven-cell-based-therapies_en.pdf) can be used as a basis. This public statement can then be used by the national competent authorities and/or published on the EMA website. Further reflection on this will take place at the December CAT meeting.

7.8.3. FDA – Cell therapy CMC readiness for late-stage INDs

CAT: Ilona Reischl

Scope: Transcript from the FDA CBER OTP Town Hall meeting of 5 September 2024

Action: for information

The information was noted.

8. Any other business

No items

Date of next CAT meeting:

06-08 December 2024

9. List of participants

List of participants including any restrictions with respect to involvement of members/alternates/ experts following evaluation of declared interests for the 06-08 November 2024 CAT meeting, which was held in-person.

An asterisk (*) after the role, in the second column, signals that the member/alternate attended remotely. Additional experts participated in (part of) the meeting, either in person or remotely.

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 interests declared	
Claire Beuneu	Member	Belgium	No interests declared	
Olga Kholmanskikh	Alternate	Belgium	No interests declared	

Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Evelina Shumkova	Alternate	Bulgaria	No interests declared	
Azra Selimovic	Member	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	
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	
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	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Maria Gazouli	Member	Greece	No interests declared	
Angeliki Rompoti	Alternate	Greece	No restrictions applicable to this meeting	
Andras Donaszi-Ivanov	Member	Hungary	No restrictions applicable to this meeting	
Viola Bardoczy	Alternate	Hungary	No restrictions applicable to this meeting	
Joseph De Courcey	Member	Ireland	No interests declared	
Concetta Quintarelli	Member	Italy	No restrictions applicable to this meeting	
Barbara Bonamassa	Alternate	Italy	No interests declared	
Una Riekstina	Member	Latvia	No interests declared	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No interests declared	

Alessia Pochesci	Member	Luxembourg	No restrictions applicable to this meeting	
Nancy De Bremaeker	Alternate	Luxembourg	No interests declared	
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	
Emmely de Vries	Member	Netherlands	No interests declared	
Berendina Maria (Tineke) van den Hoorn	Alternate	Netherlands	No interests declared	
Rune Kjeken	Member	Norway	No participation in discussion, final deliberations and voting on:	5.3.2. INO-3112
Ole Henrik Myrdal	Alternate	Norway	No interests declared	
Dariusz Sladowski	Member	Poland	No restrictions applicable to this meeting	
Maria Isabel Borba Vieira	Alternate (to CHMP representative)	Portugal	No interests declared	
Denisa Marilena Margina	Member	Romania	No interests declared	
Liviu Nitulescu	Alternate	Romania	No restrictions applicable to this meeting	
Katarina Kollarova	Member	Slovakia	No interests declared	
Margareta Fogelová	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	Cannot act as rapporteur, other leading/co-ordinating role or peer reviewer	5.1.1.7 Delandistrogene moxeparvovec
Charlotte Anderberg	Alternate	Sweden	No interests declared	
Bernd Gansbacher	Alternate	Clinicians' Representative	No interests declared	
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	

Kerstin Sollerbrant Melefors	Member	Patients' Representative	No interests declared	
Mencia de Lemus Belmonte	Alternate	Patients' Representative	No interests declared	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No interests declared	
Federica Chiara	Alternate	Patients' Representative	No interests declared	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Torbjörn Callréus	Expert	Malta	No interests declared	
Attila Sebe	Expert	Germany	No interests declared	
Federico De Angelis	Expert	Italy	No interests declared	
Odoardo Maria Olimpieri	Expert	Italy	No interests declared	
Antonella Isgro	Expert	Italy	No interests declared	
Elisabeth Wischnitzki	Expert	Sweden	No interests declared	
Charlotte de Wolf	Expert	Netherlands	No interests declared	
Elina Rönnemaa	Expert	Sweden	No interests declared	
Representatives from the European Commission attended the meeting				
Meeting run with support from relevant EMA staff				

Experts 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.

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

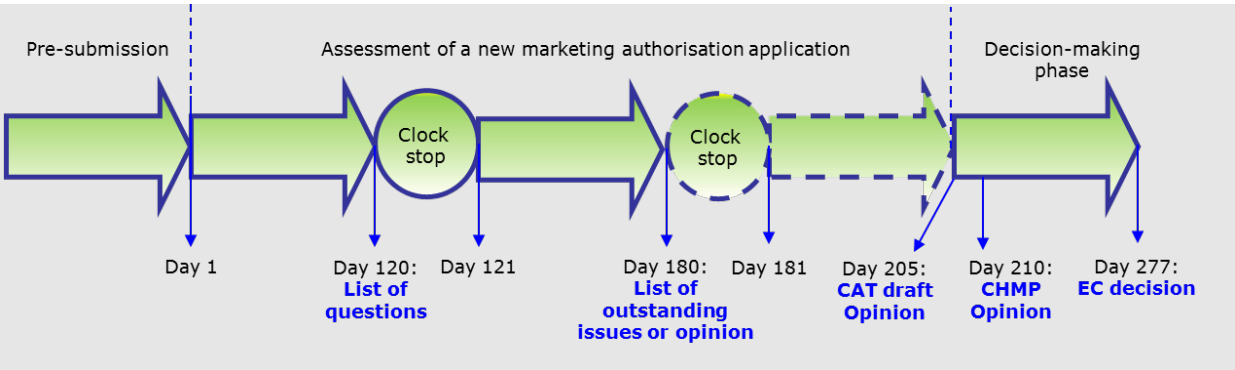
Evaluation of ATMPs (section 2)

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/