6 November 2024
EMA/CAT/554433/2024
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes for the meeting on 09 & 11 October 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

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1. Introduction

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

The Chairperson opened the meeting by welcoming all participants. The meeting was held in person with some members connected remotely.

In accordance with the Agency's policy on handling of declarations of interests of scientific committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and on the topics in the agenda of the current meeting, the Committee Secretariat announced the restricted involvement of some Committee members, alternates and experts for concerned agenda topics.

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

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

1.2. Adoption of agenda

The CAT agenda for 09-11 October 2024 meeting was adopted with one addition: 8.2. Unauthorised dendritic cell therapies.

1.3. Adoption of the minutes

The CAT minutes for 11-14 September 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

2.3.1. Beremagene geperpavec - PRIME - Orphan - EMEA/H/C/006330

Krystal Biotech Netherlands B.V.; Treatment of patients from birth with dystrophic epidermolysis bullosa (DEB) with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene

Scope: Day 180 list of outstanding issues

Action: for adoption

List of Questions adopted on 15.03.2024.

The Rapporteur presented the outcome of the assessment of the responses to the list of questions.

The list of outstanding issues and the review timetable was adopted.

2.4. Day 120 list of questions

2.4.1. Delandistrogene moxeparvovec - Orphan - EMEA/H/C/005293

Roche Registration GmbH; Treatment of ambulatory patients aged 3 to 7 years old with Duchenne muscular dystrophy

Scope: Day 120 list of questions

Action: for adoption

The Rapporteurs presented the assessment of the MAA.

The list of outstanding issues and the review timetable were adopted.

2.4.2. Dorocubicel / Allogeneic umbilical cord-derived CD34- cells, non-expanded - PRIME - Orphan - EMEA/H/C/005772

Accelerated assessment

Cordex Biologics International Limited; Treatment of adult patients with haematological malignancies

Scope: Day 120 list of questions

The Rapporteurs presented the assessment of the MAA. CAT decided to revert to a standard assessment timetable.

The list of questions and review timetable was adopted.

Action: for adoption

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

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

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

Scope: Request for extension of clock stop

Action: for discussion

CAT granted a clock stop extension in June 2024. The applicant provided justification for a further clock stop extension. CAT noted the stricter application of the clock stop rules in future but agreed with the Rapporteurs' position to grant this clock stop extension on basis of the justifications provided.

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. Kymriah - Tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0086/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Safety, Opinion

A grouped application consisting of:

C.I.4: Update of section 4.4 of the SmPC in order to amend existing warnings on cytokine release syndrome based on literature.

C.I.4: Update of section 4.4 of the SmPC in order to amend existing warnings on

neurological adverse reaction based on literature. The Package Leaflet is updated accordingly.

C.I.4: Update of section 4.4 of the SmPC in order to amend existing warnings on hypersensitivity reactions based on post marketing data and literature. The Package Leaflet is updated accordingly. In addition, the MAH took the opportunity to update the list of local representatives in the Package Leaflet and to implement editorial changes to the PI.

Action: for adoption

The Rapporteur presented the outcome of the assessment. The request for supplementary information was adopted.

2.11.2. Upstaza - Eladocagene exuparvovec - Orphan - EMEA/H/C/005352/II/0023/G

PTC Therapeutics International Limited

Rapporteur: Joseph DeCourcey

Scope: Quality, request for supplementary information

Action: for adoption

The Rapporteur presented the outcome of the assessment. The request for supplementary information was adopted.

2.11.3. Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0075/G

Kite Pharma EU B.V.

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

Scope: Clinical, opinion

Grouped application comprising two type II variations as follows:

C.I.13 - Submission of the final report from study KTE-C19-101 (ZUMA-1) listed as a category 3 study in the RMP. This is a Phase 1/2 Multicenter Study Evaluating The Safety And Efficacy Of Kte-C19 In Subjects With Refractory Aggressive Non-Hodgkin Lymphoma.

C.I.13 - Submission of the final report from study KTE-C19-106 (ZUMA-6) listed as a category 3 study in the RMP. This is a Phase 1-2 Multi-Center Study Evaluating The Safety And Efficacy Of Kte-C19 In Combination With Atezolizumab In Subjects With Refractory Diffuse Large B-Cell Lymphoma (DLBCL).

The RMP version 11.0 has also been submitted.

Action: for adoption

Request for supplementary information adopted on 21.06.2024.

The Rapporteur presented the outcome of the assessment. All issues in the request for supplementary information are satisfactorily resolved. The opinion was adopted.

2.11.4. Zolgensma - Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/II/0052

Novartis Europharm Limited

Rapporteur: Emmely de Vries

Scope: Clinical, request for supplementary information

Update of sections 5.1 and 5.2 of the SmPC in order to update efficacy and vector shedding data following request in procedure EMA/H/C/004750/P46/022 and based on data from study COAV101A12306. In addition, a reference to section 5.2 is added to section 4.4, as requested in final Assessment report of procedure EMA/H/C/004750/P46/022.

Action: for adoption

The Rapporteur presented the outcome of the assessment. The request for supplementary information was adopted.

2.11.5. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2500

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

Request for supplementary information adopted on 24.05.2024, 16.02.2024.

The Rapporteur presented the outcome of the assessment. The third request for supplementary information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/P46/023

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Clinical, opinion

Paediatric studies submitted in accordance with Article 46 of Regulation (EC) No 1901/2006, as amended.

Clinical study report of Study No. JCAR017-BCM-004
Title: A Phase 1/2, Open-label, Single Arm, Multicohort, Multicenter Trial to Evaluate the Safety and Efficacy of JCAR017 in Pediatric Subjects with Relapsed/Refractory B-cell acute

lymphoblastic leukemia (B-ALL) and B-cell non-Hodgkin lymphoma (B-NHL) (TRANSCEND PEDALL; hereafter referred to as BCM-004).

Action: for adoption

The Rapporteur presented the outcome of the assessment. The report was adopted.

2.13.2. Casgevy - Exagamglogene autotemcel - Orphan - EMEA/H/C/005763/R/0006

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Heli Suila, PRAC Rapporteur: Bianca Mulder

Scope: 1 year renewal of marketing authorisation

Action: for adoption

The Rapporteur presented the outcome of the assessment of the first renewal of Casgevy. The request for supplementary information was adopted.

2.13.3. Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/R/0020

CSL Behring GmbH

Rapporteur: Silke Dorner, Co-Rapporteur: Heli Suila, PRAC Rapporteur: Bianca Mulder

Scope: 1 year renewal of marketing authorisation

Action: for adoption

The Rapporteur presented the outcome of the assessment of the renewal of Hemgenix. One of the specific obligations has been fulfilled (submission of 5-year follow-up data). The renewal 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:	11.10.2024
-EMA Coordinator's draft report:	22.10.2024
-CAT Coordinator's comments:	30.10.2024
-Revised scientific recommendation:	31.10.2024
-CAT's discussion of scientific recommendation:	08.11.2024

4.1. New requests – Appointment of CAT Coordinator

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

Treatment of CD19-expressing haematologic malignancies

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

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

Treatment of Asherman's syndrome

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. 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: 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. Human allogeneic cardiosphere-derived cells

Treatment of muscular dystrophy

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 28.10.2024

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

Treatment of chronic and refractory ulcers

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 28.10.2024.

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

4.3.1. hiPSC derived Ovarian Support Cells (OSCs)

For ex vivo maturation of human oocytes

Scope: Response to questions; revised ATMP scientific recommendation

Action: for adoption

CAT discussed the revised scientific recommendation. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 28.10.2024

4.4. Finalisation of procedure

4.4.1. Autologous cells mainly composed of CD45+CD3+ T cells and to minor extent of other cells like B cells and NK cells derived from the regional lymph node cells and enriched for neoantigen specific T cells

For the treatment of cancers in adults

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. In vitro transcribed mRNA encoding the peptide VMAPRTLFL, a ligand for the activating immune receptor CD94/NKG2C

Treatment and / or prevention of leukaemia relapse, e.g. after haematopoietic stem cell transplant

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

4.4.3. Recombinant adeno-associated virus vector containing an expression cassette of Padua factor IX transgene

For the treatment of hemophilia B

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:	30.09-03.10.2024
- Appointment of CAT Peer Reviewers:	09.-11.10.2024
- SAWP first reports:	21.10.2024
- CAT Peer Reviewer comments (NC/C):	25.10.2024
- CAT Peer Reviewer comments (Q):	30.10.2024
- Discussion at SAWP:	28-31.11.2024
- Discussion at CAT and feedback to SAWP:	06-08.11.2024

5.1.2. Scientific advice procedures starting at the next SAWP meeting

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

5.2. 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:	30.09-03.10
SAWP recommendation:	31.10.2024
CAT recommendation:	08.11.2024
CHMP adoption of report and final recommendation:	14.11.2024

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

None

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

CAT: Andras Donaszi-Ivanov, Viola Bardóczy

Scope: Draft agenda of the upcoming SRLM

Action: for discussion

The draft agenda was presented. CAT discussed the proposed topics for the CAT-specific sessions.

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

Scope: Verbal report from the teleconference of 19.09.2024

Action: for information

Topic postponed to the November CAT meeting.

7.6. CAT work plan

7.6.1. CAT symposium – 10.10.2024, Amsterdam, the Netherlands

Scope: Feedback from the symposium

Action: for information

Topic postponed to the November CAT meeting.

7.6.2. CAT work plan 2025

CAT: Ilona Reischl

Scope: Identification of topics for inclusion in the 2025 CAT workplan

Action: for discussion

The CAT work plan topics for 2025 were presented. CAT members were asked to review and provide specific actions for 2025.

The CAT work plan topics and actions will be discussed at the November CAT meeting and/or CAT SRLM.

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

CAT: Ilona Reischl

Scope: Draft guideline, updated following the second public consultation

Action: for discussion

The CAT members were asked to review the pre-final version of the guideline and provide comments to the CAT Secretariat by 30.10.2024. The guideline will be presented to the Clinical Trial Coordination Group (CTCG) for their comments.

7.7. Planning and reporting

7.7.1. Business Pipeline Report

Scope: Q3/2024 Update of the Business Pipeline report for the human scientific committees

Action: for information

The information was noted.

7.8. Others

7.8.1. REVAMP overview template

Scope: To update members of project

Action: for information

EMA presented the new overview template and the new process.

7.8.2. Innovative features of developments

Scope: To highlight enabling technologies applying to their development from an established list. The current list of enabling technologies is outdated and we propose an updated list

Action: for discussion

EMA presented the updated list of enabling technologies. The CAT members were asked to provide comments by end of October 2024.

7.8.3. 4th China Biologics CMC Conference

CAT: Ilona Reischl

Scope: Opportunity for remote presentation at the 4th China Biologics CMC Conference (26-27.12.2024)

Action: for information

CAT members interested to give a remote or pre-recorded presentation on quality aspects of ATMP should inform the CAT Secretariat.

8. Any other business

8.1. Transition to IRIS of post-authorisation procedures in January 2025

Scope: To present the training plan for Committee members to use IRIS

Action: for information

CAT noted the information. The members were informed about the training activities to help staff from the NCAs to prepare for IRIS and the post-go-live Network support.

8.2. Unauthorised Dendritic cell therapies

CAT: Joseph De Courcey

Scope: Information from HPRA on the investigations of the companies involved

Action: for (initial) discussion

Further to press inquiries and press articles on the use of unauthorised dendritic cell therapies in cancer patients in some member states, HPRA provided feedback on the investigations of the companies involved. The CAT members were asked to provide this information to their colleagues involved in enforcements actions. A further discussion will take place at the November CAT meeting.

Date of next CAT meeting:

06-08.11.2024

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 09-11 October 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	
Claire Beuneu	Member*	Belgium	No interests declared	
Olga Kholmanskikh	Alternate*	Belgium	No interests declared	
Rozalina Kulaksazova	Member*	Bulgaria	No interests declared	
Azra Selimovic	Member*	Croatia	No interests declared	
Rafaella Pontou	Member*	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	
Toivo Maimets	Member*	Estonia	No interests declared	
Pille Saalik	Alternate*	Estonia	No interests declared	
Heli Suila	Member*	Finland	No interests declared	
Violaine Closson Carella	Member*	France	No interests declared	
Jean-Michel Race	Alternate	France	No interests declared	
Jan Mueller-Berghaus	Member (CHMP co-opted member) *	Germany	No interests declared	
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	
Richard Carroll	Alternate	Ireland	No interests declared	
Concetta Quintarelli	Member*	Italy	No restrictions applicable to this meeting	
Barbara Bonamassa	Alternate*	Italy	No interests declared	
Raimondas Benetis	Alternate (to CHMP representative) *	Lithuania	No interests declared	
Nancy De Bremaeker	Alternate	Luxembourg	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:	
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	
Metoda Lipnik-Stangelj	Alternate	Slovenia	No interests declared	
Sol Ruiz	Member (CHMP co-	Spain	No interests declared	

	opted member)			
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	
Maria Luttgen	Member*	Sweden	No restrictions applicable to this meeting	
Charlotte Anderberg	Alternate*	Sweden	No interests declared	
Bernd Gansbacher	Alternate*	Clinicians' Representative	No interests declared	
Paolo Gasparini	Member*	Clinicians' Representative	No interests declared	
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	
Andreea Barbu	Expert*	Sweden	No interests declared	
Torbjörn Callréus	Expert	Malta	No interests declared	
Nathalie Dumarcet	Expert	France	No interests declared	
Stéphanie Hueber	Expert	France	No interests declared	
Nathalie Morgensztejn	Expert	France	No interests declared	
Elisabeth Fürst	Expert	Austria	No interests declared	
Bernhard Majer	Expert	Austria	No interests declared	
Brigitte Müller	Expert	Austria	No interests declared	
Melanie Ramberger	Expert	Austria	No interests declared	
Christine Vaculik	Expert	Austria	No interests declared	
Annemarie den Harder	Expert	Netherlands	No restrictions applicable to this meeting	
Johanna de Groot	Expert	Netherlands	No interests declared	
Victoria Hamelinck	Expert	Netherlands	No restrictions applicable to this meeting	
Juliane Rau	Expert	Germany	No interests declared	

Kathrin Bayanga	Expert	Germany	No interests declared	
Attila Sebe	Expert	Germany	No interests declared	
Clara Dingert	Expert	Germany	No restrictions applicable to this meeting	
Finbarr Leacy	Expert	Ireland	No interests declared	
Elma O'Reilly	Expert	Ireland	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.

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

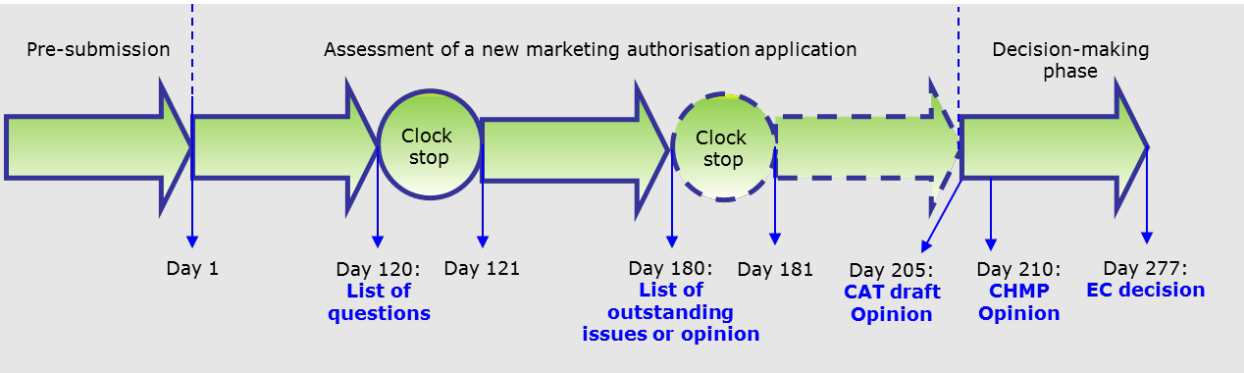
Evaluation of ATMPs (section 2)

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.

GMP and GCP Inspections Issues (section 2.10.)

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).

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.

Companion diagnostics (section 2.14)

This section lists applications for initial and follow-on consultation of companion diagnostics.

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/