22 January 2025
EMA/CAT/36325/2025
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 04-06 December 2024

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

Health and safety information

In accordance with the Agency's health and safety policy, delegates were briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

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

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

Note on access to documents

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

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1. Introduction

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

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

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

CAT agenda for 04-06 December 2024 meeting was adopted with following additions to 8. AOB:

- Webinar on monograph for gene therapy: feedback from EDQM

- Feedback from the scientific meeting: *"The Product is the Process – Is it?" Manufacturing and Translation of ATMPs and Tissue & Cell-Based Products* (Potsdam, 28.11.2024)

1.3. Adoption of the minutes

The CAT minutes for 06-09 November 2024 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

2.2.1. 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: List of Outstanding issues

Action: for discussion

List of Outstanding Issues adopted on 11.10.2024. List of Questions adopted on 15.03.2024.

The Rapporteur presented the outcome of the assessment of responses to the list of outstanding issues. The scheduled oral explanation was cancelled.

The second list of outstanding issues and the response timetable were adopted.

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

2.4.1. Lifileucel - EMEA/H/C/004741

Treatment of unresectable or metastatic melanoma

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 were adopted.

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

2.6.1. Delandistrogene moxeparvovec - EMEA/H/C/005293

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

Scope: Clock stop extension request

Action: for adoption

The request for clock stop extension was agreed.

2.7. New applications

No items

2.8. Withdrawal of initial marketing authorisation application

2.8.1. Alofisel - Darvadstrocel - Orphan - EMEA/H/C/004258

Takeda Pharma A/S

Rapporteur: Maria Luttgen; PRAC Rapporteur: Gabriele Maurer

Scope: Letter from the MAH on withdrawal of marketing authorisation for Alofisel

Action: for discussion

See also 2.11.1

The CAT Rapporteur informed the CAT on the background for the withdrawal of the marketing authorisation (MA) by the MAH: as part of the authorisation, the MAH took the obligation to perform a phase 3 study (study ADMIRE-CD II). The results of this study were submitted (variation II/51; see 2.11.1).

As part of the withdrawal of the MA, the MAH prepared a Direct Health Provider Communication (DHPC), which was discussed by CAT.

The latest adopted assessment report of variation II/51 will be made available as part of the withdrawal EPAR.

Post-meeting note: On 13.12.2024, the European Commission withdrew the marketing authorisation of Alofisel. On 19.12.2024, the DHPC is published on the EMA website.

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. Alofisel - Darvadstrocel - Orphan - EMEA/H/C/004258/II/0051/G

Takeda Pharma A/S

Rapporteur: Maria Luttgen; PRAC Rapporteur: Gabriele Maurer

Scope: Safety

A grouped application comprised of 4 Type II Variations, as follows:

(C.I.4): Update of sections 4.8 and 5.1 of the SmPC in order to update the safety information, based on pooled safety data from the two phase 3 controlled studies (ADMIRE-CD & ADMIRE-CD II) and to update efficacy information based on final results from study ADMIRE-CD II, listed as an obligation in the Annex II. ADMIRE-CD II (Cx601-0303) is a Phase III randomised double blind, placebo controlled study to assess efficacy and safety of Cx601, adult allogeneic expanded adipose-derived stem cells (eASC) for the treatment of complex perianal fistula(s) in patients with Crohn's disease. The Annex II is updated accordingly. In addition, the MAH took the opportunity to introduce minor changes to the PI, including to section 4.2 of the SmPC and to the Package Leaflet.

3 x (C.I.13): Submission of interim results from studies Darvadstrocel-3003 and Alofisel-5003 (INSPIRE) and final results from study Darvadstrocel-3002 to support the benefit-risk assessment of darvadstrocel based on all new available clinical data.

The RMP version 8.0 has also been submitted.

Action: for adoption

Request for Supplementary Information adopted on 19.07.2024.

The variation was withdrawn (see also 2.8.1).

2.11.2. Casgevy - Exagamglogene autotemcel - Orphan - EMEA/H/C/005763/II/0009/G

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

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

Pierre Fabre Medicament

Rapporteur: Egbert Flory

Scope: Quality, opinion

Action: for adoption

Request for Supplementary Information adopted on 08.11.2024, 13.09.2024.

The opinion was adopted.

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

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Clinical, opinion

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

Request for Supplementary Information adopted on 08.11.2024.

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

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

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Safety

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

Request for Supplementary Information adopted on 11.10.2024.

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

2.11.6. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/II/0051

Kite Pharma EU B.V.

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

Scope: Clinical, Request for Supplementary Information

Submission of the final study report for the non-interventional study KT-EU-472-5966 titled "Quantitative Testing of Health Care Professional Knowledge About Tecartus Risk Minimisation Measures'' listed as a category 3 study in the RMP (PRAC lead procedure)

Action: for adoption

The request for supplementary information was adopted.

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

PTC Therapeutics International Limited

Rapporteur: Joseph DeCourcey

Scope: Quality, opinion

Action: for adoption

Request for Supplementary Information adopted on 11.10.2024.

The opinion was adopted.

2.11.8. Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0077

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, opinion

Action: for adoption

Request for Supplementary Information adopted on 19.07.2024.

The opinion was adopted.

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

Novartis Europharm Limited

Rapporteur: Emmely de Vries

Scope: Clinical, opinion

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

Request for Supplementary Information adopted on 11.10.2024.

The opinion was adopted.

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

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghus

Scope: Quality, opinion

Action: for adoption

Request for Supplementary Information adopted on 11.10.2024, 24.05.2024, 16.02.2024.

The opinion was adopted.

2.11.11. Tecartus; Yescarta - Axicabtagene ciloleucel; Brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2736

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghus

Scope: Quality, Request for Supplementary Information

Action: for adoption

Request for Supplementary Information adopted on 13.09.2024.

The second 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/REC/018.1

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality, opinion

Action: for adoption

The outcome of the assessment of the quality recommendation was adopted.

2.13.2. CARVYKTI - Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095/ANX/003.3

Janssen-Cilag International NV

Rapporteur: Jan Mueller-Berghaus

Scope: Pharmacovigilance, opinion

Second Interim Report / PASS Study 68284528MMY4004
Title: An Observational Post-authorization Safety Study to Evaluate the Safety of Multiple Myeloma Patients Treated with Ciltacabtagene Autoleucel.
(From initial MAA)

Action: for adoption

The outcome of the PRAC assessment was adopted.

2.13.3. 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, opinion

Action: for adoption

Request for Supplementary Information adopted on 11.10.2024.

The 1 year renewal of the marketing authorisation was adopted.

2.13.4. Upstaza - Eladocagene exuparvovec - Orphan - EMEA/H/C/005352/S/0025

PTC Therapeutics International Limited

Rapporteur: Joseph DeCourcey, Co-Rapporteur: Maria Luttgen, PRAC Rapporteur: Gabriele Maurer

Scope: Annual Re-assessment, Request for Supplementary Information

Action: for adoption

The Rapporteur presented the outcome of the annual re-assessment. The quality specific obligation is resolved. The request for supplementary information was adopted.

2.13.5. Zolgensma - Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/LEG/024.1

Novartis Europharm Limited

Rapporteur: Emmely de Vries

Scope: Non-clinical

MAH's response to LEG 024 [from PSUSA-00010848-202305: Study reports for 2170026 & 222018] RSI as adopted in July 2024.

Study no. 2170026 (OAV101: Single Dose Intravenous or Intracerebroventricular Germline Transduction and Integration Study in Neonatal FVB/NCrl Mice with up to a 24-Week Evaluation Period).

Study no. 2220183: AAV Vector expression in cynomolgus macaque gonadal tissue of scAAV9-CB-GFP and scAAV9-CB-mCherry 28 days after administration, Molecular Localization Report.

Action: for adoption

The Rapporteur presented the outcome of the assessment of this post authorisation activity. Not changes to the SmPC (section 5.2) are required. The outcome of the assessment was adopted.

2.13.6. Tecartus - Brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/R/007

Kite Pharma EU B.V.

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

Scope: Renewal

Action: for adoption

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

4. Scientific Recommendation on Classification of ATMPs

4.1. New requests – Appointment of CAT Coordinator

4.1.1. mRNAs encoding IL-12 and IL-18

Treatment of gastric cancer

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. BCMA targeting Chimeric Antigen Receptor expressing mRNA transfected autologous T cells

Treatment of Myasthenia Gravis

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Autologous Tumor Infiltrating Lymphocytes (TILs)

Treatment of adult patients with advanced or 2/4 metastatic solid tumors who have not responded to standard therapies (chemotherapy, radiation therapy, molecule-targeted therapy) or are ineligible for alternative treatment options or in associated treatment with chemotherapy in multimodel therapy

Scope: Appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.4. Adeno-associated virus serotype 5 containing the human RORA gene (AAV5-hRORA)

Treatment of adult and paediatric patients with vision loss due to Geographic Atrophy secondary to dry age-related macular degeneration and Stargardt Disease

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. Autologous primary urothelial cells expanded

For use of cystoplasty/orthotopic neobladder

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 10.01.2025.

4.2.2. 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: 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 10.01.2025.

4.2.3. CD70 CAR+, TCRαβ− viable cells

Treatment of renal cell carcinoma

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 10.01.2025.

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

No items

4.4. Finalisation of procedure

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

Treatment of CD19-expressing hematologic malignancies

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

The classification report was adopted. The product does fulfil the definitions of a gene therapy medicinal product and a somatic cell therapy medicinal product and is therefore classified as a gene therapy medicinal product as provided in Article 2(5) of Regulation (EC) No. 1394/2007.

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

Treatment of Asherman's syndrome

Scope: European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

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

4.4.3. 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: 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:	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

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	13-16.01.2025
- Appointment of CAT Peer Reviewers:	22-24.01.2025
- SAWP first reports:	03.02.2025
- CAT Peer Reviewer comments (NC/C):	07.02.2025
- CAT Peer Reviewer comments (Q):	12.02.2025
- Discussion at SAWP:	10-13.02.2025
- Discussion at CAT and feedback to SAWP:	19-21.02.2025

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:	25-28.11.2024
SAWP recommendation:	16.01.2025
CAT recommendation:	24.01.2025
CHMP adoption of report and final recommendation:	30.01.2025

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

The Chair welcomed Linga Kunrade as the new alternate for Latvia and thanked Petr Soukup for his contribution as member for Czechia.

7.1.2. Vote by proxy

Heli Suila gave a proxy to Silke Dorner to vote on behalf of Finland on Friday 06.12.2024

Radka Nejezchlebova gave a proxy to Katarína Kollarova to vote on behalf of Czechia during the entire meeting

Vilma Perikaite gave a proxy to Toivo Maimets to vote on behalf of Lithuania during the entire meeting

Concetta Quinterelli gave a proxy to Violaine Closson-Carella will vote on behalf of Italy during the entire meeting.

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

CAT: Andras Donaszi-Ivanov, Viola Bardóczy

Scope: Feedback from the meeting

Action: for information

Viola Bardoczy provided a short feedback from the discussion at the SRLM meeting that was held in Budapest (Hungary) on 19-20.11.2024

7.1.4. CAT deadlines around the end of year holiday period

CAT: Emmely de Vries, Tineke van den Hoorn

Scope: Challenges for NCAs with the timetables of centralised procedures during the end-of-year period

Action: for information

Note: topic raised in September by delegates from Netherlands in CAT, PRAC and CHMP

EMA presented the proposals to address the challenges of NCA with timetables during the end-of-year period. This information was noted.

7.1.5. CAT Strategic Review & Learning meeting (SRLM) under the Polish presidency

CAT: Dariusz Sladowski

Scope: Preparation for the meeting

Action: for information

CAT noted the date of the SRLM meeting that will be held under the Polish presidency: 29-30 April 2025 in Warsaw, jointly with COMP.

7.2. Coordination with EMA Scientific Committees

No items

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

7.3.1. Guidelines for Sick Cell Disease and Beta-Thalassemia

Scope: CAT involvement in the drafting of the Guidelines for Sick Cell Disease and Beta-Thalassemia by HAEMWP

Action: for discussion

CAT noted the guidelines that will be developed by the HAEMWP. CAT agreed to be involved in the commenting phase as there are already sufficient experts from the national authorities in the drafting groups.

7.4. Cooperation with the EU regulatory network

No items

7.5. Cooperation with international regulators

7.5.1. EMA/FDA Collaborations / Liaison Officials Programme

Scope: Awareness of EMA/FDA collaboration and Liaison Program (and how Committee members / Rapporteurs can make use / benefit of it)

Action: for information

CAT noted the presentation from the EMA ad FDA liaison officers on the EMA/FDA collaborations in place.

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

CAT: Ilona Reischl

Scope: Draft agenda of the teleconference of 12.12.2024

Action: for discussion

The topics on the agenda were presented and CAT contributors to the different topics were identified.

7.6. CAT work plan

7.6.1. CAT work plan 2025

CAT: Ilona Reischl

Scope: CAT work plan 2025

Action: for appointment of CAT contributors to the workplan topics

CAT contributors and CAT topic leads were appointed for the different work plan topics. The work plan is scheduled for adoption at the January 2025 CAT meeting.

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

CAT: Ilona Reischl

Scope: Draft guideline, updated following comments from GCG, CAT and BWP members

Action: for adoption

CAT adopted the Guideline on quality, non-clinical and clinical requirements for investigational ATMPs in clinical trials. The CAT chair and CAT secretariat thanked all the drafting group members (present and previous CAT members, alternates and experts) for their enormous collaboration in the extended drafting of this guideline.

7.7. Planning and reporting

7.7.1. Business Pipeline Report

Scope: Q4/2024 Update of the Business Pipeline report for the human scientific committees

Action: for information

Topic postponed

7.8. Others

7.8.1. Clinical study data pilot

Scope: Feedback on experience with the clinical study data pilot and next steps.

Action: for information

EMA presented the background and experience from the pilot. EMA has decided to extend the pilot's duration. As a result, pilot participation requests from pharmaceutical industry will continue to be accepted until further notice. Following CAT member agreed to be part of the EMRN Advisory Group on raw materials: Olga Kholmanskikh.

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

CAT was informed of the actions taken by France against illegal advertisement of these unauthorised dendritic cell therapies. CAT proposed that the EMA statement of 28 April 2020 ('*EMA warns against using unproven cell-based therapies*') should be re-issued and reinforced following the recent advertisement campaigns in the EU for unauthorised dendritic cell therapies. CAT also mentioned that international collaboration would be beneficial.

8. Any other business

8.1.1. Webinar on monograph for gene therapy

EDQM: Catherine Milne

Scope: Feedback from the EDQM on the webinar that was held on 03.12.24

Action: for information

EDQM provided a short feedback. The slides from the webinar will be made available available on the EDQM website. As EDQM plans to embark on genome editing, it was considered advisable that a link with CAT activities on this issue is established.

8.1.2. Feedback from the scientific meeting: "The Product is the Process – Is it?" Manufacturing and Translation of ATMPs and Tissue & Cell-Based Products (Postdam, 28.11.2024)

CAT: Ilona Reischl

Scope: Feedback from the meeting

Action: for information

Ilona Reischl provided feedback from this scientific meeting that was organised by PEI in collaboration with other German associations. See here for more information: Events - "The Product is the Process – Is it?" Manufacturing and Translation of ATMPs and Tissue & Cell-Based Products - Paul-Ehrlich-Institut. Some points arising from the presentations were flagged to CAT.

Date of next CAT meeting:

22-24 January 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 4-6 December 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	
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	
Isavella Kyriakidou	Alternate	Cyprus	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	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)*	Germany	No interests declared	
Maria Gazouli	Member	Greece	No interests declared	

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	
Una Riekstina	Member	Latvia	No interests declared	
Alessia Pochesci	Member	Luxembourg	No restrictions applicable to this meeting	
John J. Borg	Member (CHMP member)	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	Cannot act as rapporteur, other leading/co-ordinating role or peer reviewer for:	
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-	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	
Alessandra Renieri	Alternate	Clinicians' Representative	No restrictions applicable to this meeting	
Kerstin Sollerbrant Melefors	Member	Patients' Representative	No interests declared	
Mencia de Lemus Belmonte	Alternate	Patients' Representative	No interests declared	
Kieran Breen	Member (Vice-Chair)	Patients' Representative	No interests declared	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Torbjörn Callréus	Expert	Malta	No interests declared	
Jenny (Zhiyi) You	Expert	Denmark	No interests declared	
Kinga Nowicka-Matus	Expert	Denmark	No interests declared	
Boje Kvorming Pires Ehmsen	Expert	Denmark	No interests declared	
Kristina Bech Jensen	Expert	Denmark	No interests declared	
Raquel Martín	Expert	Spain	No interests declared	
Irene Izquierdo	Expert	Spain	No interests declared	
Jorge Martínez	Expert	Spain		
Liana Martirosyan	Expert	Netherlands	No interests declared	
Finbarr Leacy	Expert	Ireland	No interests declared	
Caoimhin Concannon	Expert	Ireland	No interests declared	
Elma O'Reilly	Expert	Ireland	No interests declared	
Stéphanie Hueber	Expert	France	No interests declared	
Paolo Petracci	Expert	France	No interests declared	

Filip Josephson	Expert	Sweden		
Annemarie den Harder	Expert	Netherlands	No restrictions applicable to this meeting	
Daiana Vasilcanu	Expert	Sweden	No interests declared	
Jayne Crowe	Expert	Ireland	No interests declared	
Representatives from the European Commission 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.

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

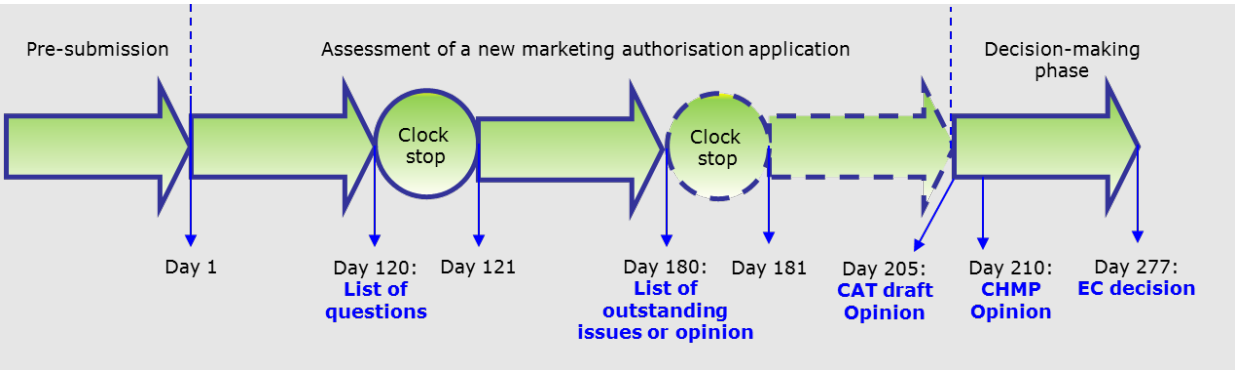
Evaluation of ATMPs (section 2)

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 2.14.)

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).

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 2.14-2.15)

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/