08 December 2021
EMA/CAT/636354/2021
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 03-05 November 2021

Chair: Martina Schuessler-Lenz; Vice-Chair: Ilona Reischl

Disclaimers

Some of the information contained in these minutes are considered commercially confidential or sensitive and therefore not disclosed. Regarding 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. Due to the current coronavirus (COVID-19) outbreak, and the associated EMA Business Continuity Plan (BCP), 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 meeting, the Committee Secretariat announced that no restriction in the involvement of meeting participants in upcoming discussions was identified. Participants in this meeting 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 and as included in the list of participants. All decisions taken at this meeting were made in the presence of a quorum of members. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

1.2. Adoption of agenda

The CAT agenda for 03-05 November 2021 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for 06-08 October 2021 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

2.2.1. Eladocagene exuparvovec - Orphan - EMEA/H/C/005352

PTC Therapeutics International Limited; treatment of aromatic L-amino acid decarboxylase (AADC) deficiency

Scope: adoption of the list of outstanding issues

Action: for adoption

List of outstanding issues adopted on 16 April 2021

The CAT agreed that an oral explanation was not needed at this time.

The Rapporteurs presented the outcome of the assessment of the list of outstanding issues.

On the quality questions, feedback was received from the discussion in BWP.

The second list of outstanding issues was adopted.

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

2.4.1. Valoctocogene roxaparvovec - Orphan - EMEA/H/C/005830

Accelerated assessment

BioMarin International Limited; treatment of severe haemophilia A

Scope: Day 120 list of questions

Action: for adoption

The Rapporteurs presented the assessment of the marketing authorisation application.

Feedback was provided from the BWP discussion on the quality part of the application.

The list of questions was adopted.

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

2.6.1. Lenadogene nolparvovec - Orphan - EMEA/H/C/005047

GenSight Biologics S.A.; treatment of vision loss due to Leber hereditary optic neuropathy (LHON)

Scope: MAA's request for additional clock stop extension

Action: for adoption

D120 List of Questions adopted in February 2021

CAT discussed justifications for the additional clock stop included in the letter from the applicant.

2.7. New applications

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. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0001/G

Celgene Europe B.V.

Rapporteur: Rune Kjeken

Scope: Quality. Opinion

Action: for adoption

The opinion was adopted

2.11.2. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0002

Celgene Europe B.V.

Rapporteur: Rune Kjeken

Scope: Quality. Request for Supplementary Information

Action: for adoption

The Request for Supplementary Information was adopted.

2.11.3. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/II/0047/G

Amgen Europe B.V.

Rapporteur: Heli Suila

Scope: Quality. Opinion

Action: for adoption

The opinion was adopted

2.11.4. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0042

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Claire Beuneu, PRAC Rapporteur: Anette Kirstine Stark

Scope: Clinical. Request for Supplementary Information

Extension of indication to include the treatment of adult patients with relapsed or refractory follicular lymphoma (FL) after three or more lines of systemic therapy. Consequently, sections 4.1, 4.8, 5.1 and 5.2 of the SmPC, Annex II (Section D) and Package Leaflet are

proposed to be updated. As a consequence, the RMP (version 5.1) has been updated to align with the indication extension.
In addition, the applicant has taken the opportunity to make minor editorial corrections throughout the SmPC and package leaflet to align with the current Quality Review of Documents (QRD) template.

Action: for adoption

The Rapporteur presented the assessment of this variation to extend the indication of Yescarta. CAT discussed if FL grade 3B can be included in the indication, even though excluded from the Zuma-5 trial. It was agreed to ask the company (as other concern) to justify this extrapolation/inclusion of this subpopulation.

The Request for Supplementary information was adopted.

2.11.5. Zolgensma - onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/II/0019/G

Novartis Gene Therapies EU Limited

Rapporteur: Carla Herberts

Scope: Quality. Request for Supplementary Information

Action: for adoption

The Request for Supplementary information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Libmeldy - atidarsagene autotemcel - Orphan - EMEA/H/C/005321/ANX/002.1

Orchard Therapeutics (Netherlands) BV

Rapporteur: Carla Herberts, CHMP Coordinator: Johann Lodewijk Hillege

Scope: Revised Protocol / LongTERM-MLD Study
In order to further characterise the long-term efficacy and safety of Libmeldy in children with late infantile or early juvenile forms of MLD, 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

This post-authorisation measure related to the updated protocol for the post-authorisation efficacy study (PAES). The MAH has addressed the comments from CAT on the original protocol (discussed at the June 2021 CAT meeting, agenda point 2.13.2).

A couple of minor comments have been formulated by the Rapporteur. The post-authorisation measure was adopted.

2.13.2. Libmeldy - atidarsagene autotemcel - Orphan - EMEA/H/C/005321/REC/006

Orchard Therapeutics (Netherlands) BV

Rapporteur: Carla Herberts, CHMP Coordinator: Johann Lodewijk Hillege

Scope: Quality.

Action: for adoption

The post-authorisation measure was adopted.

2.13.3. Luxturna - voretigene neparvovec - Orphan - EMEA/H/C/004451/REC/007

Novartis Europharm Limited

Rapporteur: Sol Ruiz, CHMP Coordinator: Maria Concepcion Prieto Yerro

Scope: Medical Safety Risk and Impact assessment submitted as follow-up to the 'Communication concerning Novartis ophthalmic products associated with the Field Safety Notice issued by Becton Dickinson (BD, MPS-18-1209).

Action: for adoption

This post-authorisation measure was related to the device used for administration ofthe medicinal product. The MAH considered that the problem report with the device (Field safety notice) does not have an impact on the quality, safety or efficacy of Luxturna.

The post-authorisation measure was adopted.

2.13.4. Withdrawal of the marketing authorisations for Zynteglo and Skysona

Bluebird bio.

Rapporteur (Zynteglo): Carla Herberts, CoRapporteur (Zynteglo): Violaine Closson-Carella, Rapporteur (Skysona): Lisbeth Barkholt, CoRapporteur (Skysona): Denmark

Scope: bluebird bio has decided to withdraw the MAs for Zynteglo and Skysona and also the PRIME bb-1111. The reason is commercial due to not being able to reach agreements on reimbursement.

Action: for information

Note: formal requests for the withdrawal of Skysona and Zynteglo will be submitted in late October and mid December 2021 respectively.

CAT noted the presentation by EMA on the withdrawal of the two marketing authorisations. A follow-up meeting will be organised with the company to discuss on what could have been done more or differently, e.g. harmonisation of post-authorisation data collection between EMA and the HTAs, harmonisation of clinical data for approval and for HTA/payers.

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:	22.11.2021
-EMA Coordinator's draft report:	19.11.2021
-CAT Coordinator's comments:	24.11.2021
-Revised scientific recommendation:	03.12.2021
-CAT's discussion of scientific recommendation:	10.12.2021

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Non-replicating recombinant adeno-associated virus serotype 2 (rAAV2) encoding a soluble form of human CD59 (sCD59)

Intended for the treatment of geographic atrophy (via targeting the complement pathway)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. VTXM01 messenger RNA (mRNA) encoding for an adenine base editor (ABE) and VTXG01 guide RNA (gRNA) targeting the proprotein convertase subtilisin/kexin type 9 (PCSK9) serine protease gene

Intended for the treatment of adults with heterozygous familial hypercholesterolemia (HeFH) who require additional lowering of low-density lipoprotein cholesterol (LDL-C) despite maximally tolerated lipid-lowering therapy

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Autologous anti-CD19 chimeric antigen receptor T-cells

Intended for the treatment of CD19-expressing B-cell malignancies

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. CD 19 CAR T-cells transduced with lentiviral vector

Intended for the treatment of adults and children with B-cell non-Hodgkin's lymphoma and acute lymphoblastic leukemia. CD 19 CAR-T cell therapy will be used as first salvage in patients with primary refractory disease or in first relapse, after one line of systemic therapy, and with the presence of least one pre-defined high-risk feature

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 19 November 2021.

4.2.2. Allogeneic adipose-derived mesenchymal stromal cells, ex-vivo expanded

Intended for the treatment of osteoarthritis, knee

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 19 November 2021.

4.2.3. Recombinant adeno-associated virus, serotype 2, containing human ND4 codon-optimised gene (rAAV2-ND4) - EMA/PRIME/21/039

Treatment of Leber's hereditary optic neuropathy (LHON) associated with ND4 G11778A mutation

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 19 November 2021.

4.2.4. Allogeneic T-cell precursors, mobilised peripheral blood-derived, ex vivo cultured

Intended for the treatment of paediatric and adult patients undergoing partially human leucocyte antigen (HLA) compatible allogeneic haematopoietic stem cell transplantation to accelerate adaptive immunological reconstitution

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 19 November 2021.

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

No items

4.4. Finalisation of procedure

4.4.1. Autologous red blood cells chemically coupled with 12 antigenic peptides

Intended for the treatment of multiple sclerosis

Scope: The European Commission raised no comments. ATMP scientific recommendation

Action: for information

The information was noted.

4.5. Follow-up and guidance

4.5.1. Revision of the procedural advice on ATMP classification

Scope: Revision of the procedural advice to align to current practices

Action: for adoption

The revised procedural advice was presented. The revision is to align the document with the current process of evaluating requests for ATMP classification. The revised procedural advice was adopted and will now be published on the ATMP classification webpage on the EMA website.

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:	25-28.10.2021
- Appointment of CAT Peer Reviewers:	03-05.11.2021
- SAWP first reports:	22.11.2021
- CAT Peer Reviewer comments:	26.11.2021
- Discussion at SAWP:	29.11-02.12.2021
- Discussion at CAT and feedback to SAWP:	10.12.2021

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	29.11–02.12.2021
- Appointment of CAT Peer Reviewers:	08-10.12.2021
- SAWP first reports:	03.01.2022
- CAT Peer Reviewer comments:	07.01.2022
- Discussion at SAWP:	10-13.01.2022
- Discussion at CAT and feedback to SAWP:	15.01.2022

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

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

No items

5.4. Final Advice Letters for procedures finalised the previous month

5.5. Request for clarification

6. Pre-Authorisation Activities

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

6.1. Paediatric investigation plans

No items

6.2. ITF briefing meetings in the field of ATMPs

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:	
Procedure start:	28.10.2021
SAWP recommendation:	02/12/2021
CAT recommendation:	10/12/2021
CHMP adoption of report and final recommendation:	16/12/2021

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

6.3.4. Ongoing support

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

Action: for information

The Chair welcomed Vlasta Zavadova as the new member for Lichtenstein.

7.1.2. Vote by proxy

No items

7.1.3. Joint CAT-CHMP Strategic Review & Learning (virtual) meeting (SRLM) under the Slovenian presidency, 20-21 October 2021, Ljubljana (Slovenia)

CAT: Metoda Lipnik-Štangelj, Martina Schuessler-Lenz

Scope: feedback from the meeting

Action: for information

7.1.4. Joint CAT-CHMP Strategic Review & Learning (virtual) meeting (SRLM) under the Portuguese presidency, 27 May 2021, Lisbon (Portugal)

CAT: Bruno Sepodes, Maria Isabel Vieira

Scope: minutes of the joint CAT-CHMP session

Action: for information

The minutes of the joint CAT-CHMP session were noted.

7.2. Coordination with EMA Scientific Committees

7.2.1. CHMP learning with relevance to CAT

CAT: Romaldas Maciulaitis

Scope: Topics from the October CHMP PROM with relevance to CAT decisions: (1) Structured guidance on reflection of use of extrapolation - development of an assessor's guidance template; (2) ICH E8(R1) step 5 - General considerations for clinical studies

Action: for information

Romaldas Maciulaitis and EMA colleagues introduced both documents to CAT.

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

7.3.1. Scientific advice for ATMPs

Scope: Review of the new procedure for SA for ATMPs that was implemented in February 2021

Action: for discussion

CAT members provided comments on the new SA procedure. In general, the procedure is working appropriately, and positive feedback has been received from SAWP on the CAT input on ATMP SA requests.

7.3.2. Call for interest for nomination of CAT members to join the subgroup "GCP inspection outcomes in support of B/R evaluation"

Scope: Review of the current inspections procedures to enhance inspector assessor information flow and understanding

Action: for information

CAT was informed that a survey to better understand the expectations of assessors regarding integrated inspection reports will be launched. It was highlighted that the CAT members' input would be very appreciated. Interested members should contact EMA by 12 November 2021

7.3.3. Call for interest for nomination of CAT members to join Advisory Group on Raw Data on Lifecycle Regulatory Submissions Raw Data

Scope: Call for interest for nomination of CHMP members to join Advisory Group on Raw Data in order to assist the design of the future proof-of-concept raw data pilot (estimated to kick-off in April 2022).

EMA's Lifecycle Regulatory Submissions Raw Data project is focusing on utilising raw data to generate evidence for better and more efficient regulatory decision making.

This project is part of the Data Analytics Programme also known as the Agency's vehicle for evolving to data-driven medicines regulation and constitutes one of the priority recommendations of the EMA-HMA Big Data Taskforce.

Action: for information

This information was provided to CAT members via e-mail. Interested members should contact EMA by 10 November 2021.

7.3.4. Guideline on registry-based studies

Scope: Published guideline on registry-based studies

Action: for information

Note: The guideline on registry-based studies has now been published. This new guideline is based on a discussion paper on methodological and operational aspects for use in patient registries for regulatory purposes, which was available for a public consultation that generated almost 1,000 comments from 68 stakeholder organisations. Experience gained from CHMP qualification opinions on two networks of registries and input collected during five workshops on specific patient registries organised by the Agency helped shape the final guidance.

LINK to the News Item – LINK to the Guideline

The information was noted.

7.4. Cooperation with the EU regulatory network

7.4.1. Update on the Companion Diagnostics (CDx) consultation procedure

Scope: To provide an update on interactions of CHMP-CAT-EMA experts with Notified Bodies (NBs) to develop the NB consultation procedure on CDx suitability with EMA. EMA guidance on CDx consultation and CHMP AR CDx consultation have been reviewed.

Action: for discussion

EMA provided an update on the CDx consultation procedure. Two documents were presented: procedural guidance and the guidance to assessors. Comments from CAT are awaited by 17th November 2021, to allow both documents to be finalised at the December CAT/CHMP meetings. Some comments that made during the CAT discussion will also be taken into account.

7.4.2. Pharma legislation revision

CAT: Ilona Reischl

Scope: Core definitions; Active Substance Master File.

Action: for discussion

Draft proposals were presented and discussed.

7.4.3. Revision of the EU legislation on blood, tissues and cells (BTC)

CAT: Martina Schüssler-Lenz

Scope: Feedback from discussions with the European Commission

Action: for information

EMA provided a detailed feedback from the discussion with the European Commission on the BTC revision.

The CAT chair thanked EMA for the feedback.

7.5. Cooperation with international regulators

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

CAT: Martina Schuessler-Lenz

Scope: feedback from the teleconference that took place on 14 October 2021

Action: for information

Topic postponed to the next CAT meeting.

7.6. CAT work plan

7.6.1. CAT work plan 2022

CAT: Martina Schuessler-Lenz

Scope: identification of work plan topics for 2022

Action: for discussion

EMA presented some proposal of topics for the CAT work plan for 2022. Some of the topics are a continuation of topics from the 2021 work plan and some new topic were proposed. The topics were discussed and agreed. CAT proposed to include an additional topic in the work plan for 2022: This work plan topic might be conducted jointly with the CHMP.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. CAT stakeholder meeting on 26 October 2021

CAT: Martina Schuessler-Lenz

Scope: feedback from the meeting

Action: for discussion

Feedback was given from the CAT stakeholder meeting. A short discussion took place on how to make future stakeholder meetings more interactive.

7.8.2. European Society of Gene and Cell Therapy (ESGCT)

CAT: Alessandro Aiuti

Scope: feedback from the meeting that took place on the 19-22 October 2021.

Action: for information

Topic postponed to the next CAT meeting.

8. Any other business

Date of next CAT meeting:

08-10/12/2021

9. Explanatory notes

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

Abbreviations / Acronyms

AAV: Adeno-Associated Virus

AR: Assessment Report

ATMP: Advanced Therapy Medicinal Product

BWP: Biologics Working Party

CAT: Committee for Advanced Therapies

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

CTFG: Clinical Trial Facilitation Group

DG: Drafting Group

EC: European Commission

EU NTC: European Union Network Training Centre

ERA: Environmental Risk Assessment

FDA: Food and Drug Administration

FL: Final Letter

GCG: Guideline Consistency Group

GCP: Good Clinical Practice

GLP: Good Laboratory Practice

GMO: Genetically-modified organism

GMP: Good Manufacturing Practice

GTMP: Gene Therapy Medicinal Product

HTA: Health Technology Assessment Bodies

HSPC: Hematopoietic Stem and Progenitor Cells

ITF: Innovative Task Force

JR: Joint Report

LoOI: List of outstanding issues

LoQ: List of questions

MA: Marketing Authorisation

MAA: Marketing Authorisation Application

MAH: Marketing Authorisation Holder

MNAT: Multinational assessment team

MSC: Mesenchymal stem cells

PDCO: Paediatric Committee

PMDA: Pharmaceuticals and Medical Devices Agency (Japan)

PIP: Paediatric Investigation Plan

PL: Package leaflet

PRAC: Pharmacovigilance and Risk Assessment Committee #

PRIME: Priority Medicines

QRD: Quality review of documents

RMP: Risk Management Plan

RP: Reflection paper

RSI: Request for supplementary information

SAs: Scientific Advices

SAG-O: Scientific Advisory Group Oncology

SAWP: Scientific Advice Working Party

SR: Summary Report

SWP: Safety Working Party

SME: Small and medium size enterprises

SmPC: Summary of Products Characteristics

TT: Timetable

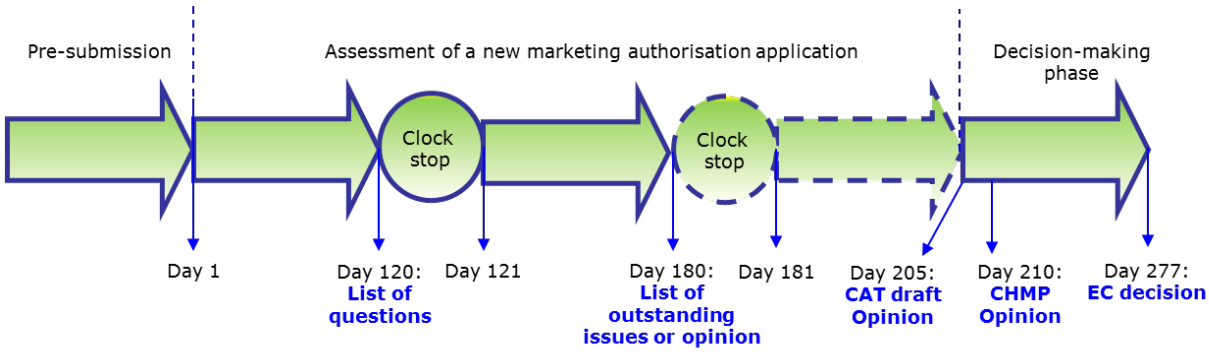
Evaluation of ATMPs (section 2)

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 any ATMP related inspection requests (section 2.9) and Post-authorisation activities (section 2.10).

New applications (sections 2.1. to 2.12.)

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 2.3) 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 2.7 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.

Re-examination procedures (new applications) under article 9(2) of regulation no 726/2004 (section 2.6.)

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.

Withdrawal of applications (section 2.7.)

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.

New applications (section 2.9.)

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.

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 2.12.)

This section lists type II variations, extension application according to Annex I of Reg. 1234/2008, re-examination procedures for type II variations (including extension of indication applications) for which the applicant has requested re-examination of the opinion previously issued by the CHMP and other issues concerning authorised medicines that are not covered elsewhere in the agenda such as 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.

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/

10. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 03-05 November 2021 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Martina Schüssler-Lenz	Chair	Germany	No interests declared	
Ilona Reischl	Member (Vice-Chair)	Austria	No interests declared	
Silke Dorner	Alternate	Austria	No interests declared	
Claire Beuneu	Member	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	
Petra Sokol	Alternate	Croatia	No interests declared	
Rafaella Pontou	Member	Cyprus	No interests declared	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	
Tomas Boran	Member	Czechia	No interests declared	
Petr Soukup	Alternate	Czechia	No interests declared	
Ebru Karakoc Madsen	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	
Maija Tarrkanen	Alternate	Finland	No interests declared	
Violaine Closson	Member	France	No interests declared	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Maria Gazouli	Member	Greece	No interests declared	
Angeliki Rompoti	Alternate	Greece	No interests declared	
Katalin Lengyel	Member	Hungary	No interests declared	
Balázs Sarkadi	Alternate	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Niamh Curran	Alternate	Ireland	No restrictions applicable to this meeting	
Concetta Quintarelli	Member	Italy	No interests declared	
Barbara Bonamassa	Alternate	Italy	No restrictions applicable to this meeting	
Una Riekstina	Member	Latvia	No interests declared	
Romaldas Mačiulaitis	Member (CHMP member)	Lithuania	No interests declared	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No interests declared	
Vlasta Zavadova	Member	Liechtenstein	No interests declared	
Guy Berchem	Alternate	Luxembourg	Restrictions applicable to this meeting	4.1.1., 5.2.2., 5.2.5., 5.4.10. & 2.11.1
Nancy De Bremaeker	Member	Luxembourg	No interests declared	
John J. Borg	Member (CHMP member)	Malta	No interests declared	
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	
Carla Herberts	Member	Netherlands	No interests declared	
Babs Fabriek	Alternate	Netherlands	No interests declared	
Rune Kjeken	Member	Norway	No restrictions applicable to this meeting	
Maja Sommerfelt Grønvold	Alternate	Norway	No restrictions applicable to this meeting	
Dariusz Śladowski	Member	Poland	No restrictions applicable to this meeting	
Marcin Kolakowski	Alternate	Poland	No interests declared	
Maria Isabel Borba Vieira	Alternate (to CHMP representative)	Portugal	No interests declared	
Silviu Istrate	Member	Romania	No interests declared	
Alexandrina Preda	Alternate	Romania	No interests declared	
Alexandra Padova	Alternate	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Sol Ruiz	Member (CHMP co-opted member)	Spain	No interests declared	
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	
Lisbeth Barkholt	Member	Sweden	No interests declared	
Maria Luttgen	Alternate	Sweden	Restrictions applicable to this meeting	5.2.1. & 5.4.9.
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	
Alessandro Aiuti	Member	Healthcare Professionals' Representative	Restrictions applicable to this meeting	2.13.1., 2.13.2. & 5.2.6.
Alessandra Renieri	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Kerstin Sollerbrant	Member	Patients' Representative	No interests declared	
Lydie Meheus	Alternate	Patients' Representative	No interests declared	
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	
Roland Pochet	Alternate	Patients' Representative	No interests declared	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Olga Kholmanskikh	Expert - via Webex	Belgium	No interests declared	
Brigitte Mueller	Expert - via Webex	Austria	No interests declared	
Christine Vaculik	Expert - via Webex	Austria	No interests declared	
Christoph Mueck	Expert - via Webex	Austria	No interests declared	
Florian Klinglmueller	Expert - via Webex	Austria	No interests declared	
Martin Walter	Expert - via Webex	Austria	No interests declared	
Philipp Janesch	Expert - via Webex	Austria	No interests declared	
Rene Anour	Expert - via Webex	Austria	No interests declared	
Susanne Wolf	Expert - via Webex	Austria	No interests declared	
Tjerk Feenstra	Expert - via Webex	Austria	No interests declared	
Andrea Laslop	Expert - via Webex	Austria	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Peter Kiely	Expert - via Webex	Ireland	No interests declared	
Nuala Kelly	Expert - via Webex	Austria	No interests declared	
Helene Blok	Expert - via Webex	Netherlands - IGZ	No interests declared	
Jean-Michel Race	Expert - via Webex	France	No interests declared	
Matthew Burbank	Expert - via Webex	France	No interests declared	
Gabriela Ullio-Gamboa	Expert - via Webex	France	No interests declared	
Norontsoa Rasolondramanitra	Expert - via Webex	France	No interests declared	
Stéphanie Jambon	Expert - via Webex	France	No interests declared	
Caroline Matko	Expert - via Webex	France	No interests declared	
Greger Abrahamsen	Expert - via Webex	Norway	No interests declared	
Christoph Liebold	Expert - via Webex	Germany	No restrictions applicable to this meeting	
Johannes Hendrikus Ovelgonne	Expert - via Webex	Netherlands	No interests declared	
A representative from the European Commission attended the meeting				
Meeting run with support from relevant EMA staff				