07 September 2022
EMA/CAT/712285/2022
Human Medicines Division

Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 07-09 September 2022

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

07 September 2022, 14:00 – 18:30, room 01-D

08 September 2022, 09:00 – 18:30, room 01-D

09 September 2022, 09:00 – 13:00, room 01-D

Health and safety information

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

Disclaimers

Some of the information contained in this agenda 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, this agenda is a working document primarily designed for CAT members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the agenda 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

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the CAT plenary session to be held 07-09 September 2022. See 07-09 September 2022 CAT minutes (to be published post 05-07 October 2022 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 07-09 September 2022 meeting

1.3. Adoption of the minutes

CAT minutes for 13-15 July 2022 meeting

CAT minutes of the 10-12 August 2022 written procedure

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. Tabelecleucel - PRIME - Orphan - EMEA/H/C/004577

Atara Biotherapeutics Ireland Limited; treatment of Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV^{+} PTLD)

Scope: Day 180 list of outstanding issues

Action: for adoption

List of Questions adopted on 18.03.2022.

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

No items

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. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0004

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality. Request for Supplementary Information

Action: for adoption

2.11.2. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0005

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: Clinical. Request for Supplementary Information

Extension of indication to include treatment of adult patients with Second-line (2L) Transplant Intended (TI) Large B-Cell Lymphoma (LBCL) for BREYANZI, based on interim analyses from pivotal study JCAR017-BCM-003; this is a global randomised multicentre Phase III Trial to compare the efficacy and safety of JCAR017 to standard of care in adult

subjects with high-risk, transplant-eligible relapsed or refractory aggressive B-cell Non-Hodgkin Lymphomas (TRANSFORM); As a consequence, sections 4.1, 4.4, 4.8, 5.1 and 5.2 of the SmPC are updated. The Package Leaflet is updated in accordance. Version 2.0 of the RMP has also been submitted.

Action: for adoption

2.11.3. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/II/0054

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Quality. Opinion

Action: for adoption

2.11.4. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/II/0056

Amgen Europe B.V.

PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: Pharmacovigilance. Request for Supplementary Information

Submission of the final report from study 20120139 listed as a category 3 study in the RMP in order to fulfil MEA/004. This is a multicentre, observational registry study to evaluate the survival and long-term safety of subjects who previously received talimogene laherparepvec in Amgen or BioVEX sponsored clinical trials.

Action: for adoption

2.11.5. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0055

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality. Opinion

Action: for adoption

Request for Supplementary Information adopted on 17.06.2022.

2.11.6. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0056

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Safety and efficacy. Opinion

Update of sections 4.2 and 5.1 of the SmPC in order to update efficacy and safety information in paediatric population based on study CCTL019C2202 (BIANCA), a phase II, single arm, multicentre open label trial to determine the safety and efficacy of tisagenlecleucel in paediatric patients with relapsed or refractory mature B-cell non-Hodgkin

lymphoma (NHL). The Package Leaflet is updated accordingly.

Action: for adoption

Request for Supplementary Information adopted on 17.06.2022.

2.11.7. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0060

Novartis Europharm Limited

Rapporteur: Rune Kjeken, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: Safety. Opinion

Update of section 4.2 of the SmPC in order to update the paediatric statement for the B-cell ALL indication and section 4.4 to update the warning on 'prior treatment with anti-CD19 therapy' as well as sections 4.4 and 4.8 in order to update safety data to reflect the pool of the 3 studies B2202, B2205J and B2001X. The proposed changes are in line with the request of the CHMP following the assessment of P46/012. The Package Leaflet is updated accordingly. In addition, the MAH took the opportunity to correct the Complete Response Rate (CRR) 95% confidence interval (CI) on enrolled set for E2202 study presented in table 8 in section 5.1 of the SmPC. The RMP version 5.0 has also been submitted.

Action: for adoption

Request for Supplementary Information adopted on 15.07.2022.

2.11.8. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0061/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality. Opinion

Action: for adoption

Request for Supplementary Information adopted on 15.07.2022.

2.11.9. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0062

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Clinical. Opinion

Submission of the final report from study CCTL019B2401 listed as a category 1 study in the Annex II of the Product Information in order to fulfil ANX/007.3. This is a post authorisation efficacy studies (PAES) sub-analysis to assess efficacy in patients with relapsed or refractory diffuse large B-cell lymphoma based on data from the registry study to assess the long-term safety of patients with B lymphocyte malignancies treated with tisagenlecleucel. The Annex II is updated accordingly.

Action: for adoption

2.11.10. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0046

Kite Pharma EU B.V.

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

Scope: Safety and efficacy. Opinion

Extension of indication to include treatment of adult patients with relapsed or refractory (r/r) diffuse large B-cell lymphoma (DLBCL) and high-grade B-cell lymphoma (HGBL) for Yescarta; as a consequence, sections 4.1, 4.2, 4.8, 5.1 and 5.2 of the SmPC are updated. The Package Leaflet is updated in accordance. Version 5.3 of the RMP has also been submitted. In addition, the Marketing authorisation holder (MAH) took the opportunity to update the product information with minor editorial changes.

Action: for adoption

Request for Supplementary Information adopted on 15.07.2022, 13.05.2022 and 18.02.2022.

2.11.11. Zolgensma - onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/II/0031

Novartis Gene Therapies EU Limited

Rapporteur: Carla Herberts

Scope: Quality. Request for Supplementary Information

Action: for adoption

2.11.12. Tecartus; Yescarta - axicabtagene ciloleucel; brexucabtagene autoleucel - Orphan - EMEA/H/C/WS2247

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality. Opinion

Action: for adoption

Request for Supplementary Information adopted on 15.07.2022 and 13.05.2022.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/010

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality

Action: for adoption

2.13.2. Abecma - idecabtagene vicleucel - Orphan - EMEA/H/C/004662/REC/011

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality

Action: for adoption

2.13.3. Alofisel - darvadstrocel - Orphan - EMEA/H/C/004258/R/0036

Takeda Pharma A/S

Rapporteur: Lisbeth Barkholt, Co-Rapporteur: Maria Isabel Borba Vieira, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: 5-year Renewal of Marketing Authorisation

Action: for adoption

2.13.4. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/ANX/001

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: In order to further assess the consistency of product quality and clinical outcomes, the MAH shall submit batch analysis and corresponding clinical safety and effectiveness data from a minimum of thirty (30) lots of Breyanzi finished product used to treat patients included in a non-interventional study based on secondary use of data from existing registries, according to an agreed protocol. Based on these data the MAH should also provide an evaluation on the need for a revision of the finished product specifications. Interim reports should be provided after approximately 15 lots and any significant out of trend results should be reported immediately.
Protocol [From Initial MAA]

Action: for adoption

2.13.5. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/REC/010

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality

Action: for adoption

2.13.6. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/P46/017

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Paediatric studies submitted in accordance with Article 46 of Regulation (EC) No1901/2006, as amended. Clinical study report (Study No. CCTL019BUS03): a phase II, open label, multi-centre trial to determine the efficacy and safety of tisagenlecleucel re-infusion in Paediatric and Adolescent Young Adult (AYA) patients with acute lymphoblastic leukaemia (ALL) experiencing loss of B cell aplasia.

Action: for adoption

Request for Supplementary Information adopted on 17.06.2022.

2.13.7. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/REC/019

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality

Action: for adoption

2.13.8. Libmeldy - atidarsagene autotemcel - Orphan - EMEA/H/C/005321/ANX/003.1

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Carla Herberts

Action: for adoption

2.13.9. Tecartus - brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/MEA/005.3

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Protocol, study no. KT-EU-472-5966: Prescriber Survey: Assess the prescribers' understanding of the risks of KTE-X19. Evaluate the effectiveness of risk minimization activities: HCP educational materials, and Patient Alert Card. Further alignment of the questionnaire with other approved product is requested. Updated protocol expected within 60 days. 60 days assessment procedure.
[MAH Response to MEA-0005.2 as adopted in May 2022]

Action: for adoption

2.13.10. Tecartus - brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/R/0025

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Rune Kjeken, PRAC Rapporteur: Menno van der Elst

Scope: 1-year Renewal of Marketing Authorisation

Action: for adoption

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:	09.09.2022
-EMA Coordinator's draft report:	23.09.2022
-CAT Coordinator's comments:	28.09.2022
-Revised scientific recommendation:	30.08.2022
-CAT's discussion of scientific recommendation:	07.10.2022

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Allogeneic adipose derived mesenchymal stem cells

Intended for the treatment of Crohn-related perianal fistula

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.2. Autologous adipose derived mesenchymal stem cells

Intended for the treatment of Crohn-related perianal fistula

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.3. Autologous anti-BCMA CAR-T cells

Intended for the treatment of multiple myeloma

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.4. Allogeneic latency-2 Epstein-Barr virus-targeted cytotoxic T lymphocytes

Intended for the treatment of multiple sclerosis

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.5. E1-deleted (replication defective) recombinant human adenovirus serotype 5 expressing TIMP3 (tissue inhibitor of metalloproteinases-3) under the control of the cytomegalovirus immediate early promoter.

Intended for the treatment of coronary artery disease requiring artery bypass grafting (CABG)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.6. Autologous CD34+ cells transfected with a lentiviral vector containing codon-optimised RPS19 gene

Intended for the treatment of transfusion-dependent, steroid-resistant paediatric patients with Diamond-Blackfan anaemia, who have a mutation in the RPS19 gene

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.2. Day 30 ATMP scientific recommendation

4.2.1. Autologous cultured limbal epithelial and limbal epithelial stem cells growing on fibrin scaffold

Intended for the treatment of moderate to severe limbal stem cell deficiency (LSCD) caused by burns, including chemical burns to the eyes

Scope: ATMP scientific recommendation

Action: for adoption

4.2.2. Human allogeneic cardiac progenitor cell subpopulation selected for the absence of the surface marker CD90

Intended to improve cardiac perfusion and function in patients with refractory angina

Scope: ATMP scientific recommendation

Action: for adoption

4.2.3. Allogeneic CD33-directed genetically modified T-cell immunotherapy

Intended for the treatment of patients with CD33-positive acute myeloid leukaemia (AML) who are at a high risk of relapse

Scope: ATMP scientific recommendation

Action: for adoption

4.2.4. Allogeneic CRISPR/Cas9-edited hematopoietic stem and progenitor cells (HSPCs) lacking CD33 protein expression

Intended for the treatment of patients with CD33-positive acute myeloid leukaemia (AML) who are at a high risk of relapse

Scope: ATMP scientific recommendation

Action: for adoption

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

No items

4.4. Finalisation of procedure

No items

4.5. Follow-up and guidance

No items

5. Scientific Advice

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

5.1. New requests - appointment of CAT Rapporteurs

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

Timetable:

- Start of procedure at SAWP:	29.08–01.09.2022
- Appointment of CAT Peer Reviewers:	07–09.09.2022
- SAWP first reports:	19.09.2022
- CAT Peer Reviewer comments (NC,C):	23.09.2022
- CAT Peer reviewer comments (Q):	28.09.2022

- Discussion at SAWP: 26–29.09.2022
- Discussion at CAT and feedback to SAWP: 07.10.2022

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP: 26-29 September 2022
- Appointment of CAT Peer Reviewers: 05-07.10.2022
- SAWP first reports: 17.10.2022
- CAT Peer Reviewer comments (NC,C): 21.10.2022
- CAT Peer reviewer comments (Q): 26.10.2022
- Discussion at SAWP: 24-27.10.2022
- Discussion at CAT and feedback to SAWP: 04.11.2022

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

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

No items

5.4. Final Advice Letters for procedures finalised the previous month

6. Pre-Authorisation Activities

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

6.1. Paediatric investigation plans

No items

6.2. ITF briefing meetings in the field of ATMPs

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:

Procedure start:	01/09/2022
SAWP recommendation:	29/09/2022
CAT recommendation:	07/10/2022
CHMP adoption of report and final recommendation:	13/10/2022

No items

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

No items

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

Action: for information

7.1.2. Vote by proxy

No items

7.1.3. Rules of procedure

Scope: Following the entry into force of Regulation (EU) 2017/746 on *in vitro* diagnostic medical devices (IVDR) certain changes in the CAT Rules of Procedure (RoP) are required so that the relevant legislative provisions related to EMA work in the area of medical devices are fully reflected in the CAT mandate and RoP.

Action: for adoption

7.1.4. Update on procedure for Chair election

Action: for information

7.2. Coordination with EMA Scientific Committees

7.2.1. PRIME implementation of 5-year review recommendations

Scope: Presentation of the proposals for implementation of the recommendations arising from the first 5 years' experience with the scheme (see also prime-analysis-first-5-years-experience_en.pdf (europa.eu) as discussed and agreed by the PRIME oversight group.

Action: for adoption

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

No items

7.4. Cooperation with the EU regulatory network

7.4.1. European Institute of Innovation and Technology (EIT) Health / European Medicines Agencies Regulatory Network (EMRN) joint workshop on genome editing

Scope: The agenda of this genome editing expert workshop is tabled for endorsement.

The workshop aims to close knowledge gaps relating to the regulation of future genome editing products.

Action: for adoption

7.4.2. The European Pharmacopoeia Commission texts amendment in the area of cell and gene therapy

CAT: Barbara Bonamassa

Scope: All new European Pharmacopoeia (Ph. Eur.) texts and texts that have undergone technical revisions are published in Pharmeuropa for public consultation https://www.edqm.eu/en/-/pharmeuropa-34.3-just-released. The deadline for comments on Pharmeuropa 34.3 is 30 September 2022.

Action: for information

7.5. Cooperation with international regulators

No items

7.6. CAT work plan

No items

7.7. Planning and reporting

7.7.1. Planning estimates of forthcoming ATMP MAAs

Scope: Q3/2022 update of the business pipeline report for the human scientific committees

Action: for information

7.8. Others

7.8.1. Pilot on formal expert elicitation in the context of benefit-risk assessment

Scope: Project proposal on systematic approaches in expert groups on benefit risk

Action: for discussion

7.8.2. Adeno-associated viral (AAV) vector toxicities: regulatory considerations

CAT: Carla Herberts, Egbert Flory

Scope: Discussion paper insertional mutagenesis and follow-up for AAV gene therapy

Action: for discussion

7.8.3. European Society for Gene and cell therapy (ESGCT) annual meeting

CAT: Martina Schüssler-Lenz

Scope: Agenda of the CAT regulatory session at the ESGCT annual meeting that will take place in Edinburgh on 14 October 2022.

Action: for discussion

7.8.4. CAT Learnings

Action: for adoption

8. Any other business

No items

Date of next CAT meeting:

05-07/10/2022

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

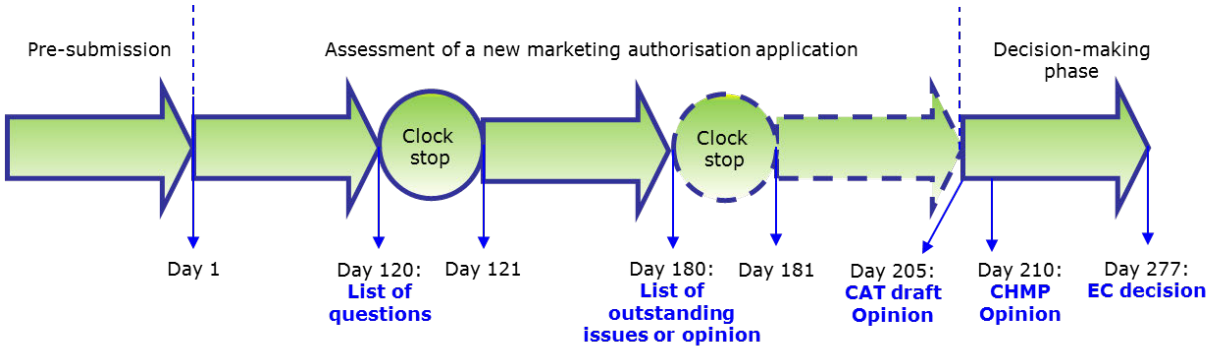
Evaluation of ATMPs (section 2)

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/