17 March 2021
EMA/CAT/165783/2021
Human Medicines Division

Committee for Advanced Therapies (CAT)

Agenda for the meeting on 17-18 March 2021

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

17 March 2021, 14:00 – 18:00, virtual meeting

18 March 2021, 09:00 – 18:30, virtual meeting

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regards 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 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 17-19 March 2021. See March 2021 CAT minutes (to be published post-March 2021 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 17-19 March 2021 meeting

1.3. Adoption of the minutes

CAT minutes for the 17-19 February 2021 meeting

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

2.6.1. Idecabtagene vicleucel - Orphan - EMEA/H/C/004662

Celgene Europe BV; treatment of multiple myeloma

Scope: communication from the applicant dated 08.03.2021 requesting an extension of the clock stop

Action: for adoption

List of Questions adopted on 11.09.2020. List of outstanding issues adopted on 04.12.2020. List of outstanding issues adopted on 19.02.2021

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. Spherox - spheroids of human autologous matrix-associated chondrocytes - EMEA/H/C/002736/II/0020

CO.DON AG

Rapporteur: Lisbeth Barkholt

Scope: Clinical. RSI

Extension of the indication for use in the paediatric population (15 to 18 years).

Action: for adoption

2.11.2. Spherox - spheroids of human autologous matrix-associated chondrocytes - EMEA/H/C/002736/II/0022

CO.DON AG

Rapporteur: Lisbeth Barkholt

Scope: Clinical. RSI

Update of section 5.1 of the SmPC with the final results of study cod 16 HS 13, a 60-month follow up data assessing long-term efficacy and safety of Spherox.
Annex II has also been updated to reflect the completion of the study.

Action: for adoption

2.11.3. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0030

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality. Opinion

Action: for adoption

Request for Supplementary Information adopted on 22.01.2021.

2.11.4. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0031

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality. Opinion

Action: for adoption

Request for Supplementary Information adopted on 22.01.2021.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Glybera – Alipogene tiparvovec – EMA/H/C/0002145/SOB/001.10

uniQure biopharma B.V.; treatment lipoprotein lipase deficiency (LPLD)

Rapporteur: Egbert Flory; CHMP Coordinator: Jan Mueller-Berghaus

Scope: Clinical. Annual safety update report
Long term surveillance programme/ disease registry to collect information on the epidemiology of the disease and the demographics, safety, and the effectiveness outcomes of patients treated with Glybera.
The patients enrolled in clinical studies (CT-AMT-010 -10, CT-AMT 011-01, CT-AMT 011-02) should be followed up in the LPLD registry.

Action: for adoption

2.13.2. Zolgensma - onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/R/0012

Novartis Gene Therapies EU Limited

Rapporteur: Carla Herberts, Co-Rapporteur: Egbert Flory; PRAC Rapporteur: Ulla Wändel Liminga

Scope: 1-year renewal of Marketing Authorisation

Action: for adoption

Request for Supplementary Information adopted on 22.01.2021.

2.13.3. Zynteglo - betibeglogene autotemcel - Orphan - EMEA/H/A-20/1504

bluebird bio (Netherlands) B.V

CAT-PRAC working group: CAT: Carla Herberts (Rapporteurs) and Violaine Closson-Carella (Co-Rapporteur), Alessandro Aiuti; PRAC: Brigitte Keller-Stanislawski, Menno van der Elst

Scope: Referral procedure under Article 20 PhV. List of Questions to the MAH

Action: for information

2.13.4. Tecartus – Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured – EMEA/H/C/005102

Kite Pharma EU B.V.; indicated for treatment of adult patients with relapsed or refractory Mantle cell lymphoma (MCL).

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Rune Kjeken; PRAC Rapporteur: Menno van der Elst, PRAC Co-Rapporteur: Brigitte Keller-Stanislawski

Scope: question from the MAH

Action: for discussion

2.13.5. Committees discussion and outcome on milestones and performance indicators for the deadline for improvement for imposed PASS using the European Society for Blood and Marrow Transplantation (EBMT) as data source

Action: for discussion and 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

4.1. New requests – Appointment of ITF Coordinator

4.1.1. Allogeneic, expanded, engineered E4ORF1+ human umbilical cord endothelial (CD31+) cells [E-CEL UVEC® cells]

Intended to treat organ vascular niche injuries caused by myeloablative, non-central nervous system penetrating high-dose chemotherapy (HDT) to prevent the development of severe regimen-related toxicities (SRRT) in patients diagnosed with aggressive systemic lymphoma

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.2. Oncolytic adenovirus

Intended for the treatment of histologically and radiologically confirmed progressive neuroendocrine neoplasm (NEN) of gastrointestinal, pancreatic or bronchial origin with multiple liver metastases (liver-dominant disease)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.3. Autologous antigen presenting cells loaded with SARS-CoV-2 antigen

Intended as a vaccine against SARS-CoV-2

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.4. Autologous mesenchymal stem cells combined with a matrix pre-loaded with BMP2

Intended to treat femoral osteochondral lesion (grade III to IV)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.5. DNA plasmid encoding several neoepitopes from the tumor of a patient, a live wild-type modified vaccinia strain Ankara (MVA) and a monoclonal antibody against Cytotoxic T-lymphocyte associated protein 4 (CTLA4)

Intended for the treatment of cancer

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.6. Autologous cultured chondrocytes

Intended for the treatment of filling of cartilage defects

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.7. Recombinant adeno-associated virus encoding for the human α-sarcoglycan-protein

Intended for the treatment of patients with a confirmed diagnosis of Limb-Girdle muscular dystrophy Type 2D/R3 (LGMD2D/R3)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.2. Day 30 ATMP scientific recommendation

4.2.1. Autologous antigen specific Cytotoxic T Lymphocytes

Intended for the treatment of cancer patients that are over expressing the specific antigen

Scope: ATMP Scientific recommendation

Action: for adoption

4.2.2. Autologous dendritic cells activated against tumour peptides

Intended for the treatment of cancer patients; *in vivo* immune stimulation against specific cancer overexpressing the tumour antigen

Scope: ATMP Scientific recommendation

Action: for adoption

4.2.3. Autologous M1-polarized macrophages

Intended for the treatment of cancer patients

Scope: ATMP Scientific recommendation

Action: for adoption

4.2.4. Autologous Cytotoxic Natural Killer (NK) cells

Intended for the treatment of cancer patients

Scope: ATMP Scientific recommendation

Action: for adoption

4.2.5. Autologous plasma cells producing monoclonal antibodies against specific tumor antigen, for treatment of cancer patients

Intended for the treatment of cancer patients

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

4.4.1. Allogeneic human mesenchymal stem cells derived from Wharton's jelly , muscle and tendons disease

Intended for diseases of muscles and tendons

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.2. Allogeneic human mesenchymal stem cells derived from Wharton's jelly , anal fistula

Intended for the treatment of anal fistula

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.3. Allogeneic human mesenchymal stem cells derived from Wharton's jelly , androgenic alopecia

Intended for the treatment of androgenic alopecia, unspecified

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.4. Allogeneic human mesenchymal stem cells derived from Wharton's jelly , diabetic foot syndrome

Intended for the treatment of diabetic foot syndrome (DFS)

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.5. Allogeneic human mesenchymal stem cells derived from Wharton's jelly , Parkinson's disease

Intended for the treatment of Parkinson's disease

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.6. Allogeneic human mesenchymal stem cells derived from Wharton's jelly seeded on the dermal scaffold , skin ulcers

Intended for the treatment of skin ulcers

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.7. Autologous human mesenchymal stem cells derived from adipose tissue , anal fistula

Intended for the treatment of anal fistula

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.8. Autologous human mesenchymal stem cells derived from adipose tissue , androgenic alopecia

Intended for the treatment of androgenic alopecia, unspecified

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.9. Autologous human mesenchymal stem cells derived from adipose tissue (, muscle and tendons disease

Intended for diseases of muscles and tendons

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.10. Two mRNA active substances, encoding separately for Human Papilloma Virus type (HPV) 16 E6 and HPV16 E7 protein

Intended for the treatment of recurrent/metastatic HPV16-positive carcinoma

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.11. Human amniotic membrane, allogeneic, sterile, cryomilled and lyophilized

Intended for the treatment of symptoms of osteoarthritis

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.12. Autologous dendritic cells activated against SARS-COV-2 peptides

Intended for the prevention of SARS-COV-2 infection

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.13. Human umbilical cord MSC derived exosomes carrying recombinant hTERT mRNA and protein, hsa-miR-125b-5p, hsa-miR-125b-1-3p, AntimiR-21-5p

Intended for the treatment of Acute Respiratory Distress Syndrome and Chronic Obstructive Respiratory Disease

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.14. DNA plasmid encoding human transferring gene

Intended for the treatment of retinitis pigmentosa

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

4.4.15. Bacteriophage cocktail consisting of four CRISPR-armed phages

Intended for the treatment of prophylaxis of bloodstream *E. coli* infection in neutropenic patients with haematological malignancy

Scope: the European Commission has raised no comments. ATMP scientific recommendation

Action: for information

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

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

Timetable:

-Start of procedure at SAWP:	08-11.03.2021
-Appointment of CAT Peer Reviewers:	19.03.2021
-SAWP first reports:	29.03.2021
-CAT Peer reviewer comments:	02.04.2021
-Discussion at SAWP:	06-09.04.2021
-Discussions at CAT and feedback to SAWP:	15.04.2021

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

-Start of procedure at SAWP:	06-09.04.2021
-Appointment of CAT Peer Reviewers:	16.04.2021
-SAWP First Reports:	26.04.2021
-CAT Peer reviewer comments:	30.04.2021
-Discussion at SAWP:	03-06.05.2021
-Discussions at CAT and feedback to SAWP:	11.05.2021

5.2. CAT discussion

5.3. Finalisation of SA procedures

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

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:	11.03.2021
SAWP recommendation:	09.04.2021
CAT recommendation:	16.04.2021
CHMP adoption of report and final recommendation:	22.04.2121

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

Croatia – Azra Semilovic – membership mandate (member) started 21.02.2021
Italy - Concetta Quintarelli - membership mandate (member) started on 22.02.2021
Italy – Paolo Gasparini – membership mandate (member) ended 21.02.2021
Latvia – Liga Kunrade - membership mandate (alternate) ended 03.09.2021
Luxembourg – Nancy de Bremaeker – membership mandate (member) started 06.03.2021
Luxembourg – Guy Berchem – swap of role from member to alternate started 06.03.2021
Luxembourg – Anne-Cecile Vuillemin – membership mandate (member) ended 05.03.2021

Action: for information

7.1.2. Strategic Review & Learning meeting (SRLM) under the Portuguese presidency of the European Union - Lisbon, Portugal

CAT: Bruno Sepodes, Maria-Isabel Vieira

Scope: draft agenda of the joint CAT-CHMP meeting that is scheduled to take place at the SRLM on 27th May 2021

Action: for discussion

7.2. Coordination with EMA Scientific Committees

7.2.1. CHMP learnings that impact CAT decisions

CAT: Jan Mueller-Berghaus, Romaldas Mačiulaitis, John-Joseph Borg, Bruno Sepodes, Sol Ruíz

Scope: procedure to identify CHMP learnings that are relevant to CAT

Action: for information

Note: The aim is to present the excel sheet with the new CHMP learnings and check their relevance to CAT. CHMP-CAT double members will be involved and provide input. This will be a pilot for the upcoming 6 months.

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

7.3.1. Inter-Committee Scientific Advisory Group (SAG) Oncology

Scope: request for nominations

Action: for information

7.3.2. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

CAT: Martina Schüßler-Lenz, Kieran Breen

Scope: feedback on the PCWP/HCPWP joint meeting that took place on 3-4 March 2021

Action: for information

Note: the CAT Chair - Martina Schüßler-Lenz - took part on the topic of: *'Timely patients' access to advanced therapy medicinal products in the EU'*

7.3.3. CAT-COMP Working Group

CAT: Martina Schüßler-Lenz

Scope: feedback from the CAT-COMP working group meeting that took place on 15 March 2021

Action: for information

7.3.4. New active substance (NAS) status of ATMPs

CAT topic lead: Rocío Salvador Roldán; BWP Rapporteur: Martijn van der Plas

Scope: development of the BWP guidance on the structure and properties for the determination of new active substance (NAS) status of biological substances: timeline and plan of actions

Action: for discussion

Note: CAT will provide input to BWP on ATMP specific aspects (see CAT work plan). Following CAT members and experts will contribute to this activity: Ilona Reischl, Heli Suila, Niamh Curran, Marja van der Bovenkamp and Barbara Bonamassa. CAT topic lead for this work plan topic: Rocío Salvador Roldán.

7.4. Cooperation within the EU regulatory network

7.4.1. Inspection of manufacturers of viral vectors used as starting materials for genetically modified cells

CAT drafting group members: Heli Suila, Ivana Haunerova, Marcos Timón, Violaine Closson Carella

Scope: draft Q&A on principles for GMP

Action: for discussion

Note: CAT members are requested to send comments by 17 March 2021

7.4.2. Product information for medicinal products that contain or consist of modified viruses

Scope: wording agreed regarding GMO aspects (in the context of Covid-19 vaccines): consequences for the SmPC of the gene therapy products that contain or consist of viral vectors

Action: for discussion

7.4.3. Questions and Answers related to the assessment of similarity for ATMPs in the context of the orphan legislation

CAT members and experts: Claire Beuneu, Barbara Bonamassa, Violaine Closson-Carella, Niamh Curran, Rune Kjeken, Ilona Reischl, Heli Suila, Marja van der Bovenkamp

Scope: revised Questions and Answers

Action: for discussion

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

CAT: Martina Schüßler-Lenz

Scope: Appointment of CAT members to be involved in workshops organised by the Commission on the revision of the BTC legislation

Action: for discussion

Note:
-The Commission is hosting different workshops on the revision of the BTC legislation. For some of them, EMA / CAT will be consulted.
-The proposal is to appoint approx. 5 CAT members that will form the CAT expert group to join these workshops. The CAT experts will provide feedback to the CAT plenary.

7.5. Cooperation with international regulators

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

CAT: Martina Schüßler-Lenz

Scope: draft agenda of the teleconference to take place on 25 March 2021

Action: for discussion

7.5.2. International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) – ICH-S12 guideline

CAT: Rune Kjeken, Claire Beuneu

Scope: feedback on the development of the ICH-S12 guideline: nonclinical biodistribution studies for gene therapy products

Action: for discussion

7.5.3. International Pharmaceutical Regulators Programme (IPRP) – Gene therapy and cell therapy working group

CAT: Pille Säälik, Ivana Haunerova

Scope: feedback from the joint gene and cell therapy working groups teleconference that took place on 4 March 2021

Action: for discussion

7.6. CAT work plan

None

7.7. Planning and reporting

7.7.1. Planning estimates of forthcoming ATMP MAAs

Scope: Q1/2021 update of the business pipeline report for the human scientific committees

Action: for information

7.8. Others

7.8.1. Quality Review of Document (QRD) – core SmPC for genetically modified cells of ATMPs

CAT Ad Hoc labelling group: Martina Schüßler-Lenz, Alessandro Aiuti, Violaine Closson-Carella, Metoda Lipnik-Stangelj, Ilona Reischl, Isabel Vieira

Scope: presentation of the new core SmPC for consultation with CAT

Action: for discussion

7.8.2. Curriculum on Advanced Therapies Medicinal Products (ATMPs)

CAT: Ilona Reischl

Scope: plan of trainings for 2021

Action: for discussion

8. Any other business

No items

Date of next CAT meeting:
14-16 April 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: Scientific Working Party

SME: Small and medium size enterprises

SmPC: Summary of Products Characteristics

TT: Timetable

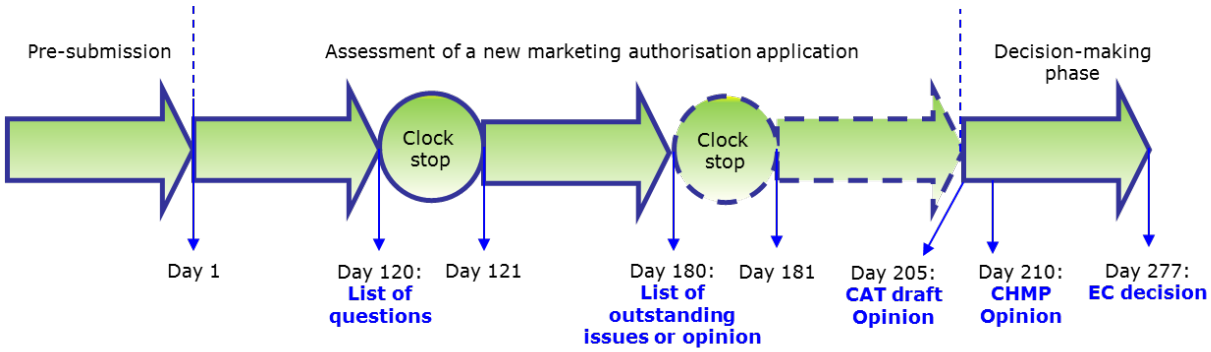
Evaluation of ATMPs (section 2)

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/