18 May 2020
EMA/CAT/297683/2020
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 22-24 April 2020

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

Disclaimers

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

Of note, the 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

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 based on the topics in the agenda of the current 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.

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 (i.e. 21 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

1.2. Adoption of agenda

The CAT agenda for 22-24 April 2020 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for 18-20 March 2020 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

2.4.1. Valoctocogene roxaparvovec - Orphan - EMEA/H/C/004749

Accelerated assessment

BioMarin International Limited; treatment of haemophilia A

Scope: Day 120 list of questions

Action: for adoption

The CAT Rapporteurs presented their assessment of the marketing authorisation application. The list of questions was discussed. CAT agreed with the BWP report .

The revised list of questions was adopted by CAT.

2.5. Day 80 assessment reports

2.5.1. Autologous anti-CD19-transduced CD3+ cells - Orphan - EMEA/H/C/005102

Accelerated assessment

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

Scope: Day 80 assessment report

Action: for information

The Rapporteurs provided an update from the ongoing assessment.

Discussion on the list of question will take place at the May CAT meeting.

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

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

Scope: Day 80 assessment report

Action: for information

The Rapporteurs provided an update from the ongoing assessment.

Discussion on the list of question will take place at the May CAT meeting.

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 - variation of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008

2.11.1. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/II/0036

Amgen Europe B.V.

Rapporteur: Olli Tenhunen

Scope: quality: Opinion

Action: for adoption

Request for Supplementary Information adopted on 21.02.2020.

The opinion was adopted.

2.11.2. Spherox - spheroids of human autologous matrix-associated chondrocytes - EMEA/H/C/002736/II/0015

CO.DON AG

Rapporteur: Lisbeth Barkholt, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: safety: Request for supplementary information
update of section 4.8 and 5.1 of the SmPC following the 48-month follow up data for trial cod 16 HS 13, a study assessing the long-term efficacy and safety of Spherox.

Action: for adoption

The Request for Supplementary Information was adopted.

2.11.3. Strimvelis - autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ada cdna sequence - Orphan - EMEA/H/C/003854/II/0024

Orchard Therapeutics (Netherlands) BV

Rapporteur: Sol Ruiz, PRAC Rapporteur: Menno van der Elst

Scope: safety: Opinion
Update of sections 4.8 and 5.1 of the SmPC in order to update the safety information following the completion of the STRIM-004 study, which is a non-interventional long term follow up of the subjects who received Strimvelis gene therapy. This study included paediatric patients and is listed as a category 3 study in the RMP. The Package Leaflet is updated accordingly. The RMP version 3.0 has also been submitted. In addition, the Marketing authorisation holder (MAH) took the opportunity to introduce minor administrative changes in the PI.

Action: for adoption

Request for Supplementary Information adopted on 20.03.2020.

The opinion was adopted.

2.11.4. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0015

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: quality: Request for supplementary information (RSI)

Action: for adoption

Request for Supplementary Information adopted on 21.02.2020.

The Request for Supplementary Information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/007

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: quality
Action: for adoption

The outcome of the assessment of the post-authorisation measure (PAM) was agreed and the PAM was considered fulfilled.

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

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:	24.04.2020
-Draft CAT co-ordinator's report:	08.05.2020
-ITF peer-review comments:	13.05.2020
-Revised scientific recommendation:	15.05.2020
-Adoption of scientific recommendation by CAT:	20.05.2020

4.1.1. Recombinant adeno-associated viral vector (serotype 8) carrying an optimised gene for human cyclic nucleotide gated channel subunit beta 3 (CNGB3) protein

Intended for the treatment of achromatopsia caused by mutations in the CNGB3 gene

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Genetically modified Lactococcus lactis strain sAGX0407, engineered to secrete human pro-insulin and human IL-10

Intended for the treatment of clinical recent-onset Type 1 diabetes mellitus

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Autologous CD34+ cells transduced with a lentiviral vector encoding a modified γ-globin gene

Intended for the treatment of sickle cell disease (SCD) and β -thalassemia

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.4. Human autologous hematopoietic stem cells transduced with a lentiviral vector containing codon-optimized cDNA encoding for functional human alpha galactosidase

Intended for the treatment of Fabry disease

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.5. Human autologous hematopoietic stem cells transduced with a lentiviral vector containing codon-optimized cDNA encoding for functional human glucocerebrosidase

Intended for the treatment of Gaucher disease

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.6. Wharton's jelly derived mesenchymal cells

Intended for the treatment of patients with COVID-19

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.7. Wharton's jelly derived mesenchymal stem cell , Covid-19

Intended for the treatment of patients with COVID-19

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.8. Wharton's jelly derived mesenchymal stem cell , Optic atrophy

Intended for the treatment of optic atrophy

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.9. Wharton's jelly derived mesenchymal stem cell , IFAP syndrome

Intended for the treatment of patients with IFAP syndrome

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.10. Wharton's jelly derived mesenchymal stem cell , Bone marrow transplant rejection

Intended for the treatment of bone marrow transplant rejection

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.11. Wharton's jelly derived mesenchymal stem cell , Secondary graft failure

Intended for the treatment of secondary bone marrow transplant failure/ secondary graft failure

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.12. Wharton's jelly derived mesenchymal stem cell , Progressive Supranuclear Palsy

Intended for the treatment of progressive Supranuclear Palsy

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.13. Wharton's jelly derived mesenchymal stem cell , Multiple system atrophy

Intended for the treatment of multiple system atrophy

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. Gene-activated matrix based on octacalcium phosphate and a plasmid carrying VEGF-A gene – H0005629

Intended to various bone healing indications (sinus lift, non-unions, spinal fusion, etc.)

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.2. Leuco platelet enriched plasma – H0005630

Intended for the treatment of ulcers, chronic wounds

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.3. Recombinant adeno-associated viral vector rh74 containing the human beta-sarcoglycan gene – H0005631

Intended for the treatment of limb-girdle muscular dystrophy type 2E

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.4. Wharton's jelly derived mesenchymal stem cell, drug resistant epilepsy

Intended for the treatment of drug resistant epilepsy

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.5. Autologous adipose-derived mesenchymal stem cell, diabetic foot syndrome

Intended for the treatment of diabetic foot syndrome

Scope: ATMP scientific recommendation

Action: for adoption

- CAT discussed the ATMP classification report. Additional information and clarifications are needed before concluding on this classification. The applicant is asked to address the questions agreed by CAT.

The list of issues was adopted by CAT and the procedure is stopped awaiting responses from the applicant.

4.2.6. Wharton's jelly derived mesenchymal stem cell, Behcet disease

Intended for the treatment of Behcet disease

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.7. Wharton's jelly derived mesenchymal stem cell, choroideremia

Intended for the treatment of choroideremia

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.8. Wharton's jelly derived mesenchymal stem cell, foetal alcohol syndrome

Iintended for the treatment of foetal alcohol syndrome

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.9. Wharton's jelly derived mesenchymal stem cell, frontotemporal dementia

Intended for the treatment of frontotemporal dementia

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.10. Wharton's jelly derived mesenchymal stem cell , progressive bulbar palsy

Intended for the treatment of progressive bulbar palsy

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.11. Wharton's jelly derived mesenchymal stem cell , vitelliform macular degeneration

Intended for the treatment of vitelliform macular degeneration (Best disease)

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 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

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

4.3.1. Recombinant chimeric vesicular stomatitis virus carrying the envelope glycoprotein (GP) of the visceral non-neurotropic strain of the lymphocytic choriomeningitis virus – H0005624

Intended for the treatment of solid tumours, including non-small cell lung carcinoma.

Scope: Responses from the applicant to the LoQs. Revised ATMP scientific recommendation

Action: for adoption

Request for List of Questions adopted on 20.03.2020.

The CAT coordinator presented the additional information provided by the applicant and the updated classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 11 May 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.4. Finalisation of procedure

4.4.1. Autologous CD34+ cells transduced with CL20-4i-EF1α-hγc-OPT lentiviral vector – H0005602

Intended for the treatment of X-linked severe combined immunodeficiency (XSCID)

Scope: the European Commission raised minor comments. ATMP scientific recommendation

Action: for information

The information was noted.

4.4.2. Wharton's jelly derived mesenchymal stem cells, AMN – H0005623

Intended for the treatment of adrenomyeloneuropathy (AMN)

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. Autologous adipose-derived mesenchymal stem cells *ex-vivo* expanded, osteoarthritis – H0005529

Intended for the treatment of osteoarthritis

Scope: corrigendum to the classification report adopted at the November 2019 CAT meeting

Action: for adoption

The corrigendum was adopted and will be sent to the applicant.

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

Timetable:

-Final Briefing Package:	08.05.2020
-Start of the procedure at SAWP:	14.05.2020
-CAT report due by:	12.05.2020
-CAT recommendation:	20.05.2020

5.2. CAT reports

5.3. List of Issues

5.4. 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

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

Austria: Silke Dorner – Membership alternate mandate started on 31 March 2020

Action: for information

The information was noted.

7.1.2. Strategic Review & Learning meeting (SRLM) – Budapest, Hungary, 08 – 10 June 2020

CAT: Katalin Lengyel

Scope: topics for inclusion in the joint CAT-PDCO agenda and CAT-only agenda to take place on 08-10 June 2020

Action: for discussion

CAT noted the cancelation of the upcoming SRLM due to the current pandemic situation.

7.1.3. Strategic Review & Learning meeting (SRLM) – Helsinki, Finland, 21 – 22 November 2019

CAT: Heli Suila

Scope: minutes of the meeting that took place on 21-22 November 2019

Action: for adoption

N.B. CAT members (and especially the speakers) were asked to provided comments on the minutes of the SRLM meeting to CAT secretariat by 10 April 2020.

Comments were received from all CAT speakers. The document is now finalised incorporating feedback also from COMP and PDCO speakers.

7.1.4. CAT's Rules of Procedures

Scope: revised CAT Rules of Procedure

Action: for information

19.03.20: EMA Management Board (MB) adopted the amendments to the existing Rules of Procedure
20.03.20: CAT adopted the amendments to the existing Rules of Procedure

CAT noted the updated CAT Rules of Procedure.

7.2. Coordination with EMA Scientific Committees

7.2.1. Committee for Medicinal Products for Human Use (CHMP)

Scope: Summary of Outcomes (SoO) for the March 2020 meeting

Action: for information

The information was noted.

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

7.3.1. Quality Review of Documents (QRD)

Scope: information on the expression of the strength and the type of cells in SmPC (sections 1 and 2)

Action: for information

Note: during the March CAT meeting, feedback was provided from the discussion in the QRD on the use of scientific notification in the expression of strength and information on the type of cells (SmPC sections 1 and 2).

CAT agreed with the pragmatic approach as proposed by QRD.

7.3.2. Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells

CAT Rapporteur: Marcos Timón / Martina Schüssler-Lenz

Scope: comments received on the clinical section of the guideline: design of the confirmatory trials for CAR-T cell products

Action: for discussion

During the finalisation of the guideline for external consultation, the requirement for randomized controlled trials (RCTs) for CAR-T cells was strengthened . External commenters indicate that the guideline text does not match current experience.

CAT discussed if the wording of the guideline (Annex I on CAR-T cells) could specifically make reference to single arm trials (SAT). It was felt difficult to introduce the possibility for SAT before this has been formally agreed by CHMP and included in the Anticancer guideline. It is difficult to capture the differences of CAR-T cells that would justify a different approach than that for the development of other oncology products. It was therefore agreed to have only a high-level recommendation on the confirmatory trials for CAR-T cells in the Guideline for genetically modified cells.

7.4. Cooperation within the EU regulatory network

7.4.1. Public statement on the use of unregulated/unproven ATMPs

Scope: EMA/CAT public statement on the use of unproven cell therapies

Action: for adoption

The public statement developed by CAT in February 2020 was reworded, with the help of the EMA Communication and Press Office colleagues, the CAT chair and the CAT draft group, to make it easier to understand for the patients.

The final version of the public statement was presented to CAT and subsequently adopted. The public statement will be published on the EMA website in the week of 27 April and will be sent to the EMA stakeholders (patient organisations, etc.). CAT members are asked to disseminate the published statement as widely as possible.

Post meeting note: the public statement was published on 28 April 2020.

7.5. Cooperation with international regulators

7.5.1. ATMP cluster teleconference with FDA-USA, Health Canada and PMDA-Japan

Scope: draft agenda for the teleconference that will take place on 30 April 2020 15:00hrs

Action: for discussion

The topics for discussion in the ATMP cluster TC were presented. CAT members interested to join the TC should inform the CAT secretariat.

7.5.2. International Pharmaceutical Regulators Programme (IPRP) – Gene therapy working group

CAT: Pille Säälik

Scope: feedback on the teleconference that took place on 26 March 2020

Action: for information

CAT noted the feedback from the last IPRP Gene therapy TC.

7.6. CAT work plan

None

7.7. Planning and reporting

None

7.8. Others

None

8. Any other business

8.1. Participation of CAT members/alternates as speakers or panellist to international conferences

Scope: criteria for participation to international conferences

Action: for discussion

An initial discussion took place. This topic will be further discussed at a next CAT meeting.

Date of next CAT meeting:

18-20/05/2020

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

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

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

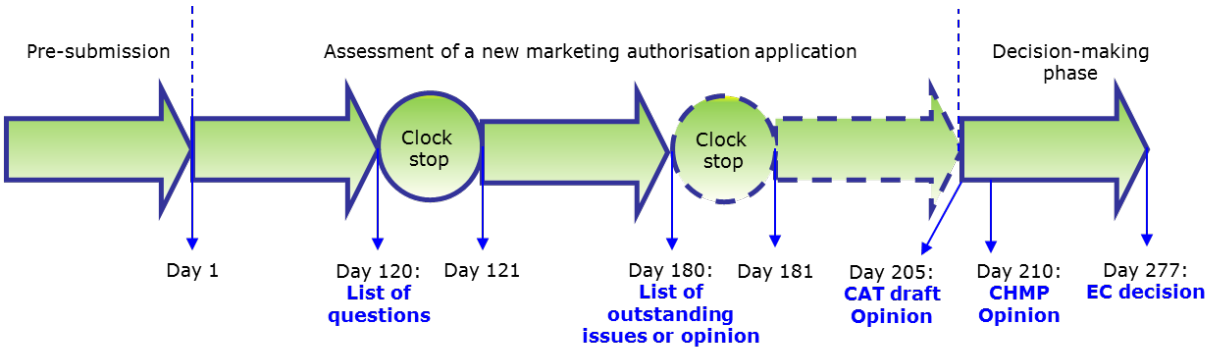
Evaluation of ATMPs (section 2)

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/

10. List of participants

including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 22-24 April 2020 virtual 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	Austria	No interests declared	
Claire Beuneu	Member	Belgium	No interests declared	
Belaïd Sekkali	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Mirna Golemovic	Member	Croatia	No interests declared	
Petra Sokol	Alternate	Croatia	No interests declared	
Isavella Kyriakidou	Alternate (replacing CAT member)	Cyprus	No interests declared	
Ivana Haunerova	Member	Czech Republic	No interests declared	
Anne Pastoft	Member	Denmark	No interests declared	
Toivo Maimets	Member	Estonia	No interests declared	
Pille Saalik	Alternate	Estonia	No interests declared	
Heli Suila	Member	Finland	No interests declared	
Violaine Closson	Member	France	No interests declared	
Nathalie Morgensztejn	Alternate	France	No interests declared	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Angeliki Rompoti	Alternate (replacing CAT member)	Greece	No interests declared	
Katalin Lengyel	Member	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	
Niamh Curran	Alternate	Ireland	No restrictions applicable to this meeting	
Paolo Gasparini	Member	Italy	No interests declared	
Una Riekstina	Member	Latvia	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Romaldas Mačiulaitis	Member (CHMP member)	Lithuania	No interests declared	
Guy Berchem	Member (to CHMP representative).Replacing CAT member	Luxembourg	No restrictions applicable to this meeting	
Anthony Samuel	Alternate (to CHMP representative).Replacing CAT member.	Malta	No interests declared	
Carla Herberts	Member	Netherlands	No interests declared	
Hans Ovelgönne	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	
Maria Isabel Borba Vieira	Alternate (to CHMP representative).Replacing CAT member.	Portugal	No interests declared	
Gianina-Nicoleta Andrei	Alternate (replacing CAT member)	Romania	No interests declared	
Alexandra Padova	Alternate (replacing CAT member)	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	
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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	
Alessandro Aiuti	Member	Healthcare Professionals' Representative	Restrictions applicable to this meeting.	
Kerstin Sollerbrant	Member	Patients' Representative	No interests declared	
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	
Roland Pochet	Alternate	Patients' Representative	No interests declared	
Barbara Bonamassa	Expert	AIFA-IT	No interests declared	
Hans Hillege	Expert	CBG-MEB-NL	No interests declared	
Tineke va den Hoorn	Expert	CBG-MEB-NL	No interests declared	
Marcel Kwa	Expert	CBG-MEB-NL	No interests declared	
Marcel Hoefnagel	Expert	CBG-MEB-NL	No interests declared	
Emmely de Vries	Expert	CBG-MEB-NL	No interests declared	
Ria Nibbeling	Expert	CBG-MEB-NL	No interests declared	
Marja van de Bovenkamp	Expert	CBG-MEB-NL	No interests declared	
Janneke van Leeuwen	Expert	CBG-MEB-NL	No interests declared	
Jan Span	Expert	CBG-MEB-NL	No interests declared	
Charlotte de Wolf	Expert	CBG-MEB-NL	No interests declared	
Juliane Rau	Expert	PEI-DE	No interests declared	
Beate Mosl	Expert	PEI-DE	No restrictions applicable to this meeting	
Daniela Melchiorri	Expert	AIFA-IT	No restrictions applicable to this meeting	
Svetlana Lorenzano	Expert	AIFA-IT	No restrictions applicable to this meeting	
Antonella Isgro	Expert	AIFA-IT	No interests declared	
Nick Lee	Expert	HPRA-IE	No restrictions applicable to this meeting	
Katrin Féchir	Expert	PEI-DE	No interests declared	
Caoimhin Concannon	Expert	HPRA-IE	No interests declared	
Wiebke Hoppensack	Expert	PEI-DE	No interests declared	
A representative from the European Commission attended the meeting				
Meeting run with support from relevant EMA staff				

* Experts were only evaluated against the agenda topics or activities they participated in.