17 June 2020
EMA/CAT/345764/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 18-20 May 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 18-20 May 2020 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes of 22-24 April 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. 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 - 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 120 list of questions

Action: for adoption

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

The list of questions was adopted, together with the evaluation timetable.

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

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

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. The BWP report was presented.

The list of questions was adopted, together with the evaluation timetable.

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

No items

2.7. New applications

No items

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. Alofisel - darvadstrocel - Orphan - EMEA/H/C/004258/II/0016/G

Takeda Pharma A/S

Rapporteur: Lisbeth Barkholt

Scope: quality: Opinion

Action: for adoption

The opinion was adopted.

2.11.2. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0021/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: quality: Opinion

Action: for adoption

The opinion was adopted.

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

CO.DON AG

Rapporteur: Lisbeth Barkholt

Scope: safety: Opinion
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

Request for Supplementary Information adopted on 24.04.2020.

The opinion was adopted.

2.11.4. Spherox - spheroids of human autologous matrix-associated chondrocytes - EMEA/H/C/002736/II/0016

CO.DON AG

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

Scope: safety: Opinion
Update of the RMP to bring it in line with GVP Module V Rev. 2 template.
The educational materials described in Annex II have been updated accordingly.

Action: for adoption

Note: this is an update of the RMP, which has been agreed by PRAC. No discussion in CAT is expected.

The opinion was adopted.

2.11.5. 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/0026

Orchard Therapeutics (Netherlands) BV

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

Scope: safety: Opinion
Submission of an updated RMP version 2.0 in order to introduce changes to the design of the post-authorisation study STRIM-002 to reflect a change in the proposed RIS analysis methodology and shifting the timelines.

Action: for adoption

The Rapporteur provided feedback from this variation. The change in methodology results in a delay in timelines. The opinion was adopted.

2.11.6. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0021

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Anette Kirstine Stark

Scope: safety: Opinion
Submission of a variation to update sections 4.2 and 4.4, of the SmPC to update the dose of tocilizumab to one dose instead of four doses in order to manage the Cytokine Release Syndrome. In addition, treatment centres should have access to an additional dose within 8 hours of each previous dose. Additional changes to the SmPC have been made with regards to the wording on GMO requirements. The Annex II, III, PL and RMP have been updated accordingly

Action: for adoption

The opinion was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/R/0039

Amgen Europe B.V.

Rapporteur: Olli Tenhunen, Co-Rapporteur: Rune Kjeken, CHMP Coordinators: Tuomo Lapveteläinen, Ingrid Wang; PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: 5-year Renewal of Marketing Authorisation. RSI

Action: for adoption

The assessment of the renewal was presented and feedback was given from the PRAC discussion. The RSI was adopted.

2.13.2. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/008

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, CHMP Coordinator: Jan Mueller-Berghaus

Scope: quality

Action: for adoption

The Recommendation is not closed.

2.13.3. Zolgensma – onasemnogene abeparvoved – Orphan – EMEA/H/C/004750

AveXis EU Limited

Rapporteur: Hans Ovelgönne, Co-Rapporteur: Egbert Flory

Scope: Letter from the MAH of 8 May 2020

Action: for discussion

CAT discussed the letter from the MAH. EMA will prepare a response to the MAH, in line with the above discussion.

EMA will prepare a response to the MAH, in line with the above discussion.

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:	20.05.2020
-Draft CAT co-ordinator's report:	04.06.2020
-ITF peer-review comments:	10.06.2020
-Revised scientific recommendation:	12.06.2020
-Adoption of scientific recommendation by CAT:	19.06.2020

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Allogeneic CD34+-enhanced cell suspension derived from umbilical cord blood

Intended for the treatment of patients with inherited metabolic disorders [cerebral adrenoleukodystrophy, Hurler syndrome] where haematopoietic stem cell transplant is indicated

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Homogenate of antlerogenic stem cells

Intended for the treatment of chronic obstructive pulmonary disease, bronchial asthma

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Aggregates of defined size of human embryonic stem cell derived insulin secreting pancreatic beta cells, encapsulated within an encapsulation device

Intended for the treatment of type I diabetes mellitus

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.4. Autologous adipose-derived mesenchymal stem cells, cartilage lesions

Intended for the treatment of cartilage lesions

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.5. Wharton's jelly derived mesenchymal cells, myelitis

Intended for the treatment of myelitis

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, meningitis

Intended for the treatment of meningitis

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 cells, meningomyelocele

Intended for the treatment of meningomyelocele, myelomeningocele, spina bifida

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 cells, cerebellum disease

Intended for the treatment of cerebellum disease

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 cells, encephalitis

Intended for the treatment of encephalitis

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 cells, Krabbe disease

Intended for the treatment of Globoid cell leukodystrophy (Krabbe disease)

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 cells, osteoarthritis

Intended for the treatment of osteoarthritis

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 cells, spinal and bulbar muscular atrophy

Intended for the treatment of spinal and bulbar muscular 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. Recombinant adeno-associated viral vector (serotype 8) carrying an optimised gene for human cyclic nucleotide gated channel subunit beta 3 (CNGB3) protein - H0005013

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

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 8 June 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. Genetically modified Lactococcus lactis strain, engineered to secrete human pro-insulin and human IL-10 - H0005671

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

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 8 June 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. Autologous CD34+ cells transduced with a lentiviral vector encoding a modified γ-globin gene - H0005672

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

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 8 June 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. 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: 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 8 June 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. 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: 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 8 June 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.

4.2.6. Wharton's jelly derived mesenchymal cells – H0005673

Intended for the treatment of patients with COVID-19 infections

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 8 June 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, COVID-19 - H0005674

Intended for the treatment of patients with COVID-19 infections

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 8 June 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, Optic atrophy - H0005688

Intended for the treatment of optic atrophy

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 8 June 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, IFAP syndrome - H0005689

Intended for the treatment of patients with Ichthyosis follicularis with alopecia and photophobia (IFAP) 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 8 June 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, bone marrow transplant rejection - H0005690

Intended for the treatment of bone marrow transplant rejection

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 8 June 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, secondary graft failure - H0005691

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

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 8 June 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.

4.2.12. Wharton's jelly derived mesenchymal stem cell, progressive supranuclear palsy - H0005692

Intended for the treatment of progressive supranuclear 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 8 June 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.

4.2.13. Wharton's jelly derived mesenchymal stem cell, Multiple system atrophy - H0005693

Intended for the treatment of multiple system atrophy

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

Intended for the treatment of diabetic foot syndrome

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

Action: for adoption

4.4. Postponed Finalisation of procedure

4.4.1. 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: Comments received by the European Commission. Final ATMP scientific recommendation

Action: for adoption

Request for List of Questions adopted on 20.03.2020.

The revised report was discussed and adopted.

4.4.2. 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: Comments received by the European Commission. Final ATMP scientific recommendation

Action: for adoption

The revised report was adopted.

4.4.3. Leuco platelet enriched plasma – H0005630

Intended for the treatment of ulcers, chronic wounds

Scope: Comments received by the European Commission. Final ATMP scientific recommendation

Action: for adoption

The revised report was adopted.

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

Action: for information

The information was noted.

4.4.5. Wharton's jelly derived mesenchymal stem cell, drug resistant epilepsy - H0005662

Intended for the treatment of drug resistant epilepsy

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

Action: for information

The information was noted.

4.4.6. Wharton's jelly derived mesenchymal stem cell, Behcet disease - H0005663

Intended for the treatment of Becht disease

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

Action: for information

The information was noted.

4.4.7. Wharton's jelly derived mesenchymal stem cell, choroideremia - H0005664

Intended for the treatment of choroideremia

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

Action: for information

The information was noted.

4.4.8. Wharton's jelly derived mesenchymal stem cell, foetal alcohol syndrome - H0005665

intended for the treatment of foetal alcohol syndrome

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

Action: for information

The information was noted.

4.4.9. Wharton's jelly derived mesenchymal stem cell, frontotemporal dementia - H0005666

Intended for the treatment of frontotemporal dementia

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

Action: for information

The information was noted.

4.4.10. Wharton's jelly derived mesenchymal stem cell, progressive bulbar palsy - H0005667

Itended for the treatment of progressive bulbar palsy

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

Action: for information

The information was noted.

4.4.11. Wharton's jelly derived mesenchymal stem cell, vitelliform macular degeneration - H0005668

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

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

Action: for information

The information was noted.

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

Timetable:
-Final Briefing Package: 05.06.2020
-Start of the procedure at SAWP: 11.06.2020
-CAT report due by: 14.06.2020
-CAT recommendation: 19.06.2020

5.2. CAT reports

5.3. List of Issues

No items

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

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

No items

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

Romania: Simona Badoi – membership mandate started on 07 May 2020

Action: for information

The information was noted.

7.1.2. Procedure on voting remotely with Adobe Connect

Scope: improved procedure on the voting procedure, management and recording

Action: for information

The information was noted.

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 28-30 April 2020 meeting

Action: for information

The information was noted.

7.2.2. Scientific Coordinator Board (SciCoBo) – meeting of 7 May 2020

CAT: Martina Schüßler-Lenz

Scope: feedback on the outcome of the SciCoBo meeting that will take place on 7 May 2020

Action: for information

The CAT chair provided a short feedback from the discussion in the SciCoBo meeting. The main topic on the agenda was on COVID-19 related activities at EMA (see 7.8.1).

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

7.3.1. GMP/GCP inspections for ATMPs

Scope:
-GMP/GCP inspections during the COVID-19 pandemic
-Mutual Recognition Agreement (MRA) for ATMPs

Action: for discussion

Feedback was provided on how to perform GMP/GCP inspections (remotely) when travel restrictions do not allow for inspection of the manufacturing facility / investigators or sponsor site. It was also clarified that ATMPs are excluded from the scope of MRA between EU and US.

7.3.2. Scientific advice for ATMPs

Scope: new procedure for providing CAT input to SAWP on scientific advice for ATMPs

Action: for information

Note: CAT members can provided comments on the proposed new procedure by 12 June 2020 (comments to: CATsecretariat@ema.europa.eu). A formal discussion will be scheduled for the June CAT meeting.

Postponed to the next CAT meeting.

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

Scope: meeting Summary from the PCWP/HCPWP joint meeting, held on 3-4 March 2020

Action: for information

Postponed to the next CAT meeting.

7.4. Cooperation within the EU regulatory network

None

7.5. Cooperation with international regulators

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

CAT: Martina Schüssler-Lenz, Carla Herberts, Ilona Reischl, Pille Saalik

Scope: feedback on the teleconference that took place on 30 April 2020

Action: for information

Postponed to the next CAT meeting.

7.6. CAT work plan

None

7.7. Planning and reporting

None

7.8. Others

7.8.1. EMA initiative to support the development of medicines against Covid-19

Scope: EMA initiative to support the development of medicines against Covid-19

Action: for information

A presentation was given by EMA on the EMA initiative to support development of vaccines and therapeutics against Covid-19. Initiatives include: rapid scientific advice for developers of such medicines (open to commercial and non-commercial developers) and additional regulatory flexibility (e.g. with regards to GMP, manufacturing, rapid assessment and approval, labelling).

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

Postponed to the next CAT meeting.

8.1. American Society of Gene & Cell Therapy (ASGCT)'s annual meeting, 11th May 2020, Boston MS (USA)

Scope: feedback from Jan Mueller-Berghaus who presented at the ASGCT pre-meeting workshop: 'Commercialization I Workshop'

Action: for information

Postponed to the next CAT meeting.

Date of next CAT meeting:

17-19 June 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

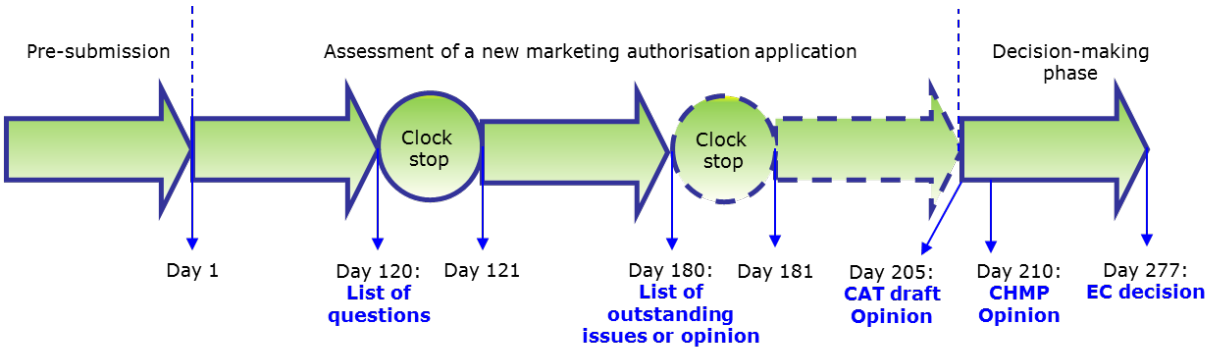
Evaluation of ATMPs (section 2)

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 18-20 May 2020 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	
Silke Dorner	Alternate	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	
Isavella Kyriakidou	Alternate (replacing member)	Cyprus	No interests declared	
Ivana Haunerova	Member	Czech Republic	No interests declared	
Ondřej Palan	Alternate	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	
Olli Tenhunen	Alternate	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 member)	Greece	No interests declared	
Katalin Lengyel	Member	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	
Niamh Curran	Alternate (replacing member)	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	Luxembourg	No restrictions applicable to this meeting	
Anthony Samuel	Alternate (to CHMP representative) (replacing member)	Malta	No interests declared	
Carla Herberts	Member	Netherlands	No interests declared	
Johannes 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 member)	Portugal	No interests declared	
Simona Badoi	Member	Romania	No interests declared	
Lukas Slovak	Member	Slovakia	No interests declared	
Alexandra Padova	Alternate	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	
Marcos Timón	Alternate (to CHMP representative) (replacing member)	Spain	No interests declared	
Lisbeth Barkholt	Member	Sweden	No interests declared	
Björn Carlsson	Alternate	Sweden	No interests declared	
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	
Alessandro Aiuti	Member	Healthcare Professionals' Representative	Restrictions application to this meeting.	
Kerstin Sollerbrant	Member	Patients' Representative	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Lydie Meheus	Alternate	Patients' Representative	No interests declared	
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	
Roland Pochet	Alternate	Patients' Representative	No interests declared	
Michael Rosu-Myles	Observer/Alternate	European Directorate for the Quality of Medicine & HealthCare(EDQM)		
Barbara Bonamassa	Expert - virtual by Adobe Connect	AIFA-IT	No interests declared	
Maria Lüttgen	Expert - virtual by Adobe Connect	MPA-SE	No restrictions applicable to this meeting	
Karin Nylén	Expert - virtual by Adobe Connect	MPA-SE	No interests declared	
Peter Kiely	Expert - virtual by Adobe Connect	HPRA-EI	No interests declared	
Philipp Berg	Expert - virtual by Adobe Connect	PEI-DE	No interests declared	
Ingrid Wang	Expert - virtual by Adobe Connect	NOMA-NO	No interests declared	
Attila Sebe	Expert - virtual by Adobe Connect	PEI-DE	No interests declared	
Beate Mosl	Expert - virtual by Adobe Connect	PEI-DE	No restrictions applicable to this meeting	
Jürgen Scherer	Expert - virtual by Adobe Connect	PEI-DE	No interests declared	
Menno van der Elst	Expert - virtual by Adobe Connect	CBG-MEB-NL	No interests declared	
Marcel Hoefnagel	Expert - virtual by Adobe Connect			
Helga Haugom Olsen	Expert - virtual by Adobe Connect	NOMA-NO	No interests declared	
Fabrice Eroukhmanoff	Expert - virtual by Adobe Connect	NOMA-NO	No restrictions applicable to this meeting	
Ingebjørg Buajordet	Expert - virtual by Adobe Connect	NOMA-NO	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Kristine Moltu	Expert - virtual by Adobe Connect	NOMA-NO	No interests declared	
Greger Abrahamsen	Expert - virtual by Adobe Connect	NOMA-NO	No interests declared	
Gabriele Ruppert-Seipp	Expert - virtual by Adobe Connect	PEI-DE	No interests declared	
Sonja Schönefeld	Expert - virtual by Adobe Connect	PEI-DE	No interests declared	
Jorge Camarero-Jiménez	Expert - virtual by Adobe Connect	AEMPS-ES	No restrictions applicable to this meeting	
Blanca García-Ochoa	Expert - virtual by Adobe Connect	AEMPS-ES	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.