8 November 2019
EMA/CAT/40164/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

# Committee for Advanced Therapies (CAT)

## Minutes of the meeting on 09-11 October 2019

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

**Health and safety information**

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

**Disclaimers**

Some of the information contained in 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).

**Official address** Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
**Address for visits and deliveries** Refer to www.ema.europa.eu/how-to-find-us
**Send us a question** Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

# Table of contents

# 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. 22 or more members were present in the room). No new or additional interests or restrictions were declared.

The CAT chair welcomed the new alternate from Cyprus (Isavella Kyriakidou), who attended the CAT for the first time.

## 1.2. Adoption of agenda

The CAT agenda for 09-11 October 2019 meeting was adopted.

## 1.3. Adoption of the minutes

The CAT minutes for 11-13 September 2019 meeting were adopted.

# 2. Evaluation of ATMPs

## 2.1. Opinions

No items

## 2.2. Oral explanations

### 2.2.1. Viable T-cells - Orphan - EMEA/H/C/002397

Kiadis Pharma Netherlands B.V.; indicated as an adjunctive treatment to a haploidentical, T-cell depleted haematopoietic stem cell transplantation (HSCT) in adult patients with high-risk haematological malignancies in complete remission.

Scope: oral explanation to take place on Thursday 10th, 09:00hrs

**Action:** for discussion

List of Outstanding Issues adopted on 21.06.2019, 14.09.2018, 25.05.2018. List of Questions adopted on 08.09.2017.

The Rapporteur presented the assessment of the responses to the list of outstanding issues and the questions to be addressed by the applicant during the oral explanation (OE). The OE

in front of CAT took place on 10 October 2019. Further to the discussion, a trend vote was taken.

The CAT Rapporteurs will update the assessment report in line with the OE, discussion and trend vote for adoption during the November CAT meeting.

## 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

No items

## 2.7. New applications

## 2.8. Withdrawal of initial marking 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. 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/0022

Orchard Therapeutics (Netherlands) BV

Rapporteur: Sol Ruiz

Scope: safety: submission of an updated RMP version 2.0 in order to introduce changes to the design of the post-authorisation study STRIM-002, from a prospective to a retrospective study. Following additional minor changes to the RMP are also included: Update of the RMP in line with EMA Rev.2.0.1 template; update of the RMP to make the necessary amendments to the name of the MAH following the MAH transfer; update of the data in the RMP in line with the updated data lock point; update of timelines for the STRIM-001 study. Opinion

**Action:** for adoption

The opinion was adopted.

### 2.11.2. YESCARTA - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0011

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: quality Opinion

**Action:** for adoption

Request for Supplementary Information adopted on 13.09.2019.

See also 2.12.2 and 2.12.3.

The opinion was adopted.

### 2.11.3. Zalmoxis - nalotimagene carmaleucel - Orphan - EMEA/H/C/002801/II/0016

MolMed S.p.A

Rapporteur: Carla Herberts; PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: Proposal from the MAH to terminate the study TK008 (specific obligation for the CMA) and replace it with study TK013.

**Action:** for information

Request for Supplementary Information adopted on 19.07.2019

CAT noted the withdrawal of the type II variation.

### 2.11.4. Zalmoxis - nalotimagene carmaleucel - Orphan - EMEA/H/C/002801/R/0015

MolMed S.p.A

Rapporteur: Carla Herberts; PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: update on the status of the annual renewal.

**Action:** for discussion

CAT noted the confirmation from the European Commission of the withdrawal of the marketing authorisation of Zalmoxis, due to commercial reasons. The company's decision takes into account the overall results of the interim analysis of the study TK008.

### 2.11.5. Zynteglo - Orphan - EMEA/H/C/003691/II/0001-G

Bluebird bio (Netherlands)

Rapporteur: Carla Herberts

Scope: quality:Opinion

**Action:** for adoption

The Rapporteurs presented in detail the background and their assessment of this variation.

The responses from the MAH to the list of outstanding issues (adopted by CAT in September) were considered acceptable by the Rapporteur and the BWP.
The opinion was adopted.

## 2.12. Other Post-Authorisation Activities

### 2.12.1. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090

Novartis Europharm Limited

Rapporteur: Rune Kjeken; CHMP coordinator: Bjorg Bolstad; PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: CAR-T cell-imposed PASS study: issues with implementation and current status; feedback from PRAC discussion.

**Action:** for discussion

Feedback was provided from discussion in PRAC on the proposal for the PASS study and on the proposal for CAR-T cell PASS interim reports to PRAC. It was noted that the study aims to start by the end of this year.

### 2.12.2. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/MEA/003.1

Kite Pharma EU B.V.

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

Scope: Submission of an updated protocol for study KT-EU-471-0116 assessing Health Care Provider's awareness and knowledge of the routine and additional Risk Minimisation Measures addressing the key important identified risks associated with the use of Yescarta and their understanding of the handling and administration of Yescarta. Responses to PRAC RSI of 14 June 2019 are included.

**Action:** for adoption

See also 2.11.2. and 2.12.3.

Feedback was provided from outcome of the PRAC review, agreeing with the protocol for the study KT-EU-471-0116. The conclusion was endorsed by CAT.

### 2.12.3. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480

Kite Pharma EU B.V.

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

Scope: CAR-T cell-imposed PASS studies: issues with implementation and current status; feedback from PRAC discussion.

**Action:** for discussion

See 2.12.1 on the PRAC discussion on the CAR-T PASS study.

### 2.12.4. Holoclar - *ex vivo* expanded autologous human corneal epithelial cells containing stem cells - Orphan - EMEA/H/C/002450/R/0026

Chiesi Farmaceutici S.p.A.

Rapporteur: Egbert Flory; CHMP Coordinator: Jan Mueller-Berghaus; PRAC Rapporteur: Julie Williams

Scope: 5$^{th}$ annual reassessment for the renewal of marketing authorisation. RSI

**Action:** for adoption

The Rapporteur presented his assessment of the renewal of the MA of Holoclar. .

CAT adopted the RSI for the renewal.

## 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

## 3.3. New Applications

# 4. Scientific Recommendation on Classification of ATMPs

## 4.1. New requests – Appointment of CAT Coordinator

### 4.1.1. Recombinant adeno associated viral vector serotype 9 containing the human CLN6 gene - 005491

Intended for the treatment of neuronal ceroid lipofuscinosis type 6 (CLN6) disease (CLN6 Batten disease)

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed

### 4.1.2. Recombinant adeno associated viral vector serotype 9 containing the human CLN3 gene – H0005492

Intended for the treatment of neuronal ceroid lipofuscinosis type 3 (CLN3) disease (CLN3 Batten disease)

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed

### 4.1.3. Wharton's Jelly derived mesenchymal stem cell, Alopecia areata – H0005494

Intended for the treatment of alopecia areata

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed

### 4.1.4. Wharton's Jelly derived mesenchymal stem cell, Pervasive developmental disorder – H0005502

Intended for the treatment of pervasive developmental disorder

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 stem cell, Cerebral infarction – H0005503

Intended for the treatment of cerebral infarction

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 stem cell, Development delay – H0005504

Intended for the treatment of development delay

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, Diabetes – H0005505

Intended for the treatment of diabetes

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, Muscular dystrophy – H0005506

Intended for the treatment of muscular dystrophy

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, Endometrial atrophy – H0005507

Intended for the treatment of endometrial atrophy

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, Multiple sclerosis – H0005508

Intended for the treatment of multiple sclerosis

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, Optic neuropathy – H0005509

Intended for the treatment of optic neuropathy

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, Premature ovarian failure – H0005510

Intended for the treatment of premature ovarian failure

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, Retinitis pigmentosa – H0005511

Intended for the treatment of retinitis pigmentosa

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed

### 4.1.14. Wharton's Jelly derived mesenchymal stem cell, Spina bifida – H0005512

Intended for the treatment of spina bifida

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed

### 4.1.15. Wharton's Jelly derived mesenchymal stem cell, Spinal cord injury – H0005513

Intended for the treatment of spinal cord injury

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed

### 4.1.16. Wharton's Jelly derived mesenchymal stem cell, Stargardt disease – H0005514

Intended for the treatment of Stargardt disease

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 virus (AAV) vector based on the AAV serotype hu37 (AAVhu37) expressing human Factor VIII - H0005490

Intended for the treatment of haemophilia A

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 25 October 2019.

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)

No items

## 4.4. Finalisation of procedure

### 4.4.1. Adipose-derived mesenchymal stem cells - H0005458

Intended for the treatment of diabetic foot ulcers (DFU)

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

**Action:** for information

The information was noted.

### 4.4.2. Human allogeneic melanoma cells Mich1H6 and Mich2H6 - H0005459

Intended for the treatment of advanced melanoma (stage IIIB-IV)

Scope: comments raised by the European Commission. Final ATMP scientific recommendation

**Action:** for information

The information was noted.

### 4.4.3. CD1c(BDCA-1)+/CD141(BDCA-3)+ myeloid dendritic cells - H0005460

Intended for the treatment of patients with advanced, pre-treated solid tumours with injectable metastases

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

**Action:** for information

The information was noted.

### 4.4.4. Human autologous Adipose Tissue - derived Mesenchymal Stem / Stromal Cells (AT-MSCs) – H0005461

Intended for the treatment of bone and cartilage defects including osteoarthritis

Scope: comments raised by the European Commission. Final ATMP scientific recommendation

**Action:** for information

The information was noted.

### 4.4.5. Oncolytic adenovirus – H0005463

Intended for the treatment-naïve patients with localized prostate cancer

Scope: comments raised by the European Commission. Final ATMP scientific recommendation

**Action:** for information

The information was noted.

### 4.4.6. Platelet-Rich Stroma (PRS) - combination of platelet-rich plasma and stromal vascular fraction – H0005430

Intended for wound healing as additional therapy to fistula surgery in patients with complex

and therapy refractory perianal fistula

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

**Action:** for information

The information was noted.

#### 4.4.7. *In vitro* transcribed mRNA encoding the human insulin-like growth factor 1 (IGF-1) – H0005462

Intended for the treatment of skeletal muscle injury

Scope: no comments raised by the European Commission. Final 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

### 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

No items

### 6.2. ITF briefing meetings in the field of ATMPs

No items

### 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. Strategic Review & Learning meeting – Helsinki, Finland, 21 – 22 November 2019

CAT: Heli Suila

Scope: draft agenda for the CAT-only session

**Action:** for discussion

Note: CAT at its September meeting already made proposals for the agenda of the joint CAT-PDCO-COMP session and the joint CAT-COMP session. Awaiting feedback from COMP and PDCO.

CAT discussed the draft programme and identified CAT speakers for different sessions.

CAT subsequently identified topics for discussion in the CAT-only session . CAT members were asked to propose additional topics for the CAT-only session.

## 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 September 2019 meeting

**Action:** for information

The information was noted.

### 7.2.2. Scientific Coordination Board (SciCoBo) – meeting of 25 September 2019

CAT: Martina Schüßler-Lenz

Scope: feedback on the outcome of the SciCoBo meeting that took place on 25 September 2019

**Action:** for information

The CAT chair provided feedback from the discussion at the SciCoBo meeting.

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

### 7.3.1. Questions & Answers on comparability

CAT drafting group: Margarida Menezes, Ilona Reischl, Ivana Haunerová, Heli Suila, Barbara Bonamassa

Scope: draft questions and answers (Q&A) document

**Action:** for discussion

The draft document with the Q&A on comparability for ATMPs was presented. CAT members were asked to provide comments by 24 October 2019. The Q&A will be sent to BWP for discussion and comments.

## 7.4. Cooperation within the EU regulatory network

### 7.4.1. Interplay with GMO framework: new initiatives

Scope: feedback from a meeting between the GMO and medicines authorities on environmental risk assessment of GTMPs in clinical trials

**Action:** for information

The Commission representative provided detailed feedback.

## 7.5. Cooperation with international regulators

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

CAT: Martina Schüßler-Lenz

Scope: preparation of the next ATMP cluster

**Action:** for discussion

CAT noted the proposals for improvement of the interactions / discussion during the ATMP cluster teleconference calls.

### 7.5.2. Blood cluster teleconference with US-FDA and Health Canada

Scope: agenda of the blood cluster TC

**Action:** for information
CAT noted the agenda of the blood cluster TC and the two ATMP related topics thereon.

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

CAT: Pille Säälik

Scope: agenda of the IPRP-gene therapy working group

**Action:** for information

CAT was informed of the date and the agenda of the next IPRP teleconference. Dariusz Sladowski and the Commission Representative expressed interest to join this teleconference.

## 7.6. CAT work plan

### 7.6.1. CAT work plan 2020

CAT: Martina Schüßler-Lenz

**Action:** for discussion

The draft CAT work plan was discussed. Priorities were discussed, some of the objectives for 2020 were rephrased and CAT members were identified to contribute to the work plan topics. Further discussion will take place at the November CAT meeting.

## 7.7. Planning and reporting

None

## 7.8. Others

### 7.8.1. Curriculum on Advanced Therapies Medicinal Products (ATMPs) training

CAT: Ilona Reischl

Scope: plan of trainings for the end of 2019 and beginning of 2020

**Action:** for discussion

The current ATMP curriculum and the list of proposed trainings were presented. CAT discussed the timing to restart the training of CAT members and NCA assessors/experts dealing with ATMPs and agreed to start scheduling these training sessions from beginning of 2020 onwards; it was suggested to organise a training session on quality aspects for cell-based ATMPs by Margarida Menezes Ferreira in the margins of the November or December CAT meeting.

### 7.8.2. Harmonisation of communication subject naming convention received from NCAs

**Action:** for information

EMA colleagues presented the standardised naming to be included in the subject folder of product related e-mail messages that are sent to the EMA

# 8. Any other business

No items

Date of next CAT meeting:

06-08 November 2019

## 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

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

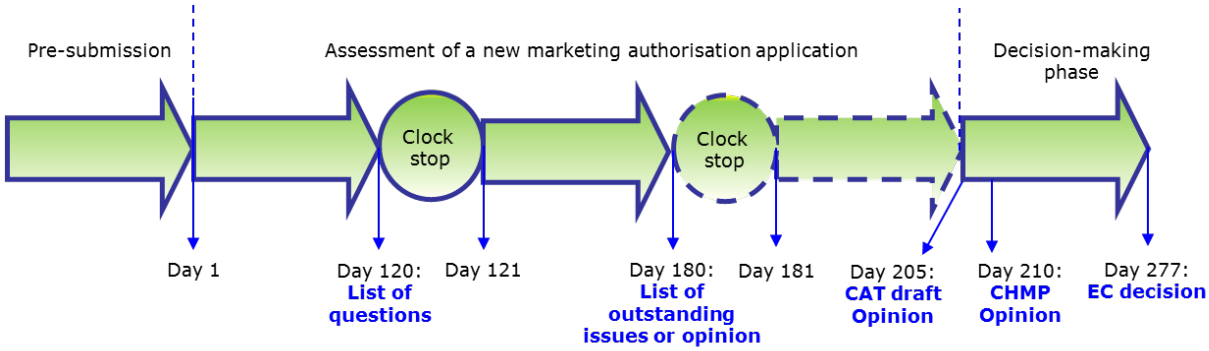
## Evaluation of ATMPs (section 2)

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 09-11 October 2019 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 | |
| Evelina Shumkova | Alternate | Bulgaria | No interests declared | |
| Mirna Golemovic | Member | Croatia | No interests declared | |
| Isavella Kyriakidou | Alternate | 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 | |
| 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 Roboti | Alternate | 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 | Member | Italy | No interests declared | |

| Name | Role | Member state or affiliation | Outcome restriction following evaluation of e-DoI | Topics on agenda for which restrictions apply |
|---|---|---|---|---|
| Gasparini | | | | |
| Giulio Pompilio | Alternate | Italy | No restrictions applicable to this meeting | |
| Una Riekstina | Member | Latvia | No interests declared | |
| Romaldas Mačiulaitis | Member (CHMP member) | Lithuania | No interests declared | |
| Guy Berchem | Member (to CHMP representative) | Luxembourg | No restrictions applicable to this meeting | |
| Carla Herberts | Member | Netherlands | No interests declared | |
| Johannes Hendrikus Ovelgonne | 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 | |
| Margarida Menezes-Ferreira | Alternate (to CHMP representative) | Portugal | No interests declared | |
| Simona Badoi | Member | Romania | No interests declared | |
| Lukas Slovak | 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 | |
| John Johnston | Member | United Kingdom | No interests declared | |
| Bernd Gänsbacher | Member | Healthcare Professionals' Representative | No interests declared | |

| Name | Role | Member state or affiliation | Outcome restriction following evaluation of e-DoI | Topics on agenda for which restrictions apply |
|---|---|---|---|---|
| Alessandro Aiuti | Member | Healthcare Professionals' Representative | No restrictions applicable to this meeting | |
| Alessandra Renieri | Alternate | Healthcare Professionals' Representative | No interests declared | |
| Kerstin Sollerbrant | Member | Patients' Representative | No interests declared | |
| Lydie Meheus | Alternate | Patients' Representative | No interests declared | |
| Kieran Breen | Member | Patients' Representative | No restrictions applicable to this meeting | |
| Karl-Heinz Buchheit | Observer | Conseil de l'Europe | No restrictions applicable to this meeting | |
| Catherine Milne | Observer/Alternate | European Directorate for the Quality of Medicine & HealthCare(EDQM) | No interests declared | |
| Barbara Bonamassa | Expert - in person* | AIFA-IT | No interests declared | |
| Susanne Poley-Ochmann | Expert - via telephone* | PEI-DE | No interests declared | |
| Maren Hammann | Expert - via telephone* | PEI-DE | No interests declared | |
| Anke Zobywalski | Expert - via telephone* | PED-DE | No interests declared | |
| Benjamin Hofner | Expert - via telephone* | PEI-DE | Indirect interests | |
| Alexandra Moreau | Expert - via telephone* | ANSM.SANTE-FR | No interests declared | |
| Paula Hennik | Expert - via telephone* | CBG-MEB-NL | No interests declared | |
| Marja van de Bovenkamp | Expert - via telephone* | CBG-MEB-NL | No interests declared | |
| Daniela Melchiorri | Expert - via telephone* | AIFA-IT | Indirect interests | |
| | | | | |
| 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.