17 March 2017
EMA/CAT/278840/2017
Inspections, Human Medicines Pharmacovigilance & Committees Division

Committee for Advanced Therapies (CAT)

Minutes for the meeting on 15 – 17 February 2017

Election of CAT chair person

Chair: Anabela Luis de Lima Marçal (Head of Committees and Inspections Department, EMA)

Scope: election of Chair to take place on 15 February 2017 at 15.00.

Action: election of CAT chair

The election of the Chairperson took place on 15 February 2017 at 15:00hrs. EMA reminded the CAT members of the Rule of Procedure pertaining to the election of the chair person.

Candidates addressed the CAT.

The election took place in the presence of 27 CAT members that were eligible to vote. Martina Schüßler-Lenz was elected as CAT chair for a period of 3 years.

The newly elected chair thanked Paula Salmikangas, the previous CAT chair, for her contributions to the CAT over the last 3 years.

Martina Schüßler-Lenz thereafter chaired the February meeting.

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Chair: Martina Schüßler-Lenz; Vice-chair: vacant

15 February 2017, 15:00 – 19:00, room 03-F
16 February 2017, 09:00 – 18:30, room 03-F
17 February 2017, 09:00 – 12:00, room 03-F

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).

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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. 22 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 the 15-17 February 2017 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for the 18-20 January 2017 meeting were adopted with an amendment to section 5.4.1.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

2.3.1. Expanded human allogeneic mesenchymal adult stem cells extracted from adipose tissue; Orphan; EMA/H/C/0004258

TiGenix S.A.U.; Treatment of complex perianal fistula(s)

Scope: List of Outstanding Issues

Action: for adoption

The Rapporteurs presented the joint response assessment report. CAT discussed the major objections (MO).

The revised list of outstanding issues was adopted.

2.3.2. Human autologous spheroids of matrix – associated chondrocytes for transplantation; EMA/H/C/0002736

Intended for the repair of symptomatic articular cartilage defects of the knee and hip (International Cartilage Repair Society [ICRS] grade III or IV) with defect sizes up to 10 cm^2 in skeletally mature patients. Treatment is eligible for single as well as multiple adjacent defects. The medicinal product is indicated for adults and adolescents (12-18 years of age) with closed epiphyseal growth plate in the affected joint.

Scope: List of Outstanding Issues

Action: for adoption

The Rapporteurs presented the joint assessment report on the responses following the applicant's LoOIs. .

CAT discussed the major objections (MO) . The list of outstanding issues was adopted.

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

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

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

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

2.11. Type II variations - variation of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008

2.11.1. Imlygic – Talimogene laherparepvec; EMA/H/C/002771/II/0008

Amgen Europe B.V.

Rapporteur: Olli Tenhunen; CHMP Coordinator: Tuomo Lapveteläinen

Scope: quality Request for supplementary information

Action: for adoption

Note: the CAT adopted the evaluation timetable by written procedure in January 2017.

The RSI was adopted.

2.12. Other Post-Authorisation Activities

2.12.1. Glybera – Alipogene tiparvovec; Orphan; EMA/H/C/002145/S/57, ANN 011

UniQure Biopharma B.V.

Rapporteur: Christiane Niederlaender; CHMP Coordinator: Greg Markey; PRAC Rapporteur: Julie Williams

Scope: Opinion

Action: for adoption

The positive opinion on the 4th annual reassessment was adopted by consensus.

2.12.2. Glybera – Alipogene tiparvovec; Orphan; EMA/H/C/002145/SOB002.6

UniQure Biopharma B.V.

Rapporteur: Christiane Niederlaender; CHMP Coordinator: Greg Markey

Scope: RSI. Clinical and PhV: assessment of postprandial chylomicron metabolism in at least 12 patients before 12 months and 24 months after treatment with Glybera to be chosen in addition to the patients included in study CT-AMT.011.02 and eight healthy subjects in the second study.

Action: for adoption

The 2nd RSI was adopted.

2.12.3. Glybera – Alipogene tiparvovec; Orphan; EMA/H/C/002145/SOB002.7

UniQure Biopharma B.V.

Rapporteur: Christiane Niederlaender; CHMP Coordinator: Greg Markey

Scope: Clinical. Assessment of MAH's response to SOB-002.5: open label, multi-centre trial of Glybera (alipogene tiparvovec) for the treatment of lipoprotein lipase deficiency Patients, as adopted in November 2016. Protocol amendment to phase IV clinical trial.

Action: for adoption of an RSI

The RSI was adopted.

2.12.4. Strimvelis - Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence; EMEA/H/C/003854/REC007

UniQure Biopharma B.V.

Rapporteur: Christiane Niederlaender; CHMP Coordinator: Greg Markey

Scope: Quality

Action: timetable for adoption

The review timetable was adopted.

2.12.5. Strimvelis - Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence; EMEA/H/C/003854/REC008

UniQure Biopharma B.V.

Rapporteur: Christiane Niederlaender; CHMP Coordinator: Greg Markey

Scope: Quality

Action: timetable for adoption

The review timetable was adopted.

3. Certification of ATMPs

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

4. Scientific Recommendation on Classification of ATMPs

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

4.1. New requests – Appointment of CAT Coordinators

4.1.1. Implantable continuous glucose monitoring system; EMA/H0004762

Intended for glucose monitoring in diabetes patients

Scope: appointment of CAT Coordinator

Action: for initial discussion

Note: involvement of the EU-Innovation Network / Head of Medicines Agency (HMA) Borderline group on the borderline discussion.

Nominations were received. The CAT member was appointed as CAT coordinator for this procedure.

4.1.2. Autologous bone marrow derived mesenchymal stems cells (MSC); EMA/H0004766

Intended for the treatment of coma (brain injury, stroke)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

Nominations were received. The CAT member was appointed as CAT coordinator for this procedure.

4.1.3. Allogeneic umbilical cord derived mesenchymal stems cells (MSC); EMA/H0004758

Intended for the intervertebral disc degeneration

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

Note: A similar product (MSCs form umbilical cord, adipose tissue or bone marrow) for treatment of amyotrophic lateral sclerosis was classified by CAT in November 2015 .

Nominations were received. The CAT member was appointed as CAT coordinator for this procedure.

4.1.4. Stimulated resistant cells suspension cancer vaccine; EMA/H0004763

Intended for the treatment of colorectal cancer

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

Nominations were received. The CAT member was appointed as CAT coordinator for this procedure.

4.1.5. Recombinant adeno-associated virus serotype 8 thyroid binding globulin recombinant human UGT1A1 enzyme; EMA/H0004757

Intended for the treatment of Crigler-Najjar (CN) syndrome

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

Nominations were received. The CAT member was appointed as CAT coordinator for this procedure.

4.1.6. Oncolytic adenovirus; EMA/H0004767

Intended for the treatment of pancreatic cancer

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

Nominations were received. The CAT member was appointed as CAT coordinator for this procedure.

4.2. Day 30 ATMP scientific recommendation

4.2.1. Autologous tumour-infiltrating lymphocytes; EMA/H0004741

Intended for the treatment of metastatic melanoma

Scope: scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments

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 ATMP scientific recommendation (following list of questions)

No items

4.4. Finalisation of procedure

4.4.1. Adeno-associated virus type 8 encoding the human myotubularin (MTM1) gene; EMA/H0004719

Intended for the treatment of X-linked myotubular myopathy (XLMTM)

Scope: no comments raised by the European Commission

Action: for information

The CAT noted this information.

4.4.2. Messenger RNA (mRNA) components encoding six non-small cell lung cancer associated antigens; EMA/H0004716

Intended for the treatment of non-small cell lung cancer (NSCLC)

Scope: no comments raised by the European Commission

Action: for information

The CAT noted this information.

4.4.3. mRNA construct encoding the wild type human OX40L protein; EMA/H0004726

Intended for the treatment of solid tumours

Scope: no comments raised by the European Commission

Action: for information

The CAT noted this information.

4.4.4. Bone marrow derived mesenchymal cells (MSCs); EMA/H0004718

Intended for the treatment of acute graft *versus* host disease

Scope: no comments raised by the European Commission

Action: for information

The CAT noted this information.

4.4.5. Allogeneic cytomegalovirus-specific cytotoxic T lymphocytes (CMV-CTLs) - Orphan; EMA/H0004717

Intended for the treatment of cytomegalovirus-associated viraemia or disease after allogeneic haematopoietic cell transplant or solid organ transplant

Scope: no comments raised by the European Commission

Action: for information

The CAT noted this information.

4.5. Follow-up and guidance

4.5.1. Leukocytes with cancer killing activity; EMA/H0004704

Intended for the treatment of metastatic pancreatic ductal adeno carcinoma

Scope: comments received from applicant on classification

Action: for discussion

Note: the CAT classified this procedure as non-ATMP at its December 2016 plenary meeting.

Further to the letter from the applicant on the outcome of the CAT Classification for their product, CAT proposed that the applicant should resubmit the application for ATMP classification with new information included. The CAT coordinator appointed for the original application will evaluate the application.

5. Scientific Advice (SA)

Disclosure of 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 Coordinators

5.2. CAT Rapporteurs' reports

5.3. List of Issues

5.4. Finalisation of SA procedures

6. Pre-Authorisation Activities

Disclosure of 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. Month 3 – Nomination of Rapporteurs

6.3.5. Ongoing support

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. Election for Chairperson to CAT

EMA: Anabela Luis de Lima Marçal,

Scope: election of Chair took place on 15 February 2017 at 15.00.

Action: election of CAT chair

Martina Schüßler-Lenz (DE) was elected as CAT chair.
The vice chair election will take place at the March CAT meeting.

7.1.2. Organisational change in the Scientific Committees Secretariat Service (SCS)

Scope: Thorsten Olski was appointed as the head of the Scientific Committees Secretariat service at EMA, replacing Nikolaus Kriz.

Action: for information

CAT noted the organisational changes and welcomed T Olski.

There was a short discussion on resources to CAT and to the CAT secretariat.

7.1.3. Strategic Review & Learning meeting

CAT Strategic Review & Learning meeting will take place in Gozo, Malta on 1-2 June 2017 under the auspices of the Maltese Presidency of the Council of the European Union

Scope: draft programme

Action: for discussion

The draft programme for the upcoming CAT Strategic Review & Learning meeting was presented.

CAT members were asked to provide information on who is planning to attend this meeting, as this will influence the topic on the programme. A further discussion of the programme will take place at the March 2017 CAT meeting.

7.1.4. Survey to committees members on the service provided by the Scientific Committees Service

Scope: findings of the survey that was conducted in July 2016

Action: for information

EMA provided feedback on the outcome of the above mentioned survey.

7.1.5. Combination packs requirements for ATMPs

CAT resources: Claire Beuneu, Ilona Reischl and Violaine Closson

Scope: draft eligibility criteria for combination packs, updated to reflect the specificities of ATMPs.

Action: for discussion

EMA presented the draft eligibility criteria for ATMP containing combination packs.

The topic will be further discussed at the March 2017 CAT meeting after further discussion in the CAT drafting group.

7.1.6. GMP requirements for ATMPs

Scope: feedback from the European Commission

Action: for information

The European Commission representative reported back from the face to face meeting that was held on 31 January 2017 in Brussels. She indicated that the consultation of the experts is now completed and the adoption process at the level of the European Commission has

now started. She thanked the experts from the CAT and the Inspectors Working Group for their support in the preparation of this important document.

7.1.7. GLP requirements for ATMPs

Scope: feedback from the European Commission

Action: for information

The European Commission representative indicated that the pragmatic approach developed by the CAT at the end of 2015 was presented and endorsed by the National Competent Authorities for clinical trials. The document will be published on the European Commission's website as part of the Questions and Answers that are prepared for the coming into force of the Clinical Trial Regulation. EMA was asked to publish this document also on the ATMP webpage on the EMA external website.

7.1.8. Similarity for ATMPs

Scope: feedback from the European Commission

Action: for information

The European Commission representative indicated that some comments were received during the public consultation and that the CAT group that was involved in the preparation of the ATMP similarity part should review the comments. A meeting via Adobe Connect will be organised.

7.1.9. CAT meeting dates 2019 - 2021

Scope: meeting dates for 2019 – 2021

Action: for adoption

The topic was postponed to the March 2017 CAT meeting.

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 January 2017 meeting

Action: for information

Documents:
-Summary of Outcomes

The information was noted.

7.2.2. Scientific Co-ordination Board (SciCoBo) – meeting of 31 January 2017

CAT: Paula Salmikangas

Action: for information

Paula Salmikangas provided a short feedback from the topic discussed at the last SciCoBo meeting.

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

7.3.1. ATMP guideline on safety and efficacy follow-up and risk management

Scope: discussion of the comments received.

Action: for discussion

EMA provided feedback from the presentation to PRAC and CHMP. CAT already provided a lot of comments, but additional feedback can be provided until 1 March 2017: then the drafting group will be reconvened to discuss and implement all comments.

7.4. Cooperation within the EU regulatory network

7.4.1. EDQM/Council of Europe – 3rd edition of the guide to the quality and safety of tissues and cells for human application

CAT: Paula Salmikangas

Scope: invitation to participate in the consultation of the 3rd edition of the guide to the quality and safety of tissues and cells for human application. Deadline: 20 February 2017.

Action: for discussion

Note: weblink to the consultation:

http://extranet.edqm.eu/dropboxout/0409nKR1KjbrCl40aFo8VdZtzEozYcHdpwkIdtCRPFu/TO%2016%2042-Draft%20TC%20Guide%203rd%20edition.zip

A short discuss took place. CAT members agreed to go through the document and provide written comments (high level) . EMA will inform EDQM of the short delay of submitting the CAT comments to the public consultation.

7.4.2. Evaluation of the EU blood and tissue and cells legislation

CAT: Paula Salmikangas

Scope: public consultation on the evaluation of the legislation for blood and tissues and cells. Deadline: 15 February 2017.

Action: for information

Note: weblink to the consultation: https://ec.europa.eu/health/blood_tissues_organs/policy/evaluation_en

CAT noted the information.

7.4.3. EU regulatory network

Scope: regulatory affairs awareness sessions for 2017

Action: for information

EMA presented the upcoming regulatory trainings with the EU-Network Training Centre (EU-NTC). One training on ATMPs is scheduled : CAT members were asked if they can contribute (give a presentation/training). EMA will investigate if members that are located outside of the national competent authorities can follow the training in EU-NTC.

7.5. Cooperation with international regulators

7.5.1. International Pharmaceutical Regulators Forum (IPRF) gene therapy discussion group

Feedback from recent international teleconference calls of the IPRF Gene therapy group

CAT: Paula Salmikangas

Action: for information

The topic was postponed to the March 2017 CAT meeting.

7.6. CAT work plan

7.6.1. Questions and Answers document on minimally manipulated ATMPs

CAT drafting group: Egbert Flory, Mikuláš Hrubiško, Marit Hystad, Metoda Lipnik-Stangelj, Margarida Menezes Ferreira, Tiina Palomäki, Paula Salmikangas

Scope: draft Questions & Answers

Action: for final discussion

Note: the Questions-and-Answers document describes the application of the risk-based approach for minimally manipulated ATMP (e.g. CD34+ cells for cardiac repair). In the answers, a practical explanation will be provided on how to use the risk based approach to identify and justify deviations from the standard requirements for cell-based ATMPs as included in Annex I Part IV of Directive 2001/83/EC.

EMA provide feedback: following comments received from CAT members, GMP inspectors and EMA, the drafting group reworked the Q&A. The lasted version of the Q&A was presented to the CAT for final comments by 1 March 2017. The document will be adopted at the March CAT meeting.

7.7. Planning and reporting

7.7.1. Issues identified by stakeholders: follow-up from EMA's ATMP workshop held in May 2016

Scope: published document

Action: for information

Note: EMA presented the report to the CAT at their December 2016 and January 2017 meetings.

The information was noted.

7.8. Others

7.8.1. Sterility testing requirement for cell-based ATMPs

European Directorate for the Quality of Medicines (EDQM)

Scope: further to discussions at the January 2017 CAT meeting, a follow-up discussion in the presence of representatives of the EDQM/European Pharmacopoeia on alternative sterility assays will take place

Action: for discussion

Further to a presentation of the EDQM colleagues, CAT discussed the alternative sterility testing methods as described in Ph.Eur general chapter 2.6.27 (Microbial examination of

cell-based preparation). It was clarified that only when referred in a monograph, these general chapters become binding requirements; there are at present no monograph for cell-based ATMPs. It was agreed that no cross validation of the methods describe in chapter 2.6.27 against the sterility assay in chapter 2.6.1 is expected. However, the applicant will have to investigate the suitability of the method (e.g. absence of interfering substances, sensitivity of the method).

8. Any other business

8.1.1. EMA's operation and relocation preparedness - Workstream 2

Scope: operational preparedness

Action: for information

A presentation was given on EMA's operational preparedness in the light of Brexit.

Date of next CAT meeting:
Wednesday 15 to Friday 17 March 2017

9. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Abbreviations / Acronyms

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

DNA: Deoxyribonucleic acid

DG: Drafting Group

EC: European Commission

EDQM: European Directorate for the Quality of Medicines

ERA: Environmental Risk Assessment

FDA: Food and Drug Administration

FL: Final Letter

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 Applicant

MAH: Marketing Authorisation Holder

MNAT: Multinational Assessment Team

MSC: Mesenchymal stem cells

PDCO: Paediatric Committee

PMDA: Pharmaceuticals and Medical Devices Agency (Japan)

PIP: Paediatric Investigation Plan

PL: Package leaflet

PRAC: Pharmacovigilance and Risk Assessment Committee #

PRIME: Priority Medicines

RMP: Risk Management Plan

RNA: Ribonucleic acid

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

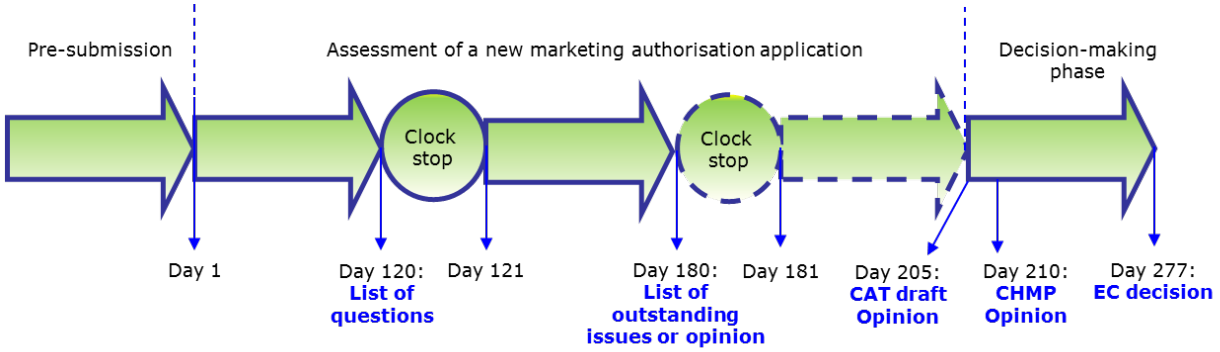
Evaluation of ATMPs (section 2)

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.

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 15 – 17 March 2017 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Ilona Reischl	Member	Austria	No interests declared	
Martin Brunner	Alternate	Austria	No restrictions applicable to this meeting	
Claire Beuneu	Member	Belgium	No interests declared	
Belaïd Sekkali	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Evelina Shumkova	Alternate	Bulgaria	No interests declared	
Mirna Golemovic	Member	Croatia	No interests declared	
Ivica Malnar	Alternate	Croatia	No interests declared	
Anna Paphitou	Member	Cyprus	No interests declared	
Ioannis Kkolos	Alternate	Cyprus	No interests declared	
Tomáš Boráň	Member	Czech Republic	No interests declared	
Ivana Haunerova	Alternate	Czech Republic	No interests declared	
Nanna Aaby Kruse	Member	Denmark	No restrictions applicable to this meeting	
Anne Pastoft	Alternate	Denmark	No interests declared	
Toivo Maimets	Member	Estonia	No interests declared	
Tarmo Tiido	Alternate	Estonia	No interests declared	
Paula Salmikangas	Member	Finland	No interests declared	
Olli Tenhunen	Alternate	Finland	No interests declared	
Violaine Closson	Member	France	No interests declared	
vacant	Alternate	France		
Martina Schüssler-Lenz	Member	Germany	No interests declared	
Egbert Flory	Alternate	Germany	No interests declared	
Asterios Tsiftsoglou	Member	Greece	No interests declared	
Angeliki Roboti	Alternate	Greece	No interests declared	
Krisztian Fodor	Member	Hungary	No interests declared	
Balázs Sarkadi	Alternate	Hungary	No interests declared	
vacant	Member	Iceland		

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
vacant	Alternate	Iceland		
Maura O'Donovan	Member	Ireland	No interests declared	
Niamh Curran	Alternate	Ireland	No interests declared	
Paolo Gasparini	Member	Italy	No interests declared	
Luca Sangiorgi	Alternate	Italy	No interests declared	
Una Riekstina	Member	Latvia	No interests declared	
Aija Linē	Alternate	Latvia	No interests declared	
Romaldas Mačiulaitis	Member (CHMP member)	Lithuania	No restrictions applicable to this meeting	
Jolanta Gulbinovic	Alternate (to CHMP representative)	Lithuania	No interests declared	
vacant	Member	Liechtenstein		
vacant	Alternate	Liechtenstein		
Jean-Louis Robert	Member (CHMP co-opted member)	Luxembourg	No interests declared	
Guy Berchem	Alternate (to CHMP representative)	Luxembourg	No restrictions applicable to this meeting	
John J. Borg	Member (CHMP member)	Malta	No interests declared	
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	
Johannes Hendrikus Ovelgönne	Member	Netherlands	No interests declared	
vacant	Alternate	Netherlands		
vacant	Member	Norway		
Rune Kjeken	Alternate	Norway	No restrictions applicable to this meeting	
Dariusz Śladowski	Member	Poland	No restrictions applicable to this meeting	
Anna Cieślik	Alternate	Poland	No interests declared	
Bruno Sepodes	Member (CHMP member)	Portugal	No interests declared	
Margarida Menezes-Ferreira	Alternate (to CHMP representative)	Portugal	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Gianina-Nicoleta Andrei	Alternate	Romania	No interests declared	
Simona Badoi	Member	Romania	No interests declared	
Mikuláš Hrubiško	Member	Slovakia	No restrictions applicable to this meeting	
Ján Kyselovič	Alternate	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	
Nevenka Trsinar Brodt	Alternate	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	
Lennart Åkerblom	Member	Sweden	No interests declared	
Björn Carlsson	Alternate	Sweden	No interests declared	
Christiane Niederlaender	Member	United Kingdom	No interests declared	
James McBlane	Alternate	United Kingdom	No interests declared	
Marc Turner	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
vacant	Alternate	Healthcare Professionals' Representative		
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	
vacant	Alternate	Healthcare Professionals' Representative		
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	
Michelino Lipucci di Paola	Alternate	Patients' Representative	No restrictions applicable to this meeting	
Mariëtte Driessens	Member	Patients' Representative	No restrictions applicable to this meeting	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Erik Briers	Alternate	Patients' Representative	No restrictions applicable to this meeting	
Karl-Heinz Buchheit	Observer	Conseil de l'Europe	No restrictions applicable to this meeting	
Chris Sotirelis	Expert - in person*	Healthcare Professionals' Representative	No interests declared	
Nathalie Morgensztejn	Expert - via telephone*	France	No interests declared	
Marion Perrin	Expert - via telephone*	France	No interests declared	
Tuomo Lapveteläinen	Expert - via telephone*	Finland	No interests declared	
Tiina Palomäki	Expert - via telephone*	Finland	No interests declared	
Heli Suila	Expert - via telephone*	Finland	No interests declared	
Mari Martikainen	Expert - via telephone*	Finland	No restrictions applicable to this meeting	
Juha Kolehmainen	Expert - via telephone*	Finland	No restrictions applicable to this meeting	
Paulina Lehtolainen-Dalkilic	Expert - via telephone*	Finland	No restrictions applicable to this meeting	
Tiina Reinivuori	Expert - via telephone*	Finland	No restrictions applicable to this meeting	
Andreea Barbu	Expert - via telephone*	Sweden	No restrictions applicable to this meeting	
Monique Wakelkamp	Expert - via telephone*	Sweden	No restrictions applicable to this meeting	
Åsa Sullivan	Expert - via telephone*	Sweden	No interests declared	
Fátima Ventura	Expert - via telephone*	Portugal	No restrictions applicable to this meeting	
Angelo Ferreira da Silva	Expert - via telephone*	Portugal	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Joost Romme	Expert - via telephone*	Netherlands	No interests declared	
Alain Fischer	Expert - via telephone*	Independent scientific expert		
Marina Cavazzana	Expert - via telephone*	Independent scientific expert		
Emmanuelle Charton	Expert - via telephone*	EDQM	No interests declared	
Sébastien Jouette	Expert - via telephone*	EDQM	No interests declared	
Isabelle Bekeredjian-Ding	Expert - via telephone*	Germany	No interests declared	
Jan-Oliver Karo	Expert - via telephone*	Germany	No interests declared	
Giovanni Migliaccio	Expert - via telephone*	AEATRIS	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.