20 March 2019
EMA/CAT/400411/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Advanced Therapies (CAT)

Minutes for the meeting on 20-22 February 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).

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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.

The CAT chair welcomed John Johnston, the new alternate member of the UK, and Ondrej Palan, the new alternate member of the Czech Republic, who joined the CAT for the first time.

1.2. Adoption of agenda

The CAT agenda for 20-22 February 2019 meeting was adopted.

1.3. Adoption of the minutes

The CAT minutes for 23-24 January 2019 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. Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750

Accelerated assessment

AveXis Netherlands B.V.; treatment of spinal muscular atrophy (SMA)

Scope: Day 120 list of questions

Action: for adoption

The Rapporteurs presented their assessment of the MAA.

CAT adopted the List of Questions.

CAT agreed to maintain the accelerated assessment: the response assessment timetable was adopted.

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 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. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/II/0029

Amgen Europe B.V.

Rapporteur: Olli Tenhunen, CHMP Coordinator: Outi Mäki-Ikola, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: safety: RSI
Update of section 5.2 of the SmPC in order to update the pharmacokinetic properties information based on the final results from study 20120324, a phase 2, multicentre, single-arm trial to evaluate the biodistribution and shedding of talimogene laherparepvec in subjects with unresected, stage IIIB to IVM1c melanoma. This submission fulfils MEA 006.1. In addition, the marketing authorisation holder (MAH) took the opportunity to update Annex II as per the already assessed EMEA/H/C/002771/ANX/001 procedure.

Action: for adoption

The Rapporteur presented the assessment of the type II variation. CAT adopted the RSI.

2.12. Other Post-Authorisation Activities

2.12.1. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/REC/004

Novartis Europharm Limited

Rapporteur: Rune Kjeken, CHMP Coordinator: Bjorg Bolstad

Scope: Quality

Action: for adoption

The review of this quality post-authorisation recommendation was presented. CAT agreed with the conclusion.

2.12.2. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/ANX/003

Novartis Europharm Limited

Rapporteur: Rune Kjeken, CHMP Coordinator: Bjorg Bolstad

Scope: Clinical: the Statistical Analysis Plan from CTL019B2401 has been submitted to address the post-authorisation measures for Kymriah (INN: tisagenlecleucel) described below (also in Annex II of the Product Information and in RMP): In order to further evaluate the efficacy of Kymriah in patients with relapsed/refractory diffuse large B-cell lymphoma (DLBCL), the applicant will conduct and submit a prospective, observational study in patients with r/r DLBCL based on data from registry with efficacy outcome measures in line with study C2201, including details of the manufacturing turnaround time (i.e., time from last relapse or confirmed refractory status, time from decision to treat, and time from leukapheresis to infusion). Pre-specified subgroup analysis will be conducted to evaluate the effectiveness of Kymriah.

Action: for adoption

The review of this clinical post-authorisation measure, addressing the Annex II condition, was presented. The protocol submitted by Novartis is for the post authorisation safety and efficacy study. CAT adopted an RSI.

2.12.3. Kymriah - tisagenlecleucel - Orphan

Novartis Europharm Limited

Rapporteur: Rune Kjeken; CHMP Coordinator: Bjorg Bolstad

Scope: Quality

Action: for 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

No items

3.2. Day 60 Evaluation Reports

3.3. New Applications

No items

4. Scientific Recommendation on Classification of ATMPs

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Recombinant adeno-associated viral vector serotype 8 (AAV8) encoding a codon optimised cDNA encoding human Retinitis Pigmentosa GTPase Regulator (coRPGR) – H0005315

Intended for the treatment of X-linked retinitis pigmentosa (XLRP)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

Nominations were received. The following CAT member was appointed as CAT-coordinator.

4.1.2. Heterogeneous mixture of human milk-derived nutritional and viable non-nutritional ingredients

Human milk substitute/supplementation of premature infants and children of mothers with milk shortage

Scope: letter to the applicant

Action: for discussion

CAT discussed the letter to the applicant. The letter was agreed.

4.1.3. Allogeneic adult bone-marrow-derived stem cells transiently transfected with a plasmid construct encoding the intracellular domain of human Notch-1 – H0005313

Intended for the treatment of motor deficits arising from acquired brain injury, including traumatic brain injury, ischaemic stroke and haemorrhagic stroke

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

Nominations were received. The following CAT member was appointed as CAT-coordinator.

4.1.4. Autologous T cells transduced with a T cell receptor (TCR) targeting human Telomerase Reverse Transcriptase (hTERT) – H0005314

Intended for the treatment of various cancer types expressing hTERT

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

Nominations were received. The following CAT member was appointed as CAT-coordinator.

4.2. Day 30 ATMP scientific recommendation

4.2.1. Recombinant adeno-associated viral vector serotype-5 expressing human 21-hydroxylase gene – H0005295

Treatment of congenital adrenal hyperplasia

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 18 March 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.

4.2.2. Autologous skeletal muscle derived cells attached to biodegradable poly(DL-lactide-co-glycolide) microparticles combined with skeletal muscle derived cells – H0005289

Treatment of faecal incontinence and anorectal malformation

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 18 March 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.

4.2.3. Allogeneic, *ex vivo* expanded, umbilical cord (UC) blood-derived, haematopoietic CD34+ progenitor cells and allogeneic, non-expanded, UC blood-derived, haematopoietic mature myeloid and lymphoid cells - H0005288

Haematopoietic reconstitution of patients who are medically indicated for allogeneic haematopoietic stem cell transplantation

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. This product is composed of two components: an expanded and a non-expanded cell fraction.

CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 18 March 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.

4.2.4. *In vitro* transcribed single-stranded messenger RNA (mRNA) molecules encoding human interferon-α2b, interleukin-12, interleukin-15sushi, and Granulocyte-macrophage colony-stimulating factor – H0005291

Treatment of solid tumours

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 18 March 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.

4.2.5. Allogeneic cord blood mononuclear cells - H0005292

Treatment of neurological disorders, autism spectrum disorders, cerebral palsy

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report.

CAT decided to request following additional information from the applicant.

4.2.6. Recombinant adeno-associated viral vector (AAV) containing a human micro-dystrophin gene – H0005293

Treatment of patients with Duchene muscular dystrophy (DMD)

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 18 March 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.

4.2.7. Plasmid vector expressing interleukin-12 gene – H0005294

Treatment of advanced melanoma

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 18 March 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.

4.2.8. *Ex vivo* expanded allogeneic bone marrow derived mesenchymal stromal cells – H0005290

Treatment of graft-*versus*-host 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 18 March 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. Whole lipoaspirate containing viable autologous adipose-derived regenerative cells - H0005212

Treatment of progressive hemifacial atrophy (Parry-Romberg syndrome)

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

Action: for information

The information was noted.

4.4.2. Viable autologous adipose-derived regenerative cells combined with whole lipoaspirate - H0005213

Treatment of progressive hemifacial atrophy (Parry-Romberg syndrome)

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

Action: for adoption

The revised ATMP classification report was adopted.

4.4.3. Viable autologous adipose-derived regenerative cells combined with whole lipoaspirate - H0005214

Treatment of progressive hemifacial atrophy (Parry-Romberg syndrome)

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

Action: for adoption

The revised ATMP classification report was adopted.

4.4.4. Whole lipoaspirate containing viable autologous adipose-derived regenerative cells - H0005215

Treatment of burn scars

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

Action: for information

The information was noted.

4.4.5. Viable autologous adipose-derived regenerative cells - H0005216

Treatment of burn scars

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

Action: for adoption

The revised ATMP classification report was adopted.

4.4.6. Viable autologous adipose-derived regenerative cells - H0005217

Treatment of burn scars

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

Action: for adoption

The revised ATMP classification report was adopted.

4.4.7. Recombinant adeno-associated virus (serotype 5) containing the human retinal guanylate cyclase 1 (GUCY2D) gene – H0005261

Treatment of inherited retinal disease caused by biallelic mutations in GUCY2D, including Leber congenital amaurosis type 1 (GUCY2D-LCA)

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

Action: for information

The information was noted.

4.4.8. Autologous cord blood nucleated cells – H0005260

Treatment of paediatric brain damage, hypoxic-ischemic encephalopathy, cerebral palsy

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

Action: for information

The information was noted.

4.4.9. Recombinant adeno-associated virus (serotype 0) containing the human α-L-iduronidase (hIDUA) gene - H0005258

Treatment of mucopolysaccharidosis type I

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

Action: for information

The information was noted.

4.4.10. Cultured autologous adipose-derived stem cells - H0005257

Treatment of urinary diversion in patients requiring radical cystectomy for the treatment of bladder cancer

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

Action: for information

The information was noted.

4.4.11. Recombinant adeno-associated virus serotype rh10 (AAVrh10) containing a transgene that encodes a micro ribonucleic acid (miRNA) targeting superoxide dismutase 1 (SOD1) messenger RNA (mRNA) - H0005259

Treatment of amyotrophic lateral sclerosis (ALS) due to mutations in SOD1 gene

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

Action: for information

The information was noted.

4.4.12. Autologous dendritic cell, electroporated with messenger ribonucleic acid (mRNA) encoding tumour antigen Wilms tumour r (WT)-1 – H0005240

Postponed

Treatment of lung cancer

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

Action: for information

The postponement was noted.

4.5. Follow-up and guidance

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

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

6.3.5. Follow-up

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

UK: John Johnston – new alternate. New membership mandate from 30 January 2019

UK: James McBlane – membership ended on 29 January 2019

Croatia: Nenad Medic – membership ended on 06 February 2019

Action: for information

The CAT chair welcomed the new UK alternate, John Johnston and expressed her thanks to James McBlane for his contributions to the CAT during the last years.

7.1.2. Strategic Review & Learning meeting – joint CAT/Clinical trial facilitation group (CTFG), Bucharest, Romania, 13-14 June 2019

CAT resources: Simona Badoi

Scope: initial topics for the agenda

Action: for discussion

The upcoming Strategic Review and Learning meeting (SRLM), which will be organised on the auspices of Romania, who holds the presidency of the EU in the first half of 2019, was announced. A half day of this SRLM will be held jointly with the CTFG.

A teleconference will be organised to start the development of the agenda of the SRLM. An initial agenda will be presented at the March 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) of the January 2019 meeting

Action: for information

The information was noted.

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

None

7.4. Cooperation within the EU regulatory network

7.4.1. EDQM/Council of Europe – 4th edition of the guide to the quality and safety of tissues and cells for human application

CAT: Martina Schüssler-Lenz

Scope: invitation to participate in the consultation of the 4th edition of the guide to the quality and safety of tissues and cells for human application. Deadline: 8 March 2019.

Action: for discussion

Note: weblink to the consultation:
http://extranet.edqm.eu/dropboxout/045FeTCbK9YidCaHofWneRjQuVeHi7oxxUtyyN0mqeK/4th%20ed%20of%20TC%20Guide%20for%20open%20consultation-TO%2019%201_.zip

CAT members will comment on the document. . EMA will compile the comments and forward them to EDQM.

7.4.2. New EU initiative to optimize the interplay between the pharma and the GMO framework

Scope: update of discussions with GMO authorities

Action: for information

A short update was provided by the Commission representative.

7.5. Cooperation with international regulators

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

The teleconference will take place

CAT: Martina Schüßler-Lenz

Scope: draft agenda

Action: for discussion

7.6. CAT work plan

None

7.7. Planning and reporting

None

7.8. Others

7.8.1. EMA implementation of the new medical device and *in vitro* diagnostic regulation

CAT: Ilona Reischl

Scope: follow-up from presentation at the July 2018 CAT meeting and updates on the various work streams.

Action: for discussion

Feedback on the progress on the implementation of the new medical device and *in vitro* diagnostics regulation was provided. There was a short feedback on the development of the Guideline on drug device combinations. This GL will be presented at the CAT in March for discussion.

7.8.2. Request from the European Commission for EMA's opinion on the definitions of pharmacological, immunological, metabolic and medical diagnosis

CAT Sponsor: Margarida Menezes Ferreira: pharmacologicalCAT Sponsors: Jan Mueller-Berghaus, Ilona Reischl: immunologicalCAT Sponsor: Claire Beuneu: metabolicCAT Sponsors: Paolo Gasparini, Giulio Pompilio: medical diagnosis

Scope: definitions of pharmacological, immunological, metabolic and medical diagnosis

Action: for adoption

The EMA coordinators and CAT sponsors presented the draft definitions of pharmacological, immunological, metabolic and medical diagnosis. the 4 definitions were agreed by CAT.

8. Any other business

8.1. 22nd annual meeting of the American Society of Gene and Cell Therapy (ASGCT), Washington, DC, USA, 28 April – 02 May 2019

Scope: Workshop on 'Pre-approval commercialization' to take place on Sunday 28th April 2019.

Action: for agreement of participation of the CAT Chair

CAT agreed with the participation of the CAT chair as representative of EMA/CAT at the ASGCT conference.

Date of next CAT meeting:

20-22/03/2019

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

EDQM: European Directory for the Quality of Medicines

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

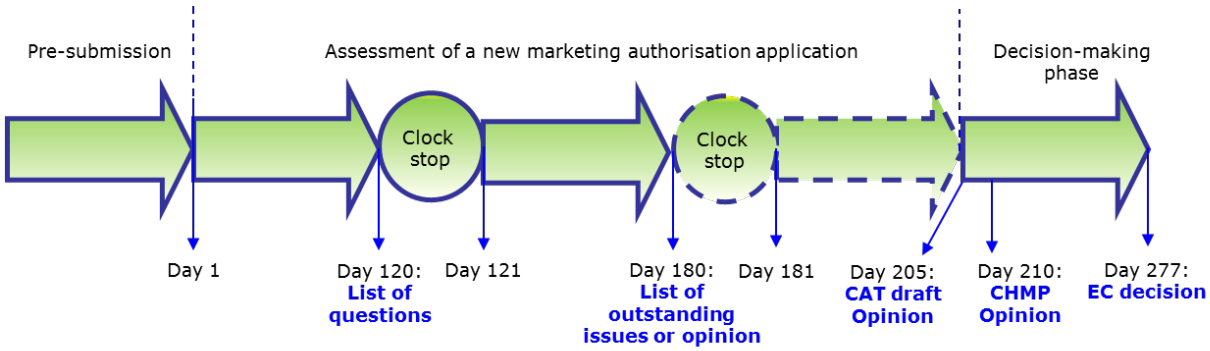
Evaluation of ATMPs (section 2)

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 20-22 February 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	N/A
Ilona Reischl	Member	Austria	No interests declared	N/A
Claire Beuneu	Member	Belgium	No interests declared	N/A
Belaïd Sekkali	Alternate	Belgium	No interests declared	N/A
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	N/A
Mirna Golemovic	Member	Croatia	No interests declared	N/A
Marina Ieridi	Member	Cyprus	No interests declared	N/A
Ondrej Palan	Alternate	Czech Republic	No interests declared	N/A
Anne Pastoft	Member	Denmark	No interests declared	N/A
Nanna Aaby Kruse	Alternate	Denmark	No interests declared	N/A
Toivo Maimets	Member	Estonia	No interests declared	N/A
Pille Saalik	Alternate	Estonia	No interests declared	N/A
Heli Suila	Member	Finland	No interests declared	N/A
Violaine Closson	Member	France	No interests declared	N/A
Jan Mueller-Berghaus	Member	Germany	No interests declared	N/A
Egbert Flory	Alternate	Germany	No interests declared	N/A
Angeliki Roboti	Alternate	Greece	No interests declared	N/A
Katalin Lengyel	Member	Hungary	No interests declared	N/A
Maura O'Donovan	Member	Ireland	No interests declared	N/A
Paolo Gasparini	Member	Italy	No interests declared	N/A
Giulio Pompilio	Alternate	Italy	No interests declared	N/A
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	N/A
Johannes Hendrikus Ovelgönne	Member	Netherlands	No interests declared	N/A
Carla Herberts	Alternate	Netherlands	No interests declared	N/A
Rune Kjeken	Member	Norway	No restrictions applicable to this meeting	N/A
Maja Sommerfelt Grønvold	Alternate	Norway	No interests declared	N/A

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dariusz Śladowski	Member	Poland	No restrictions applicable to this meeting	N/A
Margarida Menezes-Ferreira	Alternate (to CHMP representative)	Portugal	No interests declared	N/A
Simona Badoi	Member	Romania	No interests declared	N/A
Lukas Slovak	Member	Slovakia	No interests declared	N/A
Marcos Timón	Alternate, to CHMP representative	Spain	No interests declared	N/A
Lisbeth Barkholt	Member	Sweden	No interests declared	N/A
Björn Carlsson	Alternate	Sweden	No interests declared	N/A
John Johnston	Alternate	United Kingdom	No interests declared	N/A
Marc Turner	Member	Healthcare Professionals' Representative	No interests declared	N/A
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	N/A
Willem Fibbe	Alternate	Healthcare Professionals' Representative	No interests declared	N/A
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	N/A
Michelino Lipucci di Paola	Alternate	Patients' Representative	No restrictions applicable to this meeting	N/A
Maria Driessens	Member	Patients' Representative	No restrictions applicable to this meeting	N/A
Erik Briers	Alternate	Patients' Representative	No restrictions applicable to this meeting	N/A
Beate Mosl	Expert – In person*	PEI	Direct interests declared	N/A
Juliane Rau	Expert – In person*	PEI	No interests declared	N/A
Barbara Bonamassa	Expert – In person*	AIFA	No interests declared	N/A
Christos Sotirelis	Expert – In person*	Patients' Representative	No interests declared	N/A
Nathalie Morgensztejn	Expert – In person*	ANSM	No interests declared	N/A
Lieke Sandberg Smits	Expert – In person*	CBG/MEB	No interests declared	N/A
Johannes Petrus Theodorus Span	Expert – In person*	University Medical Centre ST Radboud	No interests declared	N/A
Emmely de Vries	Expert – In person*	CBG/MEB	No interests declared	N/A
Kristine Moltu	Expert – In	NOMA	Direct interests	N/A

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
	person*		declared	
Helga Haugom Olsen	Expert – Via telephone*	NOMA	No interests declared	N/A
Janneke van Leeuwen	Expert – Via telephone*	CBG/MEB	No interests declared	N/A
Nathalie Morgensztejn	Expert – Via telephone*	ANSM	No interests declared	N/A
Marcel H.N. Hoefnagel	Expert – Via telephone*	CBG/MEB	No interests declared	N/A
Berendina Maria van den Hoorn	Expert – Via telephone*	CBG/MEB	No interests declared	N/A
Charlotte de Wolf	Expert – Via telephone*	CBG/MEB	No interests declared	N/A
Johann Lodewijk Hillege	Expert – Via telephone*	MPA	No interests declared	N/A
Ulla Wändel Liminga	Expert – Via telephone*	MPA	No interests declared	N/A
Pirkko Puranen	Expert – Via telephone*	FIMEA	No interests declared	N/A
Pirjo Hanninen	Expert – Via telephone*	FIMEA	Direct interests declared	N/A
Mirka Laavola	Expert – Via telephone*	FIMEA	Direct interests declared	N/A
Anke Zobywalski	Expert – Via telephone*	PEI	No interests declared	N/A
Matthias Renner	Expert – Via telephone*	PEI	Direct interests declared	N/A
Ria Nibbeling	Expert – Via telephone*	CBG/MEB	No interests declared	N/A
Dora Hermann	Expert – Via telephone*	OGYEI	No interests declared	N/A
Biljana Temova	Expert – Via telephone*	JAZMP	Direct interests declared	N/A
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.