19 February 2020
EMA/CAT/127419/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 22-24 January 2020

Election of CAT chairperson

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

Scope: election of CAT Chair.

Action: election of CAT Chair

The Mandate of the present CAT Chair (Martina Schüßler-Lenz) will finish on 14 February 2020.

The election of the chairperson took place on 23 January 2020 at 14:00hrs. EMA reminded the CAT members of the Rule of Procedure pertaining to the election of the chairperson.

For the duration of the election, Martina Schüßler-Lenz was nominated as the CAT member for Germany.

The candidates addressed the CAT and answered to questions from the CAT members.

The election took place in the presence of 29 CAT members that were eligible to vote. Martina Schüßler-Lenz was elected as CAT Chair for a second mandate of 3 years.

Martina Schüßler-Lenz thereafter took up her present mandate as CAT Chair and chaired the January 2020 CAT meeting

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
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Committee for Advanced Therapies (CAT)

Minutes of the meeting on 22-24 January 2020

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).

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). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The CAT chair welcomed the new members from Portugal (Maria Isabel Vieira) and from Romania (Alina Muscetescu) and thanked the previous members, Margarida Menezes Ferreira and Simona Badoi for their active contributions to the working of the CAT.

1.2. Adoption of agenda

The CAT agenda for 22-24 January 2020 meeting was adopted with one addition (2.6.1)

1.3. Adoption of the minutes

CAT minutes for 04-06 December 2019 meeting

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

No items

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

2.6.1. Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750

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

Scope: feedback from discussion

Action: for information

The Rapporteurs provided feedback from the discussion .

2.7. New applications

2.7.1. Ovaloctocogene roxaparvovec - Orphan - EMEA/H/C/004749

Accelerated assessment

BioMarin International Limited; treatment of haemophilia A

Scope: timetable for assessment

Action: for adoption

The assessment timetable was adopted.

2.8. Withdrawal of initial marketing authorisation application

No items

2.9. Re-examination of initial application procedures under Article 9(2) of Regulation No. 726/2004

No items

2.10. GMP and GCP inspections requests

No items

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

2.11.1. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0014

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: quality. Opinion

Action: for adoption

Request for Supplementary Information adopted on 08.11.2019.

The opinion was adopted.

2.11.2. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0017/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: quality:

Request for Supplementary Information

Action: for adoption

The request for supplementary information was adopted.

2.12. Other Post-Authorisation Activities

2.12.1. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/ANX/002

Amgen Europe B.V.

Rapporteur: Olli Tenhunen, CHMP Coordinator: Tuomo Lapveteläinen

Scope: clinical. Opinion

From initial MAA:
To submit the preliminary results from Study 20110266 (a phase 2, multicenter, randomized, open-label trial assessing the efficacy and safety of talimogene laherparepvec neoadjuvant treatment plus surgery vs surgery alone for resectable stage IIIB to IVM1a melanoma).

Action: for adoption

The Rapporteur provided feedback on this post-authorisation study. It was agreed that the Annex II condition is fulfilled. The assessment report was adopted.

2.12.2. Zynteglo – autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/R/0005

Bluebird bio (Netherlands) B.V.

Rapporteur: Carla Herberts, CHMP Coordinator: Paula Boudewina van Hennik

Scope: 1-year renewal of conditional marketing authorisation. Request for Supplementary Information

Action: for adoption

The Rapporteur presented the renewal assessment report. This is the first renewal of the conditional marketing authorisation. An update on the fulfilment of the conditions was provided. A request for supplementary information was adopted.

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

No items

3.3. New Applications

No items

3.4. Ongoing applications

4. Scientific Recommendation on Classification of ATMPs

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Autologous, *ex vivo* expanded, clonal neoantigen specific tumour infiltrating lymphocytes – H0005575

Intended for the treatment of solid tumours

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.2. Autologous adipose derived mesenchymal stem cells, ALS – H0005580

Intended for the treatment of Amyotrophic Lateral Sclerosis (ALS)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.3. Wharton jelly derived mesenchymal stem cells

Intended for the treatment of spinal cord injury, drug resistant epilepsy and hypoxia ischemia encephalopathy

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.4. Allogeneic CRISPR/Cas9-mediated genetically modified CAR T cells targeting CD19 antigen – H0005581

Intended for the treatment of CD19+ haematological malignancies

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.5. Allogeneic CRISPR/Cas9-mediated genetically modified CAR T cells targeting B-cell maturation antigen (BCMA) – H0005582

Intended for the treatment of relapsed or refractory multiple myeloma

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.6. Micronized autologous adipose tissue particles and costal cartilage powder

Intended for the treatment of cartilage defects

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.7. Human embryonic stem cell-derived otic neural progenitor cells – H0005583

Intended for the treatment of sensorineural hearing loss

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 cells, ALS

Intended for the treatment of Amyotrophic Lateral Sclerosis (ALS)

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, Huntington's disease - H0005571

Intended for the treatment of Huntington's disease

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, Lewy body dementia (LBD) - H0005572

Intended for the treatment of Lewy body dementia (LBD)Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

The CAT coordinator was appointed.

4.1.11. Wharton's jelly derived mesenchymal stem cell, secondary progressive multiple sclerosis (SPMS) - H0005573

Intended for the treatment of secondary progressive multiple sclerosis (SPMS)

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. Adenovirus associated viral vector serotype 5 containing the human RPGR gene – H0005544

Intended for the treatment of X-linked retinitis pigmentosa owing to defects in the RPGR

Scope: ATMP scientific recommendation

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 7 February 2020.

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

4.2.2. Recombinant adeno-associated viral vector serotype 9 encoding a codon-optimised human aspartylglucosaminidase (AGA) transgene – H0005560

Intended for the treatment of aspartylglucosaminuria

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 7 February 2020.

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

4.2.3. Adipose derived mesenchymal stem cell , Alopecia - H0005567

Intended for the treatment of alopecia

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. Additional information and clarification are needed before concluding on this classification. The list of issues was adopted by CAT and the procedure is stopped awaiting responses from the applicant.

4.2.4. Adipose derived mesenchymal stem cell , Hypertrophic scars - H0005568

Intended for the treatment of hypertrophic scars

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. Additional information and clarification are needed before concluding on this classification. The list of issues was adopted by CAT and the procedure is stopped awaiting responses from the applicant.

4.2.5. Wharton's jelly derived mesenchymal stem cell , AMD – H0005562

Intended for the treatment of age-related macular degeneration (AMD)

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 7 February 2020.

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

4.2.6. Wharton's jelly derived mesenchymal stem cell , bone non-union - H0005563

Intended for the treatment of bone non-union

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 7 February 2020.

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

4.2.7. Wharton's jelly derived mesenchymal stem cell , Chorioretinal disorders - H0005564

Intended for the treatment of Behçet's disease, Choroideremia, Vitelliform macular dystrophy (Best disease), Cone rod dystrophies

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 7 February 2020.

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

4.2.8. Wharton's jelly derived mesenchymal stem cell , Epidermolysis bullosa - H0005565

Intended for the treatment of Epidermolysis bullosa

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 7 February 2020.

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

4.2.9. Wharton's jelly derived mesenchymal stem cell , hypoxic-ischemic encephalopathy (HIE) - H0005566

Intended for the treatment of hypoxic-ischemic encephalopathy

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 7 February 2020.

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

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

No items

4.4. Finalisation of procedure

4.4.1. Autologous chondrocytes in suspension - H0005498

Intended for the treatment of knee joint cartilage lesion

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

Action: for information

The final recommendation was noted.

4.4.2. Autologous chondrocytes on a fibrinogen carrier – H0005525

Intended for the treatment of knee joint cartilage lesion

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

recommendation

Action: for information

The final recommendation was noted.

4.4.3. Modulated immune cells – H0005515

Intended for prophylactic use in solid organ transplantation (e.g. kidney transplantation) and therapeutic use in autoimmune disease (e.g. multiple sclerosis)

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

Action: for information

The final recommendation was noted.

4.4.4. Wharton's jelly derived mesenchymal stem cell , Adrenoleukodystrophy – H0005526

Intended for the treatment of adrenoleukodystrophy

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

Action: for information

The information was noted.

4.4.5. Wharton's jelly derived mesenchymal stem cell , Encephalopathy – H0005527

Intended for the treatment of encephalopathy

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

Action: for information

The information was noted.

4.4.6. Wharton's jelly derived mesenchymal stem cell , Epilepsy – H0005528

Intended for the treatment of epilepsy

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

Action: for information

The information was noted.

4.4.7. Wharton's jelly derived mesenchymal stem cell , Osteoarthritis – H0005529

Intended for the treatment of osteoarthritis

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

Action: for information

The information was noted.

4.4.8. Wharton's jelly derived mesenchymal stem cell , Polyneuropathy – H0005530

Intended for the treatment of polyneuropathy

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

Action: for information

The information was noted.

4.4.9. Wharton's jelly derived mesenchymal stem cell , Spinal muscular atrophy – H0005531

Intended for the treatment of spinal muscular atrophy

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

Action: for information

The information was noted.

4.4.10. Wharton's jelly derived mesenchymal stem cell , Spinocerebellar ataxia – H0005532

Intended for the treatment of spinocerebellar ataxia

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

Action: for information

The information was noted.

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

6.4. Pre-submission Issues

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT Chairperson - election

Scope: election of Chairperson .

Action: for election

Martina-Schüßler-Lenz was re-elected as CAT chair for a period of 3 years.

7.1.2. CAT membership

Romania: Alina Musetescu – new member. Membership mandate started on 17 December 2019
Romania: Simona Badoi – membership ended on 16 December 2019

Portugal: Maria-Isabel Borba Vieira – new alternate. Membership mandate started on 10 January 2020
Portugal: Margarida Menezes-Ferreira – membership ended on 31 December 2019

Action: for information

The CAT chair welcomed the new members.

7.1.3. Seating plan for CAT members under the Croatian EU Presidency – 01 January to 30 June 2020

Scope: CAT seating plan 01 January to 30 June 2020

Action: for information

The information was noted.

7.1.4. Revision of templates for accelerated assessment requests

CAT/CHMP: Jan Mueller-Berghaus, CHMP: Johann Lodewijk Hillege

Scope: revision of templates for accelerated assessment requests

Action: for discussion

Note: following up from discussions at CHMP in October 2019, a revised draft update to the template for the briefing note on accelerated assessment requests has been prepared, addressing comments from committee sponsors.

The revised template was presented to CAT and the changes were highlighted.

There was discussion on the sentence in the document "CHMP proposal to consider non-approved medicinal products as "existing therapy" only exceptionally, when they are widely used and recognised, AND recommended in European level treatment guidelines". The difficulties of asking prospective applicants to compare their products with products used e.g. under hospital exemption were " highlighted.

Following CAT members will review the document: Jan Mueller-Berghaus, Carla Herberts and Alina Musetescu. The Rapporteurs of ATMPs that underwent an accelerated assessment were asked to comment on the document. Comments are awaited by 7 February 2020.

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 December 2019 meeting

Action: for information

The information was noted.

7.2.2. CAT-COMP working group

Scope: kick-off meeting to take place on 19th February 2020, 18:30hrs-19:30hrs. Call of interest to nominate five members from each committee (in addition to the Chair and Vice-Chairs) as participants/representatives

Action: for discussion

Following CAT members will join the kick-off meeting: Maja Sommerfelt, Maura O'Donovan, Kieran Breen, Carla Herbert and Martina Schüßler-Lenz.

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

7.4. Cooperation within the EU regulatory network

7.4.1. Genetically modified organisms (GMO)

Scope: new procedure for the consultation of GMO authorities on the environmental risk assessment (ERA) for GMOs in MAAs; guidance to applicants on data to be provided in the MAA.

Action: for information

EMA and the European Commission representative provided feedback on the new procedure for the consultation of GMO authorities. A teleconference with GMO authorities has taken place to explain the new procedure and their role (i.e. to support the Rapporteurs to assess possible risk minimisation measures related to environmental risk posed by the product). It was noted that this new process is applicable to all GMO containing medicines, e.g. GTMPs, recombinant vaccines. There was a short discussion on the role of the Rapporteurs to review the questions coming from the GMO authorities for inclusion (or not) in the list of questions.

It was agreed that more attention should be given to the inclusion of environmental risk minimisation measures in the product information, if needed. This include statements on waste disposal.

Also, the guidance to applicants on the ERA-related information to be included in the MAA has been updated.

7.4.2. Concerns over the use of unregulated ATMPs

CAT: Martina Schüßler-Lenz

Scope: CAT concerns over the use of unregulated / unproven ATMPs. Consideration on the need for revision on the 2010 CAT public statement.

Action: for discussion

Note: In 2010, CAT issued a public statement on concerns over unregulated medicinal products containing stem cells (https://www.ema.europa.eu/en/news/public-statement-concerns-over-unregulated-medicinal-products-containing-stem-cells). FDA and Health Canada issued similar statements in 2019. Various publications and press articles indicate that such practices are also taking place in Europe (see, e.g. Polish article *'Polityka' investigation: deceptive stem cell therapies*: https://www.polityka.pl/tygodnikpolityka/nauka/1935142,1,sledztwo-polityki-zludne-terapie-komorkami-macierzystymi.read).
CAT discussed the high number of ATMP classification request for MSC-based product for a whole range of disease for which there is not clear mechanism of action, and hence, for which the claimed clinical indication is for the moment unproven. These ATMP classifications are used as the basis of hospital exemption in some member states.

CAT subsequently discussed if the existing EMA statement on concerns over unregulated stem cell-based medicines should be revised. CAT considered that this statement is still valid but could be updated to all ATMPs (not only stem-cell based ATMPs). It was agreed that following CAT members will work on an update of the statement. As a next step, a publication or communication in a scientific journal could be developed.

7.5. Cooperation with international regulators

7.5.1. ICH S12 - guideline on biodistribution of gene therapy medicinal products

CAT: Claire Beuneu, Rune Kjeken

Scope: preparation of the ICH S12 concept paper: feedback from the drafting groups at the ICH meeting in Singapore (November 2019)

Action: for information

Topic postponed until the February CAT meeting.

7.6. CAT work plan

7.6.1. CAT work plan 2020

CAT: Martina Schüßler-Lenz

Scope: draft work plan 2020

Action: for adoption

Alina Musetescu will replace Simona Bodoi (for the finalisation of the guideline on requirement for ATMPs in clinical trials). Maura O'Donovan was appointed as CAT lead for the work plan topic on interactions with COMP and PDCO. With these changes, the CAT workplan was adopted.

The CAT workplan will be published on the EMA website shortly.

7.7. Planning and reporting

None

7.8. Others

7.8.1. EU NTC proposal for training on pharmacoepidemiology

Action: For information

Topic postponed until the February CAT meeting.

8. Any other business

No items

Date of next CAT meeting:

19-21/02/2020

9. Explanatory notes

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

Abbreviations / Acronyms

AAV: Adeno-Associated Virus

AR: Assessment Report

ATMP: Advanced Therapy Medicinal Product

BWP: Biologics Working Party

CAT: Committee for Advanced Therapies

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

CTFG: Clinical Trial Facilitation Group

DG: Drafting Group

EC: European Commission

ERA: Environmental Risk Assessment

FDA: Food and Drug Administration

FL: Final Letter

GCG: Guideline Consistency Group

GCP: Good Clinical Practice

GLP: Good Laboratory Practice

GMO: Genetically-modified organism

GMP: Good Manufacturing Practice

GTMP: Gene Therapy Medicinal Product

HTA: Health Technology Assessment Bodies

HSPC: Hematopoietic Stem and Progenitor Cells

ITF: Innovative Task Force

JR: Joint Report

LoOI: List of outstanding issues

LoQ: List of questions

MA: Marketing Authorisation

MAA: Marketing Authorisation Application

MAH: Marketing Authorisation Holder

MSC: Mesenchymal stem cells

PDCO: Paediatric Committee

PMDA: Pharmaceuticals and Medical Devices Agency (Japan)

PIP: Paediatric Investigation Plan

PL: Package leaflet

PRAC: Pharmacovigilance and Risk Assessment Committee #

PRIME: Priority Medicines

RMP: Risk Management Plan

RP: Reflection paper

RSI: Request for supplementary information

SAs: Scientific Advices

SAG-O: Scientific Advisory Group Oncology

SAWP: Scientific Advice Working Party

SR: Summary Report

SWP: Scientific Working Party

SME: Small and medium size enterprises

SmPC: Summary of Products Characteristics

TT: Timetable

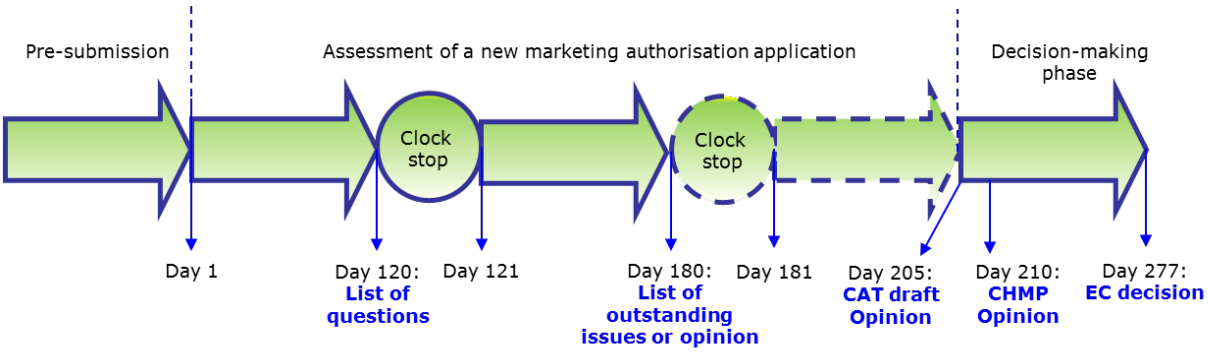
Evaluation of ATMPs (section 2)

This section lists applications for marketing authorisations of new Advanced Therapy Medicinal Products (ATMPs) that are to be discussed by the Committee. It also lists any ATMP related inspection requests (section 2.9) and Post-authorisation activities (section 2.10).

New applications (sections 2.1. to 2.12.)

Section 2.1 is for ATMPs nearing the end of the evaluation and for which the CAT is expected to adopt a draft **opinion** at this meeting on whether marketing authorisation should be granted. Once adopted, the CAT opinion is transmitted to the CHMP for final adoption. The CHMP opinion will be forwarded to the European Commission for a final legally binding decision valid throughout the EU. More information on the evaluation of ATMPs can be found here.

The other items in the section are listed depending on the stage of the evaluation, which is shown graphically below:



The assessment of an application for a new medicine takes up to 210 'active' days. This active evaluation time is interrupted by at least one 'clock-stop' during which time the applicant prepares the answers to questions from the CAT. The clock stop happens after day 120 and may also happen after day 180, when the CAT has adopted respectively a **Day 120 list of questions** (section 2.3) or a List of outstanding issues to be addressed by the company, which is listed in the agenda under sections 2.7 (**Ongoing evaluation procedures**). Section 2.7 also includes the CAT discussions at any other timepoint of the evaluation procedure of new applications.

Oral explanation (section 2.2.)

Prior to adoption of the CAT opinion, marketing authorisation applicants are normally invited to the CAT plenary meeting to address questions raised by the Committee.

Oral explanations normally relate to ongoing applications, but they can also relate to any other issue for which the CAT would like to discuss with company representatives in person.

Re-examination procedures (new applications) under article 9(2) of regulation no 726/2004 (section

2.6.)

This section lists applications for new marketing authorisation for ATMPs for which the applicant has requested a re-examination of the opinion previously issued by the CHMP. Similar to the initial evaluation of a marketing authorisation of an ATMP, CAT will adopt a draft re-examination opinion, which is transmitted to the CHMP for final adoption.

Withdrawal of applications (section 2.7.)

This section includes information on marketing authorisation applications that are withdrawn by the applicant. Applicants may decide to withdraw applications at any stage during the assessment and a CAT opinion will therefore not be issued. Withdrawals are included in the agenda for information or discussion, as necessary.

New applications (section 2.9.)

In this section, information is included on upcoming marketing authorisation applications for ATMPs, as well as information on appointment of Rapporteurs for new ATMP applications.

GMP and GCP Inspections Issues (section 2.10.)

This section lists inspections that are undertaken for ATMPs. Inspections are carried out by regulatory agencies to ensure that marketing authorisation holders comply with their obligations. Inspection can relate to good manufacturing practice (GMP), good clinical practice (GCP), good laboratory practice (GLP) or good pharmacovigilance practice (GVP).

Post-authorisation activities (section 2.12.)

This section lists type II variations, extension application according to Annex I of Reg. 1234/2008, re-examination procedures for type II variations (including extension of indication applications) for which the applicant has requested re-examination of the opinion previously issued by the CHMP and other issues concerning authorised medicines that are not covered elsewhere in the agenda such as annual reassessments, 5-year renewals, supply shortages, qualify defects. Issues that have been discussed at the previous meeting of the PRAC, the EMA's committee responsible for evaluating and monitoring safety issues for medicines, will also be included here.

Certification of ATMPs (section 3)

This section includes the scientific evaluation by the CAT of quality and non-clinical data that small and medium-sized enterprises have generated at any stage of the ATMP development process. More information on the ATMP certification procedure can be found here.

Scientific Recommendation on Classification of ATMPs (Section 4)

This section includes the scientific recommendation by the CAT on whether medicines based on genes, cells or tissues meet the scientific criteria that define ATMPs. More information on the ATMP classification procedure, including the outcomes of finalised classifications, can be found here.

Scientific Advice (section 5)

This section includes all scientific advice given to companies during the development of an ATMP. Information related to the number of ATMP related scientific advices discussed by CAT can be found in the CAT Monthly reports. Further information on SAWP can be found here.

Pre-Authorisation (section 6)

Paediatric Investigation Plan (PIP)

This section includes the discussion of an ATMP before a formal application for marketing authorisation is submitted. These cases refer for example to requests for an accelerated assessment

for medicines that are of major interest for public health or can be considered a therapeutic innovation: in case of an accelerated assessment the assessment timetable is reduced from 210 to 150 days.

CAT contributes to the evaluation of a Paediatric Investigation Plan (PIPs) for ATMPs by the Paediatric Committee. These PIPs are included in this section of the Agenda.

ITF Briefing meeting in the field of ATMPs

This section refers to briefing meetings of the Innovation Task Force and International co-operations activities of the CAT

The Innovation Task Force (ITF) is a body set up to encourage early dialogue with applicants developing innovative medicines. Minutes of meetings with applicants developing ATMPs and of other ITF meetings of interest to the CAT are included in this section of the agenda. Further information on the ITF can be found here.

Priority Medicines (PRIME)

This section includes the new requests for eligibility to PRIME for ATMPs under development, the discussions in CAT of these eligibility requests and the final recommendations for eligibility of ATMPs adopted by CHMP.

CAT will appoint one of its members as the CAT sponsor for each new ATMP eligibility request who will lead the CAT discussion based on the recommendation from the SAWP.

Organisational, regulatory and methodological matters (section 7)

This section includes topics related to regulatory and procedural guidance, CAT workplan, CAT meeting organisation (including CAT membership), planning and reporting, co-ordination with other committees, working parties and scientific advisory groups.

Furthermore, this section refers to the activities of the CAT drafting groups developing scientific guidelines for gene therapy medicinal products and for cell-based medicinal products, cooperation within the EU regulatory network and international regulators as well as direct interaction with interested parties. It also includes topics of scientific interest for the Committee that are not directly related to the work of the CAT drafting groups or CAT associated working parties.

Any other business (section 8)

This section is populated with miscellaneous topics not suitable under the previous headings.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/

10. List of participants

including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 22-24 January 2020 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Martina Schüssler-Lenz	Chair	Germany	No interests declared	
Ilona Reischl	Member	Austria	No interests declared	
Claire Beuneu	Member	Belgium	No interests declared	
Belaïd Sekkali	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Mirna Golemovic	Member	Croatia	No interests declared	
Petra Sokol	Alternate	Croatia	No interests declared	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	
Ondrej Palan	Alternate	Czech Republic	No interests declared	
Nanna Aaby Kruse	Alternate	Denmark	No restrictions applicable to this meeting	
Toivo Maimets	Member	Estonia	No interests declared	
Pille Saalik	Alternate	Estonia	No interests declared	
Heli Suila	Member	Finland	No interests declared	
Olli Tenhunen	Alternate	Finland	No interests declared	
Violaine Closson	Member	France	No interests declared	
Nathalie Morgensztejn	Alternate	France	No interests declared	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Katalin Lengyel	Member	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Paolo Gasparini	Member	Italy	No interests declared	
Giulio Pompilio	Alternate	Italy	No restrictions applicable to this meeting	
Una Riekstina	Member	Latvia	No interests declared	
John J. Borg	Member (CHMP member)		No interests declared	
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	
Maria Isabel Borba Vieira	Alternate (to CHMP representative)	Portugal	No interests declared	
Alina Musetescu	Member	Romania	No restrictions applicable to this meeting	
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	
Lisbeth Barkholt	Member	Sweden	No interests declared	
John Johnston	Member	United Kingdom	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	
Alessandro Aiuti	Member	Healthcare Professionals' Representative	Restrictions applicable to this meeting	
Alessandra Renieri	Alternate	Healthcare Professionals' Representative	No interests declared	
Kerstin Sollerbrant	Member	Patients' Representative	No interests declared	
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	
Michael Rosu-Myles	Observer/Alternate	European Directorate for the Quality of Medicine & HealthCare(EDQM)	No interests declared	
Gregor Abrahamsen	Expert - via telephone*	Norway	No interests declared	
Giuseppa Pistritto	Expert - joined by Adobe Connect (but there is no audio)	AIFA-IT	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.