18 April 2018
EMA/CAT/247071/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Advanced Therapies (CAT)

Agenda for the meeting on 18-20 April 2018

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

18 April 2018, 14:00 – 18:30, room 03-E

19 April 2018, 09:00 – 18:30, room 03-E

20 April 2018, 09:00 – 13:00, room 03-E

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 this agenda 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, this agenda is a working document primarily designed for CAT members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the agenda 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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Table of contents

1. Introduction 5

1.1. Welcome and declarations of interest of members, alternates and experts 5

1.2. Adoption of agenda 5

1.3. Adoption of the minutes 5

1.4. Technical information 5

2. Evaluation of ATMPs 5

2.1. Opinions 5

2.2. Oral explanations 5

2.3. Day 180 list of outstanding issues 5

2.3.1. Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480 5

2.4. Day 120 list of questions 5

2.5. Day 80 assessment reports 5

2.6. Update on ongoing initial applications 6

2.7. New applications 6

2.7.1. Axalimogene filolisbac - EMEA/H/C/004473 6

2.8. Withdrawal of initial marking authorisation application 6

2.9. Re-examination of initial application procedures under Article 9(2) of Regulation No. 726/2004 6

2.10. GMP and GCP inspections requests 6

2.11. Type II variations - variation of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008 6

2.11.1. Spherox - spheroids of human autologous matrix-associated chondrocytes - EMEA/H/C/002736/II/0001 6

2.11.2. Spherox - spheroids of human autologous matrix-associated chondrocytes - EMEA/H/C/002736/II/0002/G 6

2.11.3. Zalmoxis - allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2) - Orphan - EMEA/H/C/002801/II/0009/G 7

2.12. Other Post-Authorisation Activities 7

2.12.1. Zalmoxis - allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2) - Orphan - EMEA/H/C/002801/R/0010 7

3. Certification of ATMPs 7

3.1. Opinion 7

3.2. Day 60 Evaluation Reports 7

3.3. New Applications 7

4. Scientific Recommendation on Classification of ATMPs 8

4.1. New requests – Appointment of CAT Coordinator 8

4.1.1. Homogenate of antlerogenic stem cells - H0005050 8

4.1.2. Homogenate of antlerogenic stem cells - H0005051 8

4.1.3. Mixture of cultured human olfactory ensheathing cells and olfactory nerve fibroblasts - H0005049 8

4.1.4. CD34+ cells transduced with a lentiviral vector containing the Fanconi Anemia Complementation Group A (FANCA) gene – H0005064 8

4.1.5. Allogeneic CD34+ haematopoietic stem cells and allogeneic CD3+ T-cells – H0005068 8

4.2. Day 30 ATMP scientific recommendation 8

4.2.1. Allogeneic foetal neural stem cells (ALS) – H0005022 8

4.2.2. Allogeneic foetal neural stem cells (SCI) – H0005023 9

4.2.3. Fat graft - H00005024 9

4.2.4. Pegylated exosomes carrying recombinant cystic fibrosis transmembrane conductance regulator (CFTR) mRNA and microRNA-17 – H0005021 9

4.3. Day 60 revised scientific recommendation (following list of questions) 9

4.4. Finalisation of procedure 9

4.4.1. Allogeneic umbilical cord derived mesenchymal stem cells – H0004999 9

4.4.2. Autologous bone marrow derived mesenchymal stem cells - H0004998 9

4.4.3. Allogeneic human neural stem cells - H0004995 9

4.4.4. Autologous bone marrow derived mesenchymal stem cells - H0004997 10

4.4.5. *Ex vivo* fused autologous human bone marrow-derived mesenchymal stem cell (MSC) with allogenic human myoblast - H0004994 10

4.4.6. Elastin recombinamer (ELR)-encapsulated allogeneic pancreatic islets - H0004980 10

4.4.7. Autologous CD31+ Cells - H0004981 10

5. Scientific Advice 10

5.1. New requests – appointment of CAT Rapporteurs 10

5.2. CAT reports 11

5.3. List of Issues 11

5.4. Finalisation of SA procedures 11

6. Pre-Authorisation Activities 11

6.1. Paediatric investigation plans 11

6.2. ITF briefing meetings in the field of ATMPs 11

6.3. Priority Medicines (PRIME) – Eligibility requests 11

6.3.1. Month 0 - Start of the procedure 11

6.3.2. Month 1 – Discussion of eligibility 11

6.3.3. Month 2 – Recommendation of eligibility 11

6.3.4. Month 3 – Nomination of Rapporteurs 11

6.3.5. Ongoing support 11

7. Organisational, regulatory and methodological matters 12

7.1. Mandate and organisation of the CAT 12

7.1.1. CAT membership 12

7.1.2. Strategic Review & Learning meeting – Joint CHMP/PDCO/CAT, Oslo, Norway, 07-09 May 2018 12

7.1.3. Evaluation of marketing authorisation applications for advanced therapy medicinal products12

7.2. Coordination with EMA Scientific Committees..... 12

7.2.1. Committee for Medicinal Products for Human Use (CHMP) 12

7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups 12

7.3.1. Quality support to accelerated access schemes..... 12

7.3.2. Guideline on quality, non-clinical and clinical aspects of gene therapy medicinal products. 13

7.3.3. Guideline on requirements for investigational ATMPs..... 13

7.4. Cooperation within the EU regulatory network 13

7.4.1. European Union Network Training Centre (EU-NTC) 13

7.4.2. ATMP training curriculum 13

7.4.3. Orphan similarity for ATMPs 13

7.5. Cooperation with international regulators 13

7.6. CAT work plan 14

7.6.1. Expert meeting on genome editing, 18 October 2017, EMA, London..... 14

7.6.2. CAT meeting with Interested Parties, June 2018, EMA, London..... 14

7.7. Planning and reporting 14

7.7.1. Planning estimates of forthcoming ATMP MAAs 14

7.8. Others 14

7.8.1. Relocation of EMA to The Netherlands 14

7.8.2. Haemophilia registry workshop, 8 June 2018, London 14

8. Any other business 14

9. Explanatory notes 15

1. Introduction

1.1. Welcome and declarations of interest of members, alternates and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the CAT plenary session to be held on 18-19 April 2018. See April 2018 CAT minutes (to be published post-May 2018 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 18-20 April 2018 meeting

1.3. Adoption of the minutes

CAT minutes of 14-16 March 2018 meeting

1.4. Technical information

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. Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480

Kite Pharma EU B.V.; Intended for the treatment of B-cell lymphoma (DLBCL), primary mediastinal B-cell lymphoma (PMBCL) and transformed follicular lymphoma (TFL)

Scope: Day 180 list of outstanding issues

Action: for adoption

List of Questions adopted on 08.12.2017.

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

No items

2.7. New applications

2.7.1. Axalimogene filolisbac - EMEA/H/C/004473

Intended for the treatment of cervical cancerScope: timetable for assessment

Action: for adoption

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. Spherox - spheroids of human autologous matrix-associated chondrocytes - EMEA/H/C/002736/II/0001

CO.DON AG

Rapporteur: Lisbeth Barkholt, CHMP Coordinator: Kristina Dunder

Scope: Quality/non-clinical: Opinion

Update of Annex II to delete the condition “To re-validate the potency assay post marketing and to monitor its correlation with the efficacy outcome” following the submission of the revalidation of potency assay study report.

Action: for adoption

2.11.2. Spherox - spheroids of human autologous matrix-associated chondrocytes - EMEA/H/C/002736/II/0002/G

CO.DON AG

Rapporteur: Lisbeth Barkholt, CHMP Coordinator: Kristina Dunder

Scope: Safety and efficacy: RSI

Update of sections 4.2, 4.7, 4.8 and 5.1, of the SmPC in order to revise the wording and to update the safety and efficacy information based on the interim results from studies 16 HS 13 (24-month follow-up data) and 16 HS 14 (48-month follow-up data); the package leaflet is updated accordingly

Action: for adoption

2.11.3. Zalmoxis - allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2) - Orphan - EMEA/H/C/002801/II/0009/G

MolMed SpA

Rapporteur: Hans Ovelgönne, CHMP Coordinator: Paula Boudewina van Hennik

Scope: Quality: RSI

Action: for adoption

2.12. Other Post-Authorisation Activities

2.12.1. Zalmoxis - allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2) - Orphan - EMEA/H/C/002801/R/0010

MolMed SpA

Rapporteur: Hans Ovelgönne, CHMP Coordinator: Paula Boudewina van Hennik

Scope: 2nd annual renewal of marketing authorisation. RSI

Action: for adoption

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

4. Scientific Recommendation on Classification of ATMPs

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Homogenate of antlerogenic stem cells - H0005050

Intended for the treatment of recurrent corneal erosion syndrome

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.2. Homogenate of antlerogenic stem cells - H0005051

Intended for therapeutic support in spinal cord injury

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.3. Mixture of cultured human olfactory ensheathing cells and olfactory nerve fibroblasts - H0005049

Intended for the treatment of complete spinal cord injury in human patients

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.4. CD34+ cells transduced with a lentiviral vector containing the Fanconi Anemia Complementation Group A (FANCA) gene – H0005064

Intended for the treatment of Fanconi anemia type A patientsScope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.5. Allogeneic CD34+ haematopoietic stem cells and allogeneic CD3+ T-cells – H0005068

Intended for prevention of kidney transplant rejectionScope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.2. Day 30 ATMP scientific recommendation

4.2.1. Allogeneic foetal neural stem cells (ALS) – H0005022

Intended for the treatment of amyotrophic lateral sclerosis (ALS)

Scope: scientific recommendation

Action: for adoption

4.2.2. Allogeneic foetal neural stem cells (SCI) – H0005023

Intended for the treatment of spinal cord injury (SCI)

Scope: scientific recommendation

Action: for adoption

4.2.3. Fat graft - H00005024

Intended for lipofilling of anal fistula

Scope: scientific recommendation

Action: for adoption

4.2.4. Pegylated exosomes carrying recombinant cystic fibrosis transmembrane conductance regulator (CFTR) mRNA and microRNA-17 – H0005021

Intended for the treatment of cystic fibrosis

Scope: scientific recommendation

Action: for adoption

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

No items

4.4. Finalisation of procedure

4.4.1. Allogeneic umbilical cord derived mesenchymal stem cells – H0004999

Intended for the treatment of multiple sclerosis

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

Action: for information

4.4.2. Autologous bone marrow derived mesenchymal stem cells - H0004998

Intended for the treatment of multiple sclerosis

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

Action: for information

4.4.3. Allogeneic human neural stem cells - H0004995

Intended for the treatment of traumatic brain injuries (e.g. coma, minimally conscious state, persistent vegetative state) and stroke

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

Action: for information

4.4.4. Autologous bone marrow derived mesenchymal stem cells - H0004997

Intended for the treatment of articular cartilage damage and tendon injuries

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

Action: for information

4.4.5. *Ex vivo* fused autologous human bone marrow-derived mesenchymal stem cell (MSC) with allogenic human myoblast - H0004994

Intended for the treatment of Duchenne muscular dystrophy

Scope: comments received from the European Commission. Final scientific recommendation

Action: for information

4.4.6. Elastin recombinamer (ELR)-encapsulated allogeneic pancreatic islets - H0004980

Intended for treatment of severe forms of type 1 diabetes

Scope: comments received from the European Commission. Final scientific recommendation

Action: for information

4.4.7. Autologous CD31+ Cells - H0004981

Intended as adjunct therapy during primary care of proximal humeral fracture to decrease incidence of non-union and secondary displacement

Scope: comments received from the European Commission. Final scientific recommendation

Action: for information

5. Scientific Advice

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

5.1. New requests – appointment of CAT Rapporteurs

Timetable:

-Final Briefing Package:	08.05.2018
-Start of the procedure at SAWP:	17.05.2018
-CAT report due by:	17.05.2018
-CAT recommendation:	25.05.2018

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

Applications are available in MMD, PRIME, folder 2018-05:
https://docs.eudra.org/webtop/drl/objectId/0b0142b283ce9f29

Timetable for assessment:

Procedure start:	12.04.2018
SAWP recommendation:	17.05.2018
CAT recommendation:	25.05.2018
CHMP adoption of report and final recommendation:	31.05.2018

6.3.2. Month 1 – Discussion of eligibility

No items

6.3.3. Month 2 – Recommendation of eligibility

No items

6.3.4. Month 3 – Nomination of Rapporteurs

No items

6.3.5. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

Estonia: Pille Saalik – nominated as the new alternate from 2 April 2018

Italy: Giulio Pompilio - nominated as the new alternate from 20 March 2018

Luxembourg: Anne-Cécile Vuillemin - nominated as the new alternate from 24 March 2018

Action: for information

7.1.2. Strategic Review & Learning meeting – Joint CHMP/PDCO/CAT, Oslo, Norway, 07-09 May 2018

CAT resources: Helga Olsen, Rune Kjeken

Scope: final agenda. Programme can be found here: https://legemiddelverket.no/conference

Action: for adoption

Note: Strategic Review & Learning meeting partnered with CAT/CHMP/PDCO.

7.1.3. Evaluation of marketing authorisation applications for advanced therapy medicinal products

CAT: Martina Schüßler-Lenz

Scope: presentation on the workflow and timelines of the evaluation of MAAs for ATMPs by the CAT

Action: for information

Note: the procedural advice (EMA/630043/2008) can be found here (adopted by CAT and CHMP in December 2017)

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 March 2018 meeting

Action: for information

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

7.3.1. Quality support to accelerated access schemes

Scope: CAT contribution to the workshop planned for Q4 2018

Action: for discussion

7.3.2. Guideline on quality, non-clinical and clinical aspects of gene therapy medicinal products

CAT Rapporteurs: Quality: Christiane Niederlaender; Non-clinical: Kieran Breen; Clinical: Bettina Klug

Scope: final guideline and overview of comments. The documents are to be found here

Action: for information

Note: the final guideline was adopted by CAT and CHMP in March 2018. The overview of comments was adopted by CAT via written procedure after no comments were received.

7.3.3. Guideline on requirements for investigational ATMPs

Drafting group: Ilona Reischl (Rapporteur), Tiina Palomäki (Rapporteur), Simona Badoi, Tomáš Boráň, Violaine Closson-Carella, Paolo Gasparini, Carla Herberts, Metoda Lipnik-Stangelj, Margarida Menezes Ferreira, Christiane Niederlaender, Maura O'Donovan, Olli Tenhunen, Guido Pantè, Marcel Hoefnagel

Scope: feedback from drafting groups and next steps

Action: for discussion

7.4. Cooperation within the EU regulatory network

7.4.1. European Union Network Training Centre (EU-NTC)

Scope: presentation on best use of the EU-NTC

Action: for information

7.4.2. ATMP training curriculum

CAT: Ilona Reishl

Scope: update on proposal for the curriculum

Action: for discussion

7.4.3. Orphan similarity for ATMPs

CAT: Rocío Salvador-Roldán – European Commission

Scope: draft Q&A document

Action: for discussion

Note: this question and answer document will accompany the revised legislation on orphan similarity (which is expected to be published in May 2018)

7.5. Cooperation with international regulators

No items

7.6. CAT work plan

7.6.1. Expert meeting on genome editing, 18 October 2017, EMA, London

CAT: Martina Schüßler-Lenz

Scope: Final draft of the report on the genome editing expert meeting that took place on 18 October 2017 at EMA

Action: for adoption

Note: The report will, also, be adopted by the PgWP

7.6.2. CAT meeting with Interested Parties, June 2018, EMA, London

Scope: topics and timing for the meeting that will take place during the CAT plenary on 20-22 June 2018

Action: for discussion

7.7. Planning and reporting

7.7.1. Planning estimates of forthcoming ATMP MAAs

Scope: Q1/2018 update of the business pipeline report for the human scientific committees

Action: for information

7.8. Others

7.8.1. Relocation of EMA to The Netherlands

Scope: update on current state-of-play on EMA relocation

Action: for information

7.8.2. Haemophilia registry workshop, 8 June 2018, London

Scope: Haemophilia registry workshop to take place on 8 June 2018 at EMA (London, UK). The call for expressions of interest is open until 13 April.

Action: for information

Visit the EMA's website for further information, here

Note: the objective of this workshop is to ensure the practical implementation of the requirements related to patient registries for haemophilia medicinal products in Europe, in line with the draft revision of the guideline on the clinical investigation of recombinant and human plasma-derived factor VIII products.

8. Any other business

No items

Date of next CAT meeting:
23-25 May 2018

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

DG: Drafting Group

EC: European Commission

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

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

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

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

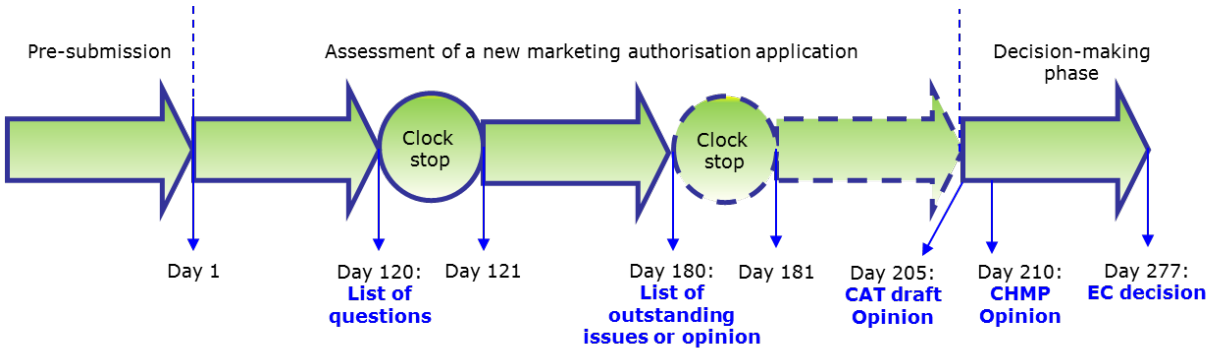
Evaluation of ATMPs (section 2)

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/