18 March 2020
EMA/CAT/143367/2020
Human Medicines Division

# Committee for Advanced Therapies (CAT)

## Agenda for the meeting on 18-20 March 2020

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

18 March 2020, 14:00 – 18:30

19 March 2020, 09:00 – 18:30

20 March 2020, 09:00 – 13:00

**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).

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# 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 18-20 March 2020. See March 2020 CAT minutes (to be published post-April 2020 CAT meeting).

## 1.2. Adoption of agenda

CAT agenda for 18-20 March 2020 meeting

## 1.3. Adoption of the minutes

CAT minutes for 19-21 February meeting

## 1.4. Technical information

# 2. Evaluation of ATMPs

## 2.1. Opinions

### 2.1.1. Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750

AveXis EU Ltd; treatment of spinal muscular atrophy (SMA)

Scope: Opinion

**Action:** for adoption

List of Outstanding Issues adopted on 06.12.2019, 21.06.2019. List of Questions adopted on 22.02.2019.

## 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. Autologous CD34+ cell enriched population that contains hematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector encoding the human arylsulfatase A gene - Orphan - EMEA/H/C/005321

**Accelerated assessment**

Orchard Therapeutics (Netherlands) BV; treatment of metachromatic leukodystrophy (MLD)

Scope: Day 120 list of questions

**Action:** for adoption

## 2.5. Day 80 assessment reports

No items

## 2.6. Update on ongoing initial applications

No items

## 2.7. New applications

## 2.8. Withdrawal of initial 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/0013/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: safety. Opinion

Submission of a group of 3 type II variations (C.I.4) to include:
- Long-term efficacy and safety of Kymriah in relapsed/refractory DLBCL based on the 24 months follow-up results from study CCTL019C2201 (update of sections 4.4, 4.8, 5.1 and 5.2 of the SmPC)
- Interim results from study CCTL019B2202 (update of sections 4.4, 4.8, 5.1 and 5.2 of the

SmPC)
- Interim results from study CCTL019B2205J (update section 5.2 of the SmPC)

The Marketing authorisation holder (MAH) took the opportunity to clarify the wording of the indication to include patients over 25 years of age and to introduce some minor editorial corrections throughout the SmPC and the Package Leaflet. The MAH also updated sections 4.2, 4.4, 4.8 of the SmPC, Annex II and the Package Leaflet with regards to the acceptability of having 1 dose of tocilizumab per patient per centre for the management of cytokine release syndrome.

In addition, the MAH requested updates to sections 2.2 and 6.5 of the SmPC, the labelling and to the package leaflet to accommodate the administration of additional infusion bags, when applicable.

The requested group of variations proposed amendments to the Summary of Product Characteristics, Annex II, Annex IIIA and Package Leaflet and to the Risk Management Plan (RMP). The RMP version 2.1 has been agreed.

**Action:** for information

Note: additional changes to the SmPC were implemented by CAT and CHMP. The revised Opinion was adopted by written procedure on 4 March 2020

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

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: quality. Opinion

**Action:** for adoption

Request for Supplementary Information adopted on 24.01.2020.

### 2.11.3. Strimvelis - autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ANA cDNA sequence - Orphan - EMEA/H/C/003854/II/0024

Orchard Therapeutics (Netherlands) BV

Rapporteur: Sol Ruiz, PRAC Rapporteur: Menno van der Elst

Scope: RSI

Safety. Update of sections 4.8 and 5.1 of the SmPC in order to update the safety information following the completion of the STRIM-004 study, which is a non-interventional long term follow up of the subjects who received Strimvelis gene therapy. This study included paediatric patients and is listed as a category 3 study in the RMP. The Package Leaflet is updated accordingly. The RMP version 3.0 has also been submitted. In addition, the Marketing authorisation holder (MAH) took the opportunity to introduce minor administrative changes in the PI.

**Action:** for adoption

### 2.11.4. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0018

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: quality

**Action:** for adoption

### 2.11.5. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0019

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: quality

**Action:** for adoption

## 2.12. Extension applications

No items

## 2.13. Other Post-Authorisation Activities

### 2.13.1. Glybera (Expired) - alipogene tiparvovec - Orphan - EMEA/H/C/002145/SOB/001.9

uniQure biopharma B.V.

Rapporteur: Egbert Flory, CHMP Coordinator: Jan Mueller-Berghaus

Scope: pharmacovigilance

Long term surveillance programme/ disease registry to collect information on the epidemiology of the disease and the demographics, safety, and the effectiveness outcomes of patients treated with Glybera.
The patients enrolled in clinical studies (CT-AMT-010 -10, CT-AMT 011-01, CT-AMT 011-02) should be followed up in the LPLD registry.
All patients treated with Glybera should be enrolled in the registry and systematic data collection carried out to enrich the database:
1) on efficacy data such as biochemical markers as part of normal practice and frequency and severity of pancreatitis and
2) on safety including immunogenicity against Glybera and LPL.
3) Dietary diary and quality of life data should also be recorded.
The diagnosis of LPLD has to be confirmed by genetic testing.
[NOTE: 15 years follow-up is recommended for every patient treated.]

**Action:** for adoption

### 2.13.2. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/006

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, CHMP Coordinator: Jan Mueller-Berghaus

Scope: quality

**Action:** for adoption

### 2.13.3. 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/REC/006.1

bluebird bio (Netherlands) B.V

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

Scope: quality.

**Action:** for adoption

### 2.13.4. 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/REC/009

bluebird bio (Netherlands) B.V

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

Scope: quality. From Initial MAA

**Action:** for adoption

### 2.13.5. 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/REC/010

bluebird bio (Netherlands) B.V

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

Scope: quality

**Action:** for adoption

### 2.13.6. 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/REC/011

bluebird bio (Netherlands) B.V

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

Scope: quality. From Initial MAA

**Action:** for adoption

### 2.13.7. 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/REC/012

bluebird bio (Netherlands) B.V

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

Scope: quality

**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. Gene-activated matrix based on octacalcium phosphate and a plasmid carrying VEGF-A gene –

Intended to various bone healing indications (sinus lift, non-unions, spinal fusion, etc.)

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.2. Leuco platelet enriched plasma –

Intended for the treatment of ulcers, chronic wounds

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.3. Recombinant adeno-associated viral vector rh74 containing the human beta-sarcoglycan gene –

Intended for the treatment of limb-girdle muscular dystrophy type 2E

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.4. Wharton's jelly derived mesenchymal stem cell , drug resistant epilepsy

Intended for the treatment of drug resistant epilepsy

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.5. Autologous adipose-derived mesenchymal stem cell , diabetic foot syndrome

Intended for the treatment of diabetic foot syndrome

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.6. Wharton's jelly derived mesenchymal stem cell , Behcet disease

Intended for the treatment of Behcet disease

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.7. Wharton's jelly derived mesenchymal stem cell , choroideremia

Intended for the treatment of choroideremia

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.8. Wharton's jelly derived mesenchymal stem cell , foetal alcohol syndrome

Intended for the treatment of foetal alcohol syndrome

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.9. Wharton's jelly derived mesenchymal stem cell , frontotemporal dementia

Intended for the treatment of frontotemporal dementia

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.10. Wharton's jelly derived mesenchymal stem cell , progressive bulbar palsy

Intended for the treatment of progressive bulbar palsy

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

### 4.1.11. Wharton's jelly derived mesenchymal stem cell , vitelliform macular degeneration

Intended for the treatment of vitelliform macular degeneration (Best disease)

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

## 4.2. Day 30 ATMP scientific recommendation

### 4.2.1. Recombinant chimeric vesicular stomatitis virus carrying the envelope glycoprotein (GP) of the visceral non-neurotropic strain of the lymphocytic choriomeningitis virus

Intended for the treatment of solid tumours, including non-small cell lung carcinoma.

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.2. Autologous CD34+ cells transduced with CL20-4i-EF1α-hγc-OPT lentiviral vector – H0005602

Intended for the treatment of X-linked severe combined immunodeficiency (XSCID)

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.3. Wharton's jelly derived mesenchymal stem cells, AMN

Intended for the treatment of adrenomyeloneuropathy (AMN)

Scope: ATMP 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. Autologous, *ex vivo* expanded, clonal neoantigen specific tumour infiltrating lymphocytes – H0005575

Intended for the treatment of solid tumours

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

**Action:** for information

### 4.4.2. Autologous adipose derived mesenchymal stem cells, ALS – H0005580

Intended for the treatment of Amyotrophic Lateral Sclerosis (ALS)

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

**Action:** for information

### 4.4.3. Wharton jelly derived mesenchymal stem cells – H0005608

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

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

**Action:** for information

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

Intended for the treatment of CD19+ haematological malignancies

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

**Action:** for information

### 4.4.5. 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: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

### 4.4.6. Micronized autologous adipose tissue particles and costal cartilage powder – H0005607

Intended for the treatment of cartilage defects

Scope: comments received from the European Commission. Revised ATMP scientific recommendation

**Action:** for information

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

Intended for the treatment of sensorineural hearing loss

Scope: comments received from the European Commission. Revised ATMP scientific recommendation

**Action:** for information

### 4.4.8. Wharton's jelly derived mesenchymal stem cells, ALS – H0005619

Intended for the treatment of Amyotrophic Lateral Sclerosis (ALS)

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

**Action:** for information

### 4.4.9. Wharton's jelly derived mesenchymal stem cell , Huntington's disease - H0005571

Intended for the treatment of Huntington's disease

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

**Action:** for information

### 4.4.10. Wharton's jelly derived mesenchymal stem cell , Lewy body dementia (LBD) - H0005572

Intended for the treatment of Lewy body dementia (LBD)

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

**Action:** for information

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

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

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

**Action:** for information

### 4.4.12. Autologous adipose-derived mesenchymal stem cells ex-vivo expanded, alopecia - H0005567

intended for the treatment of alopecia

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

**Action:** for information

LoQs adopted on 24 January 2020

### 4.4.13. Autologous adipose-derived mesenchymal stem cells *ex-vivo* expanded, hypertrophic scars - H0005568

Intended for the treatment of hypertrophic scars

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

**Action:** for information

LoQs adopted on 24 January 2020

## 4.5. Follow-up and guidance

No items

# 5. Scientific Advice

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

## 5.1. New requests – appointment of CAT Rapporteurs

## 5.2. CAT reports

## 5.3. List of Issues

No items

## 5.4. Finalisation of SA procedures

# 6. Pre-Authorisation Activities

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

## 6.1. Paediatric investigation plans

No items

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

No items

## 6.3. Priority Medicines (PRIME) – Eligibility requests

### 6.3.1. Month 0 - Start of the procedure

### 6.3.2. Month 1 – Discussion of eligibility

### 6.3.3. Month 2 – Recommendation of eligibility

### 6.3.4. Ongoing support

# 7. Organisational, regulatory and methodological matters

## 7.1. Mandate and organisation of the CAT

### 7.1.1. CAT membership

Romania: Alina Musetescu – Membership mandate ended on 12 March 2020

**Action:** for information

### 7.1.2. Strategic Review & Learning meeting (SRLM) – Budapest, Hungary, 08 – 10 June 2020

CAT: Katalin Lengyel

Scope: topics for inclusion in the joint CAT-PDCO agenda and CAT-only agenda to take place on 08-10 June 2020

**Action:** for discussion

### 7.1.3. Strategic Review & Learning meeting (SRLM) – Helsinki, Finland, 21 – 22 November 2019

CAT: Heli Suila

Scope: minutes of the meeting that took place on 21-22 November 2019

**Action:** for discussion

## 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 February 2020 meeting

**Action:** for information

### 7.2.2. CAT-COMP Working Group

CAT core members: Kieran Breen, Carla Herbert, Maura O'Donovan, Maja Sommerfelt and Martina Schüßler-Lenz

Scope: agenda for the monthly COMP-CAT Working Group to take place on 18 March 2020 from 18:30-19:30hrs

**Action:** for discussion

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

### 7.3.1. Inspectors Working Group (IWG)

*Postponed*

Scope: feedback on discussion in the IWG on inspection of viral vector manufacturer and on decentralised manufacture

**Action:** for information

### 7.3.2. Quality Review of documents (QRD) - agreement on ATMPs

**Action:** for information

### 7.3.3. Scientific Advice Working Party

Scope: SAWP re-nomination composition, including the CAT nominees.

**Action:** for information

Note**:** adopted by the CHMP at is March 2020 ORGAM meeting

## 7.4. Cooperation within the EU regulatory network

### 7.4.1. European Commission's questionnaire on new genomic techniques

Scope: stakeholders' consultation on new genomic techniques

**Action:** for information

https://ec.europa.eu/food/plant/gmo/modern_biotech/stakeholder-consultation_en

Note: the European Commission is carrying out targeted consultations with Member States and EU-level stakeholders to gather information for the Commission study on new genomic techniques.

### 7.4.2. Public statement on the use of unregulated/unproven ATMPs

Scope: feedback on next steps

**Action**: for information

## 7.5. Cooperation with international regulators

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

Scope: feedback from the teleconference

**Action:** for discussion

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

CAT: Pille Säälik

Scope: agenda of the teleconference that will take place on 26 March 2020

**Action:** for discussion

## 7.6. CAT work plan

None

## 7.7. Planning and reporting

### 7.7.1. Planning estimates of forthcoming ATMP MAAs

Scope: Q1/2020 update of the business pipeline report for the human scientific committees

**Action:** for information

## 7.8. Others

## 7.9. Others

### 7.9.1. Draft QWP/BWP guideline on the quality requirements for drug device combinations

Rapporteur: Nicholas Lee (IE, QWP)

CAT: Ilona Reischl

Scope: Presentation of the revised draft following public consultation

**Action**: for discussion

# 8. Any other business

### 8.1. European Conference for Rare Diseases

Scope: identification of CAT speaker for the session: *Innovation in Advanced Therapy* (Saturday 16th May 2020, 11:00 – 12:30)

**Action:** for action

Note: the conference will be conducted virtually. For more information on the conference, see: https://www.rare-diseases.eu/

Date of next CAT meeting:

22-24/04/2020

## 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

EU NTC: European Union Network Training Centre

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

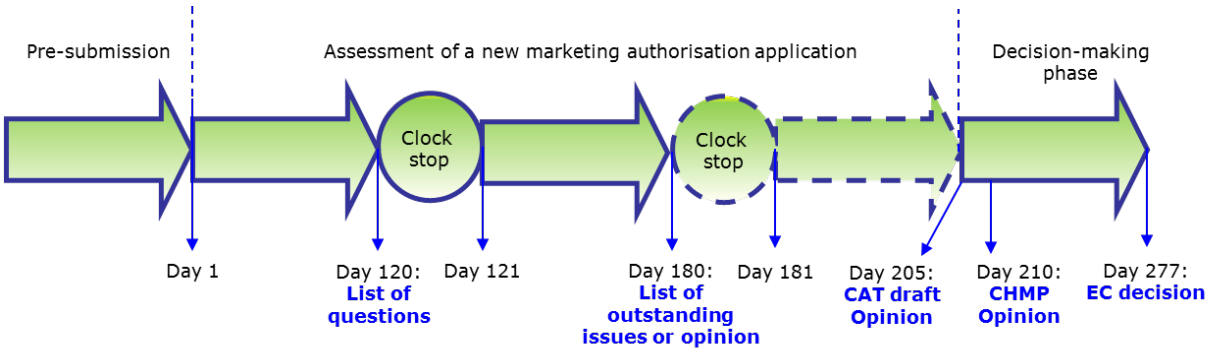
## Evaluation of ATMPs (section 2)

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/