16 June 2021
EMA/342564/2021
Human Medicines Division

Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 16-18 June 2021

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

16 June 2021, 14:00 – 18:30, virtual

17 June 2021, 09:00 – 18:30, virtual

18 June 2021, 09:00 – 13:00, virtual

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 16-18 June 2021. See June 2021 CAT minutes (to be published post-July 2021 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 16-18 June 2021 meeting

1.3. Adoption of the minutes

CAT minutes for 10-12 May meeting

2. Evaluation of ATMPs

2.1. Opinions

2.1.1. Idecabtagene vicleucel - Orphan - EMEA/H/C/004662

Celgene Europe BV; treatment of multiple myeloma

Scope: Opinion

Action: for adoption

List of Outstanding Issues adopted on 19.02.2021, 04.12.2020. List of Questions adopted on 11.09.2020.

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. Lisocabtagene maraleucel / lisocabtagene maraleucel - Orphan - EMEA/H/C/004731

Bristol-Myers Squibb Pharma EEIG; treatment of large B-cell lymphoma, diffuse large B-cell lymphoma (DLBCL), primary mediastinal large B-cell lymphoma (PMBCL) and follicular lymphoma grade 3B (FL3B)

Scope: MAA's request (dated 28.05.2021) for a clock-stop extension

Action: for adoption

List of Questions (LoQ) adopted on 06.11.2020. List of Outstanding Issues (LoOI) adopted on 16.04.2021

2.7. New applications

2.7.1. Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095

Accelerated assessment

Janssen-Cilag International NV; treatment of multiple myeloma

Scope: Timetable for assessment

Action: for adoption

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 and variations of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008

2.11.1. Zynteglo - betibeglogene autotemcel - Orphan - EMEA/H/C/003691/II/0025

bluebird bio (Netherlands) B.V

Rapporteur: Carla Herberts

Scope: Request for supplementary information (RSI)
Quality

Action: for adoption

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/ANX/003.5

Novartis Europharm Limited

Rapporteur: Rune Kjeken, CHMP Coordinator: Ingrid Wang, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: PhV
Study CCTL019B2401:
Non-interventional post-authorisation safety study (PASS): In order to further characterise the safety – including long-term safety – of Kymriah, the applicant should conduct and submit a study based on data from a disease registry in ALL and DLBCL patients. Third semi-annual report (EBMT data only).

Action: for adoption

2.13.2. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/P46/012

Novartis Europharm Limited

Rapporteur: Rune Kjeken, CHMP Coordinator: Ingrid Wang

Scope: Clinical
Paediatric studies submitted in accordance with Article 46 of Regulation (EC) No1901/2006, as amended. FINAL STUDY REPORT, Study no. CTL019B2001X, EudraCT no. 2016-001991-31 (Phase IIIb study for relapsed/refractory paediatric/young adult acute lymphoblastic leukaemia patients to be treated with CTL019).

Action: for adoption

2.13.3. Libmeldy - atidarsagene autotemcel - Orphan - EMEA/H/C/005321/ANX/002

Orchard Therapeutics (Netherlands) BV

Rapporteur: Carla Herberts, CHMP Coordinator: Johann Lodewijk Hillege. PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: clinical and PhV. Submission of the long TERM-MLD (metachromatic leukodystrophy) study protocol: In order to further characterise the long-term efficacy and safety of Libmeldy in children with late infantile or early juvenile forms of MLD, the MAH shall conduct and submit the results of a prospective study based on data from a registry, according to an agreed protocol.
Action: for adoption

2.13.4. Strimvelis - autologous cd34+ enriched cell fraction that contains cd34+ cells transduced with retroviral vector that encodes for the human ada cdna sequence - Orphan - EMEA/H/C/003854/ANX/004.3

Orchard Therapeutics (Netherlands) BV

Rapporteur: Sol Ruíz, CHMP Coordinator: Maria Concepción Prieto Yerro, PRAC Rapporteur: Menno van der Elst

Scope: Clinical and PhV. Second Interim Patient Registry / STRIM-0003: Adenosine Deaminase Severe Combined Immunodeficiency (ADA-SCID) Registry for Patients Treated with Strimvelis (or GSK2696273) Gene Therapy: Long-Term Prospective, Non-Interventional

Follow-up of Safety and Effectiveness [Interim reports submitted every 2 years].
Action: for adoption

2.13.5. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/010

Kite Pharma EU B.V.

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

Scope: From IB/0020:
In frame of on-going process verification the MAH is asked to provide batch data within 6 months of manufacturing start added at TCF04.

Action: for adoption

2.13.6. Zynteglo - betibeglogene autotemcel - Orphan - EMEA/H/A-20/1504

bluebird bio (Netherlands) B.V

CAT-PRAC group: CAT: Carla Herberts (Rapporteurs) and Violaine Closson-Carella (Co-Rapporteur), Alessandro Aiuti; PRAC: Brigitte Keller-Stanislawski, Menno van der Elst

Scope: referral procedure under Article 20 PhV. Feedback from PRAC discussion

Action: for information

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 ITF Coordinator

4.1.1. Autologous population of selected renal cells (SRC)

Intended for the treatment of chronic kidney disease (CKD)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.2. Autologous adipose mesenchymal stem cells (MSCs)

Indicated for cartilage defects of degenerative origin and for the treatment of osteoarthritis

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.3. Allogeneic natural killer cells armed with anti-CD20 monoclonal antibody

Intended for the treatment of B-Cell Non-Hodgkin lymphoma

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.4. Recombinant serotype 9 adeno-associated virus encoding a codon-optimised human galactosylceramidase transgene [ssAAV9/CBA-hsaGALCopt2-SV40p (AAV9-hGALC)]

Intended for the treatment of Krabbe disease

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.5. Minimally manipulated autologous pancreatic islets

Intended for the treatment of chronic pancreatitis and recurrent acute pancreatitis immediately following pancreatectomy

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.6. Extracellular matrix and non-viable osteogenic cells derived from human adipose-derived stem cells, associated with hydroxyapatite/beta-tricalcium phosphate (HA/βTCP) particles

Intended to stimulate bone regeneration in pathological hypoxic and/or necrotic bone conditions

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.7. HEK293 cells transfected with a lentiviral vector to express the tumour-specific antigen, WT1 and the antigen presenting molecule, cluster of differentiation 1d (CD1d).

Intended for the treatment of WT1-expressing tumours

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.8. Ribonucleoprotein (RNP), a complex of Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) Cas 9 and sgRNA, delivered by a novel synthetic non-viral vector, for the excision of exon 80 of the human COL7A1 gene

Intended for the treatment of recessive dystrophic epidermolysis bullosa (RDEB)

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.9. Isolated CD31+ cells

Intended for the treatment of erectile dysfunction

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.10. Live human mesenchymal stem cells derived from allogeneic Wharton's jelly

Intended for the treatment of rheumatoid arthritis, unspecified

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.11. Live human mesenchymal stem cells derived from allogeneic Wharton's jelly

Intended for the treatment of systemic lupus erythematosus, unspecified

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.12. Live human mesenchymal stem cells derived from allogeneic Wharton's jelly

Intended for the treatment of systemic sclerosis, unspecified

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.2. Day 30 ATMP scientific recommendation

4.2.1. Nanoparticle consisting of non-pseudotyped (bald) lentiviral vector encoding for a CD19 CAR encapsulated

Intended for the treatment of CD19+ B-cell malignancy

Scope: ATMP scientific recommendation

Action: for adoption

4.2.2. Autologous T cells genetically modified ex vivo using a synthetic chromosome encoding CCR6, IL-2, a truncated version of CD34 and two independent inducible safety switches

Intended for treatment of patients with solid tumours for which a draining lymph node can be identified. Initially the product will be developed for colon cancer and urinary bladder cancer

Scope: ATMP scientific recommendation

Action: for adoption

4.2.3. Live human mesenchymal stem cells derived from allogeneic Wharton's jelly

Intended for treatment of atherosclerosis of the arteries of the lower extremities

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. Allogeneic, expanded, engineered E4ORF1+ human umbilical cord endothelial (CD31+) cells

Intended to treat organ vascular niche injuries caused by myeloablative, non-central nervous system penetrating high-dose chemotherapy (HDT) to prevent the development of severe regimen-related toxicities (SRRT) in patients diagnosed with aggressive systemic lymphoma

Scope: the European Commission raised minor comments. ATMP Scientific recommendation

Action: for information

List of Questions adopted on16.04.2021

4.4.2. Allogeneic human Wharton's jelly derived mesenchymal stem cells

Intended for the treatment of idiopathic pulmonary fibrosis (IPF), pulmonary fibrosis after COVID-19

Scope: the European Commission raised no comments. ATMP Scientific recommendation

Action: for information

4.4.3. *Ex-vivo* expanded autologous cryopreserved Wharton's Jelly derived mesenchymal stem cells

Intended for the treatment of Bronchopulmonary Dysplasia (BPD) for preterm infants

Scope: the European Commission raised no comments. ATMP Scientific recommendation

Action: for information

4.4.4. Allogeneic corneal endothelial cells in a confluent monolayer adhering to a cornea-shaped sheet of cross-linked collagen

Intended for the treatment of corneal dysfunction

Scope: the European Commission raised minor comments. ATMP Scientific recommendation

Action: for information

4.4.5. Proliferation arrested myelomonocytic leukemic cell line-derived cells with a mature dendritic cell phenotype

Intended for the treatment of acute myeloid leukaemia

Scope: the European Commission raised no comments. ATMP 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 Peer-reviewers

5.1.1. Ongoing scientific advice procedures – Appointment of CAT Peer-Reviewers

Timetable:

-Start of the procedure at SAWP:	07-10.06.2021
-Appointment of CAT Peer-reviewers:	18.06.2021
-SAWP first reports:	28.06.2021
-CAT Peer-Reviewer's comments:	02.07.2021
-Discussion at SAWP:	05-08.07.2021
-Discussion at CAT and feedback from SAWP:	17.07.2021

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

-Start of procedure at SAWP:	05-08.07.2021
-Appointment of CAT Peer-Reviewers:	16.07.2021
-SAWP first reports:	23.08.2021
-CAT Peer-Reviewer's comments:	27.08.2021
-Discussion at SAWP:	30 Aug.-02 Sept.2021
-Discussions at CAT and feedback from SAWP:	10.09.2021

5.2. Procedures discussed at SAWP – 1st reports, D40 JRs, LoIs

5.3. Finalisation of D70 procedures – feedback from the discussion meeting

5.4. Final Advice Letters for procedures finalised the previous month

No items

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

Timetable for assessment:

Procedure start:	10.06.2021
SAWP recommendation:	08.07.2021
CAT recommendation:	16.07.2021
CHMP adoption of report and final recommendation:	22.07.2021

6.3.2. Month 1 – Discussion of eligibility

No items

6.3.3. Month 2 – Recommendation of eligibility

6.3.4. Ongoing support

No items

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

Finland – Olli Tenhunen – membership mandate (alternate) ended 14.05.2021

Action: for information

7.1.2. Strategic Review & Learning meeting (SRLM) under the Portuguese presidency of the European Union – 27th May 2021, Lisbon, Portugal

CAT: Bruno Sepodes, Maria-Isabel Vieira

Scope: feedback from the meeting that took place on 27th May 2021

Action: for information

See also 7.6.1

7.1.3. Strategic Review & Learning (virtual) meeting (SRLM) under the Slovenian presidency, 20-21 October 2021, Ljubljana (Slovenia)

CAT: Metoda Lipnik-Štangelj, Martina Schuessler-Lenz

Scope: initial discussion on agenda content for the joint CHMP/CAT meeting

Action: for discussion

7.1.4. Planning estimates of forthcoming ATMP MAAs

Scope: Q2/2021 update of the business pipeline report for the human scientific committees

Action: for information

7.2. Coordination with EMA Scientific Committees

7.2.1. CHMP learnings that impact CAT decisions

CAT: Jan Mueller-Berghaus, Romaldas Mačiulaitis, John-Joseph Borg, Bruno Sepodes, Sol Ruíz

Scope: CHMP learnings with relevance to CAT

Action: for information

7.2.2. CAT-COMP Working Group

CAT: Martina Schuessler-Lenz

Scope: feedback from the CAT-COMP working group meeting that took place on 14 June 2021

Action: for information

7.2.3. Scientific Coordination Board (SciCoBo) – meeting of 29th June 2021

CAT: Martina Schuessler-Lenz

Scope: CAT feedback is sought on innovative developments and technologies in the ATMP field that are to be taken forward in the 'Horizon Scanning' activity

Action: for discussion

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

7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

Scope: draft agenda of the PCWP/HCPWP joint meeting that took place on 01-02 June 2021

Action: for information

7.3.2. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

Scope: minutes on the PCWP/HCPWP joint meeting that took place on 3-4 March 2021

Action: for information

Note: the CAT Chair - Martina Schuessler-Lenz - took part on the topic of: *'Timely patients' access to advanced therapy medicinal products in the EU'*

7.3.3. Scientific advice (SA) procedure for ATMPs

Scope: Update of sub-headings in the CAT agenda and MMD.

Action: for information

7.4. Cooperation within the EU regulatory network

7.4.1. Revision of the EU legislation on blood, tissues and cells (BTC)

CAT: Martina Schuessler-Lenz

Scope: feedback from the workshop of 9 June 2021 on 'Borderlines with Other Regulated Frameworks: Classification Advice and Interplay'

Action: for discussion

7.4.2. EU-Innovation Network Borderline Classification Group (BLCG)

Scope: identification of CAT member to take part in BLCG

Action: for discussion

7.4.3. EU questionnaire on GMO assessment for medicinal products for human use

Action: for information

Note: the questionnaire has been sent out to the national competent authorities

7.5. Cooperation with international regulators

7.5.1. ATMP cluster teleconference with US-FDA, Health Canada and PMDA (Japan)

CAT: Martina Schuessler-Lenz *et al*

Scope: feedback on the teleconference that took place on 20 May 2021

Action: for information

Note: following CAT members/experts attend the ATMP cluster: Egbert Flory, Isabel Vieira, Belaïd Sekkali, Violaine Closson-Carella, Maura O'Donovan, Rune Kjeken, Marie Lüttgen, Alessandra Renieri, Roland Pochet, Ivana Haunerova, Maja Sommerfelt, Heli Suila, Carla Herberts, Rocio Salvador Roldan, Brigitte Keller Stanislawski, Brigitte Anliker (expert), Atilla Sebe (expert) and Beate Mosl (expert)

7.5.2. Ad-hoc teleconference between CAT and US-FDA

CAT: Maura O'Donovan

Scope: teleconference took place 17 May 2021

Action: for information

Note: CAT discussed the scientific advice for this product during its January 2021 meeting

7.5.3. International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) ICH S12

CAT members: Rune Kjeken, Claire Beuneu - EU Rapporteurs

Scope: feedback on the ICH S12 guideline: non-clinical biodistribution studies for gene therapy products definition of gene therapy medicinal products'

Action: for information

Note: ICH S12 was endorsed and signed by the ICH Assembly on 3rd June 2021.

The document will go for adoption to the CHMP at its PROM (formerly known as ORGAM) meeting on 14 June 2021 before being published for a four-month public consultation.

7.6. CAT work plan

7.6.1. Comprehensiveness of clinical data in marketing authorisations

CAT: Martina Schuessler-Lenz

Scope: criteria for comprehensiveness of clinical data in marketing authorisation applications

Action: for discussion

Note: discussed at the SRLM of 27 May 2021 - see also: 7.1.2

7.7. Planning and reporting

None

7.8. Others

7.8.1. Research and innovation workstream

Scope: horizon scanning report on genome editing

Action: for information

Note: a report on genome editing has been finalised by the EU Innovation network (EU IN; EMA Rapporteur; contributions by experts from the EU Network, Committees and Working parties). It covers the current status and activities, as well as specific challenges and opportunities, leading to recommendations for genome editing medicinal product development and regulation in the next decade.

7.8.2. Patient engagement in MAAs - pilot

Scope: the aim is to enable patients to share their experience, concerns and needs related to their condition with the Rapporteurs/committee so that this can be considered in a timely manner during the assessment process, if appropriate.

Action: for information

7.8.3. DIA Global Annual meeting 2021 – Session on Gene Therapy, 18 June 2021

CAT: Martina Schuessler-Lenz

Scope: 'Gene Therapy: Getting Back on Track After COVID-19'. Learning objective: discuss the effect of the COVID-19 pandemic on the development of gene therapies; Identify the need for new policy initiatives to help expedite the gene therapies both in the US and globally. Moderator: Janet Lynch Lambert (ARM). Panellists: Peter W. Marks (CBER-FDA), Adora Ndu (Biomarin) and Martina Schuessler-Lenz

Action: for information

Note: the session was pre-recorded on 8th June 2021.
Link: Gene Therapy: Getting Back on Track After COVID-19 (diaglobal.org)

7.8.4. CASSS: Cell and Gene Therapy Products: Manufacturing, Quality and Regulatory Considerations, 8-10 June 2021

CAT: Heli Suila

Scope: feedback from the meeting

Action: for information

7.8.5. European Health Forum Gastein (EHFG) – Health Talks: 'Transforming the future of healthcare – do cell and gene therapies hold the key?', 15 June 2021

CAT: Ilona Reischl

Scope: feedback from the meeting

Action: for information

7.8.6. 6th Industry Stakeholder Platform on R&D support, 4th June 2021

Scope: feedback from industry observations of the Q&A on similarity for ATMPs

Action: for information

8. Any other business

8.1. Alliance for Regenerative Medicine (ARM) - chemistry, manufacturing and controls (CMC) virtual workshop for 21st September 2021

CAT: Martina Schuessler-Lenz, Ilona Reischl

Scope: speaking opportunity to present EMA's PRIME toolbox

Action: for nomination of a CAT speaker

8.2. Alliance for Regenerative Medicine (ARM) - chemistry, manufacturing and controls (CMC) virtual workshop for pluripotent stem cell derived therapeutics, 16th-17th November 2021

CAT: Martina Schuessler-Lenz, Ilona Reischl

Scope: speaking opportunity on the quality development aspect of pluripotent stem cell-derived medicinal products

Action: for nomination of a CAT speaker

Date of next CAT meeting:

14-16/07/2021

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

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

QRD: Quality review of documents

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: Safety Working Party

SME: Small and medium size enterprises

SmPC: Summary of Products Characteristics

TT: Timetable

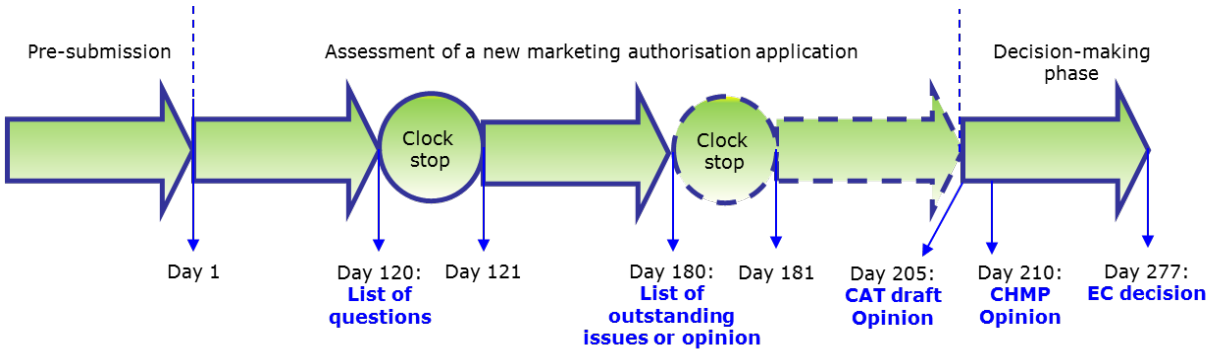
Evaluation of ATMPs (section 2)

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/