7 September 2022
EMA/CAT/646878/2022
Human Medicines Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 13-15 July 2022

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

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in these minutes are considered commercially confidential or sensitive and therefore not disclosed. Regarding 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, these minutes are a working document primarily designed for CAT members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the minutes cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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1. Introduction

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

The Chairperson opened the meeting by welcoming all participants. Due to the coronavirus (COVID-19) outbreak, and the associated EMA Business Continuity Plan (BCP), the meeting was held remotely.

In accordance with the Agency's policy on handling of declarations of interests of scientific committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and on the topics in the agenda of the current meeting, the Committee Secretariat announced the restricted involvement of some Committee members, alternates and experts for concerned agenda points.

Participants were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions were declared. Restrictions applicable to this meeting are captured in the List of participants included in the minutes.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The chairperson welcomed the new members/alternates representing the clinicians and the patient organisations.

1.2. Adoption of agenda

The CAT agenda for 13-15 July 2022 meeting was adopted with one addition to section 7.8: Regulatory and Scientific conference on RNA-based medicines.

1.3. Adoption of the minutes

The CAT minutes for 15-17 June 2022 meeting were adopted.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

2.4.1. Etranacogene dezaparvovec - PRIME - Orphan - EMEA/H/C/004827

Accelerated assessment

CSL Behring GmbH; treatment of adults with Haemophilia B

Scope: Day 120 list of questions

Action: for adoption

The CAT Rapporteurs presented the outcome of the assessment of the marketing authorisation application

CAT adopted the list of questions. CAT agreed to revert to a normal timetable.

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

2.11.1. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0002

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality. Opinion

Action: for adoption

The opinion was adopted.

2.11.2. Breyanzi - lisocabtagene maraleucel / lisocabtagene maraleucel - EMEA/H/C/004731/II/0003

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality. Request for supplementary information

Action: for adoption

The Rapporteur presented the assessment of this quality variation

The request for supplementary information was adopted.

2.11.3. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/II/0051

Amgen Europe B.V.

PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: Pharmacovigilance

Submission of the final report from study 20180062: "Observational Research Study Report (ORSR)" listed as a category 3 study in the RMP. This is a multinational, non-interventional, cross-sectional survey study for the patients aged ≥ 18 years who have received Imlygic at least once in the 3 months prior to completing the survey to evaluate the effectiveness of the patient-directed additional risk minimisation measures. The primary objective of this study is to evaluate patients' knowledge levels of the key messages included in the Imlygic Patient Safety Brochure among patients who receive Imlygic.

Action: for adoption

Request for Supplementary Information adopted on 13.05.2022.

The opinion was adopted, in line with the recommendation from PRAC.

2.11.4. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/II/0053

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Quality. Opinion

Action: for adoption

The opinion was adopted.

2.11.5. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0058

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality. Opinion

Action: for adoption

The opinion was adopted.

2.11.6. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0059

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Clinical. Request for supplementary information

Update of section 5.1 of the SmPC based on a subgroup analysis from CCTL019B2401 (B2401) disease registry listed as a PAES (ANX006) in the Annex II; this is a non-interventional study to evaluate the efficacy and safety of Kymriah in acute lymphocytic leukaemia (ALL) patients below the age of 3 years. In addition, the MAH took the opportunity to update Annex II.D of the SmPC to reflect the fulfilment of the PAES.

Action: for adoption

The request for supplementary information was adopted.

2.11.7. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0060

Novartis Europharm Limited

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

Scope: Safety. Request for supplementary information

Update of section 4.2 of the SmPC in order to update the paediatric statement for the B-cell acute lymphocytic leukaemia (ALL) indication and section 4.4 to update the warning on 'prior treatment with anti-CD19 therapy' as well as sections 4.4 and 4.8 in order to update safety data to reflect the pool of the 3 studies B2202, B2205J and B2001X. The proposed changes are in line with the request of the CAT following the assessment of P46/012. The Package Leaflet is updated accordingly. In addition, the MAH took the opportunity to correct the complete response rate (CRR) 95% confidence interval (CI) on enrolled set for E2202 study presented in Table 8 in section 5.1 of the SmPC. The RMP version 5.0 has also been submitted.

Action: for adoption

The request for supplementary information was adopted.

2.11.8. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0061/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: Quality. Request for supplementary information

Action: for adoption

The request for supplementary information was adopted.

2.11.9. 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/II/0033

Orchard Therapeutics (Netherlands) BV

Rapporteur: PRAC Rapporteur: Menno van der Elst

Scope: Safety. Opinion

Submission of the final report from study STRIM-001 "Evaluation of referring healthcare providers' and parents'/carers' understanding of specific risks associated with Strimvelis treatment" listed as a category 3 study in the RMP. The RMP version 6.1 has also been submitted.

Action: for adoption

Request for supplementary information adopted on 18.03.2022.

The opinion was adopted.

2.11.10. Tecartus - brexucabtagene autoleucel - Orphan - EMEA/H/C/005102/II/0008/G

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, PRAC Rapporteur: Menno van der Elst

Scope: Clinical and Safety. Opinion

Group of variations including an extension of indication to include treatment of adult patients with relapsed or refractory (r/r) B-cell acute lymphoblastic leukaemia (B-ALL) for Tecartus and a type IB variation to change the drug product dose specification for the new indication. As a consequence, sections 2.2, 4.1, 4.2, 4.4, 4.8 and 5.1 of the SmPC are updated. The Package Leaflet and Labelling are updated in accordance. Version 1.1 of the RMP has also been submitted. Furthermore, the product information (PI) is brought in line with the latest QRD template.

Action: for adoption

Request for supplementary information adopted on 18.03.2022 and 10.09.2021.

Feedback from the discussion at the Scientific Advisory Group (SAG) Oncology on the extension of indication of Tecartus to ALL in adult patients was provided by the chair of the SAG Oncology.

The Rapporteur presented the outcome of the assessment of the responses to the second list of outstanding issues. The opinion was adopted via a written procedure.

2.11.11. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0046

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, Co-Rapporteur: Claire Beuneu, PRAC Rapporteur: Anette Kirstine Stark

Scope: Clinical. Request for supplementary information

Extension of indication to include treatment of adult patients with relapsed or refractory (r/r) diffuse large B-cell lymphoma (DLBCL) and high-grade B-cell lymphoma (HGBL) for Yescarta; as a consequence, sections 4.1, 4.2, 4.8, 5.1 and 5.2 of the SmPC are updated. The Package Leaflet is updated in accordance. Version 5.3 of the RMP has also been submitted. In addition, the MAH took the opportunity to update the product information with minor editorial changes.

Action: for adoption

Request for supplementary information adopted on 13.05.2022 and 18.02.2022.

The Rapporteur presented the outcome of the assessment of the responses to the second list of outstanding issues. The third request for supplementary information was adopted.

2.11.12. Zolgensma - onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/II/0028/G

Novartis Gene Therapies EU Limited

Rapporteur: Carla Herberts

Scope: Quality. Opinion

Action: for adoption

The opinion was adopted.

2.11.13. Tecartus; Yescarta – axicabtagene ciloleucel; brexucabtagene autoleucel – Orphan – EMEA/H/C/WS2247

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality. Request for supplementary information

Action: for adoption

Request for supplementary information adopted on 13.05.2022.

The request for supplementary information was adopted.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

No items

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

No items

4.2. Day 30 ATMP scientific recommendation

4.2.1. Adeno-associated viral vector serotype 2 encoding glial cell line-derived neurotrophic factor

Intended for the treatment of Parkinson's disease (PD)

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

4.2.2. Ex-vivo expanded allogeneic human corneal epithelial cells containing P63 positively expressing cells

Intended for the treatment of persistent corneal epithelial defects

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

4.2.3. Allogeneic adipose-derived mesenchymal stem cells

Intended for the treatment of arthritis and diabetes type I and II

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

4.2.4. Acellular tubular graft composed of human collagen types I and III and other extracellular matrix proteins, including fibronectin and vitronectin

Intended for replacement or repair of injured blood vessels in cases of vascular trauma; for replacement or repair of diseased vessels as an arterial bypass conduit for peripheral arterial disease (PAD); and as an implanted vascular access conduit for haemodialysis in patients with end-stage renal disease (ESRD)

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

4.2.5. A heterologous vaccine regimen composed of 2 components: replication incompetent gorilla adenovirus serotype 20 (GAd20) and modified vaccinia ankara (MVA) vectors encoding tumor-specific antigens mutant calreticulin (mutCALR) and Janus kinase 2 (mutJAK2)

Intended for the treatment of patients with myeloproliferative neoplasms (MPNs)

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

4.2.6. Recombinant adeno-associated virus vector containing the human aspartoacylase complementary DNA (ASPA cDNA) with an optimized expression cassette and constitutive promoter

Intended for the treatment of Canavan disease

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

4.2.7. Adeno-associated virus serotype hu68 vector encoding human GLB1 gene

Intended for the treatment of GM1 gangliosidosis

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

4.2.8. Autologous human bone marrow derived mesenchymal stromal cells (MSCs)

Intended for the treatment of pathologies affecting the oesophageal tract in which total or partial organ replacement is required

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

4.2.9. Skin cell suspension obtained with the help of recombinant non-animal trypsin

Intended for skin regeneration after burns, skin trauma, invasive surgery

Scope: ATMP scientific recommendation

Action: for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 29 July 2022.

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

No items

4.4. Finalisation of procedure

4.4.1. Wharton's Jelly Derived Mesenchymal Stem Cells – allogeneic

Intended for the treatment of other specified inflammatory spondylopathies (non-radiographic axial spondylarthritis, M46.8)

Scope: The European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

The classification report was adopted. The product does fulfil the definition of an advanced therapy medicinal product as defined in Article 2(1) of Regulation (EC) 1394/2007. CAT considered that the applicant did not provide sufficient information to support the claimed mechanism of action of the product in the indication sought and therefore CAT concluded that the product is an ATMP, but did not decide if it is a tissue engineered product or a

somatic cell therapy medicinal product.

4.4.2. Autologous keratinocytes, fibroblasts

Intended for the treatment of partial deep dermal and full thickness burn wounds and reconstructive surgery

Scope: The European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

The classification report was adopted. The product does fulfil the definition of a tissue engineered product as defined in Article 2(1) of Regulation (EC) 1394/2007.

4.4.3. Dopaminergic neuronal microtissues containing A9 TH+ (tyrosine hydroxylase) dopaminergic mature neuron

Intended for the treatment of Parkinson's disease

Scope: The European Commission raised no comments. ATMP scientific recommendation

Action: for adoption

The classification report was adopted. The product does fulfil the definition of a tissue engineered product as defined in Article 2(1) of Regulation (EC) 1394/2007.

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

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

Timetable:

- Start of procedure at SAWP:	04-07.07.2022
- Appointment of CAT Peer Reviewers:	13-15.07.2022
- SAWP first reports:	22.08.2022
- CAT Peer Reviewer comments (NC,C):	26.08.2022
- CAT Peer reviewer comments (Q):	31.08.2022
- Discussion at SAWP:	29.08-01.09.2022
- Discussion at CAT and feedback to SAWP:	09.09.2022

5.1.2. Scientific advice procedures starting at the next SAWP meeting

Timetable:

- Start of procedure at SAWP:	29.08–01.09.2022
- Appointment of CAT Peer Reviewers:	07–09.09.2022

- SAWP first reports: 19.09.2022
- CAT Peer Reviewer comments (NC,C): 23.09.2022
- CAT Peer reviewer comments (Q): 28.09.2022
- Discussion at SAWP: 26–29.09.2022
- Discussion at CAT and feedback to SAWP: 07.10.2022

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

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

No items

5.4. Final Advice Letters for procedures finalised the previous month

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

No items

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

The chair welcomed the new members and alternates representing the clinicians:

- Alessandro Aiuti (member) and Bernd Gänsbacher (alternate)

- Paolo Gasparini (member) and Alessandra Renieri (alternate)

The chair also welcomed the new members and alternates representing the patient organisations:

- Kieran Breen (member) and Federica Chiara (alternate)

- Kerstin Sollerbrant (member) and Mencia de Lemus Belmonte (alternate)

The chair announced that Ebru Karakoc Madsen took over the role of member for Denmark.

Action: for information

7.1.2. Vote by proxy

No items

7.1.3. CAT's August 2022 written procedure

Scope: August 2022: process and timelines

Action: for information

CAT noted the information and the timelines for the August 2022 written procedure.

7.1.4. CAT Strategic Review & Learning meeting (SRLM) under the Czechia presidency, 17 – 18 November 2022 in Paris

CAT: Petr Soukup, Martina Schuessler-Lenz

Scope: Practical information and proposal for agenda content

Action: for discussion

CAT discussed possible topics for discussion at the upcoming SRLM. The following CAT members will contribute to the development of the agenda: Martina Schüssler-Lenz, Alessandra Renieri and Violaine Closson-Carella.

7.1.5. Publication of CAT regulatory outcomes on the EMA webpage

CAT: Martina Schüssler-Lenz

Scope: Presentation on planned changes to the EMA website and communication of CAT outcomes on the EMA Website.

Action: for information

CAT noted the information and feedback by EMA.

7.2. Coordination with EMA Scientific Committees

7.2.1. PRIME implementation of 5-year review recommendations

Scope: Presentation of the proposals for implementation of the recommendations arising from the first 5 years' experience with the scheme (see also prime-analysis-first-5-years-experience_en.pdf (europa.eu)) as discussed and agreed by the PRIME oversight group.

Action: for adoption

EMA presented the recommendations arising from the 5-year review of the PRIME implementation. CAT discussed the recommendations

It was agreed to bring back the results of the consultation of the different committees and working parties to CAT in September.

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

7.3.1. Diffuse large B-cell lymphoma (DLBCL) indication wording and inclusion of high-grade B-cell lymphoma (HGBL)

Scope: key points from the discussion with haematologists that took place on the 31 May 2022

Action: for information

Filip Josephson and Jan Mueller-Berghaus reported from the informal meeting to collect the personal opinions from experts on the DLBCL/HGBL categorisation and the possible extrapolation of safety and efficacy between large B-cell lymphoid neoplasms (taken into consideration that the treatment options are not guided by the categorisation).

CAT members indicated that the wordings of the SmPCs of the different CAR-Ts should be ensured, mechanism of action and scientific background taken into account, rather than full consistency with non-ATMPs in this indication (e.g. Polivy).

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

Scope: Draft Agenda - PCWP-HCPWP joint meeting on 22 September 2022

Action: for information

The information was noted.

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

Scope: PCWP-HCPWP minutes from the meeting on 1-2 June 2022

Action: for information

The information was noted.

7.3.4. Reflection paper on criteria to be considered for the evaluation of new active substance (NAS) status of biological substances

Rapporteur: Martijn van der Plas

Action: For adoption

Martijn van der Plas (NL) presented the current version of draft Reflection Paper (RP) on NAS status of biologicals. It was noted that the drafting group has agreed, by majority, the wording proposals for remaining sections relating to ATMPs. The RP is presented to BWP, CAT, and subsequently to CHMP, for adoption. Then it will be published for a 6-month public consultation period.

It was noted that the final wording is under parallel review by EMA Regulatory Affairs Office and Legal Departments. In case of any major edits/changes arising from this review the revised RP will be presented to CAT again prior to adoption/publication.

7.4. Cooperation with the EU regulatory network

No items

7.5. Cooperation with international regulators

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

CAT: Martina Schuessler-Lenz

Scope: Feedback from the teleconference that took place on 23 June 2022

Action: for information

A short feedback was provided from the discussions in the latest ATMP cluster teleconference.

7.6. CAT work plan

7.6.1. CAT workplan 2022

Scope: half-year review

Action: for discussion

EMA provided an overview of the progress of the different topics on the CAT workplan for 2022.

7.6.2. ATMP Webinar: ATMP classification and MAA at CAT; Interface to GMO, medical devices and companion diagnostics – 15 July 2022

CAT: Ilona Reischl

Scope: The goal of the module is to provide a general, high-level overview of the ATMP classification and ATMP-marketing authorisation procedures; to provide initial information and serve as a reference for further knowledge building.

Action: for information

The information was noted.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. DARWIN EU Coordination Centre

Scope: Follow up on real world evidence (RWE) and DARWIN EU® and the recently selected data partners and year 1 RWE studies.

Action: for discussion

EMA provided information on the DARWIN EU establishment including feedback on the current and planned data partners that can be used for the conduct of studies based on real world data. The difference between studies performed via DARWIN EU and studies procured under the EMA's Framework contracts was explained.

The following CAT members will act as CAT contacts points for DARWIN EU: Lisbeth Barkholt, Maura O'Donovan, Kieran Breen and Alessandra Renieri.

7.8.2. European Society for Gene and cell therapy (ESGCT) annual meeting

CAT: Martina Schüssler-Lenz

Scope: Proposal of topics for the CAT regulatory session at the ESGCT annual meeting that will take place in Edinburgh on 14 October 2022

Action: for discussion

The draft agenda of the CAT regulatory session at the ESGCT annual meeting was presented. The following CAT members will participate as speakers in this regulatory session: Martina Schüssler-Lenz, Concetta Quintarelli, Barbara Bonamassa, Claire Beuneu, Carla Herberts and Ilona Reischl.

7.8.3. Adeno-associated viral (AAV) vector toxicities: regulatory considerations

CAT: Carla Herberts, Egbert Flory

Scope: Discussion paper insertional mutagenesis and follow-up for AAV gene therapy

Action: for discussion

CAT discussed the duration of follow-up of patients treated with AAV gene therapies. The outcome will be presented at the ATMP cluster TC and at the CAT session at ESGCT (see 7.8.2).

7.8.4. CAT Learnings on blood/tissue establishment providing starting materials for ATMPs

CAT: Barbara Bonamassa

BWP: Marja van de Bovenkamp

Scope: Blood/tissue establishment in 3rd countries providing starting materials for ATMPs

Action: for adoption

Adoption postponed to September CAT meeting

7.8.5. CAT Learnings on SmPC section 2.1 for the AAV products

CAT: Niamh Curran

Scope: Harmonisation of section 2.1 of the SmPC for adeno-associated viral vector (AAV) based products

Action: for adoption

The CAT learning was adopted and will be included in the CAT learnings' table.

7.8.6. CAT-industry stakeholder meeting

CAT: Martina Schüssler-Lenz

Scope: Organisation of the next CAT stakeholder meeting at the end of 2022/beginning of 2023.

Action: for discussion

CAT agreed to start with the organisation of the next CAT stakeholder meeting.

The previous CAT stakeholder meeting was held on 26 October 2021.
https://www.ema.europa.eu/en/events/committee-advanced-therapies-cat-meeting-interested-parties-0

7.8.7. Novel Therapies and Technologies Working Party

CAT: Daiusz Sladowski

Scope: feedback on the development of guidelines in the (veterinary) Novel Therapies and Technologies Working Party

Action: for information

CAT noted the feedback on the activities of the Novel Therapies and Technologies Working Party and the guidelines currently under development.

7.8.8. Regulatory & scientific conference on RNA-based medicines

Scope: Background, objectives and content of the RNA conference

Action: for information

CAT noted the information of the conference on RNA-based medicines that is planned to be organised in November 2022.

CAT members interested to participate to the development of the agenda and to participate (as panellists) to the conference should inform EMA secretariat.

8. Any other business

No items

Date of next CAT meeting:

07-09/09/2022

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

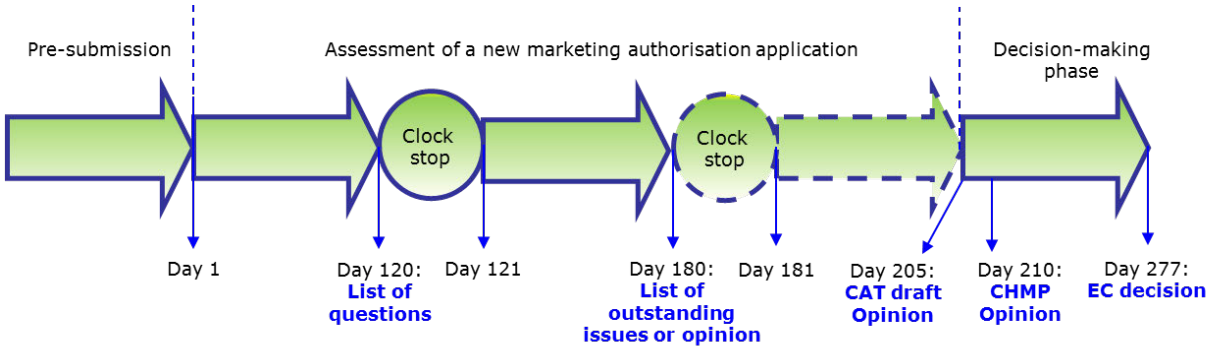
Evaluation of ATMPs (section 2)

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/

10. List of participants

Including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 13-15 July 2022 meeting.

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Martina Schüssler-Lenz	Chair	Germany	No interests declared	
Ilona Reischl	Member (Vice-Chair)	Austria	No interests declared	
Silke Dorner	Alternate	Austria	No interests declared	
Claire Beuneu	Member	Belgium	No interests declared	
Belaïd Sekkali	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Evelina Shumkova	Alternate	Bulgaria	No interests declared	
Azra Selimovic	Member	Croatia	No interests declared	
Petra Sokol	Alternate	Croatia	No interests declared	
Rafaella Pontou	Member	Cyprus	No interests declared	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	
Petr Soukup	Member	Czechia	No interests declared	
Kristyna Rehorova Hradilkova	Alternate	Czechia	No interests declared	
Ebru Karakoc Madsen	Member	Denmark	No restrictions applicable to this meeting	
Pille Saalik	Alternate	Estonia	No interests declared	
Heli Suila	Member	Finland	No interests declared	
Maija Tarkkanen	Alternate	Finland	No interests declared	
Violaine Closson	Member	France	No interests declared	
Jean-Michel Race	Alternate	France	No interests declared	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Maria Gazouli	Member	Greece	No interests declared	
Angeliki Rompoti	Alternate	Greece	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Katalin Lengyel	Member	Hungary	No interests declared	
Balázs Sarkadi	Alternate	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	
Niamh Curran	Alternate	Ireland	No restrictions applicable to this meeting	
Concetta Quintarelli	Member	Italy	No interests declared	
Barbara Bonamassa	Alternate	Italy	No restrictions applicable to this meeting	
Una Riekstina	Member	Latvia	No interests declared	
Romaldas Mačiulaitis	Member (CHMP member)	Lithuania	No interests declared	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No interests declared	
Vlasta Zavadova	Member	Liechtenstein	No interests declared	
Guy Berchem	Alternate	Luxembourg	No restrictions applicable to this meeting	
Nancy De Bremaeker	Member	Luxembourg	No interests declared	
John J. Borg	Member (CHMP member)	Malta	No interests declared	
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	
Carla Herberts	Member	Netherlands	No interests declared	
Babs Fabriek	Alternate	Netherlands	No interests declared	
Rune Kjeken	Member	Norway	No restrictions applicable to this meeting	
Dariusz Śladowski	Member	Poland	No restrictions applicable to this meeting	
Marcin Kolakowski	Alternate	Poland	No interests declared	
Bruno Sepodes	Member (CHMP member)	Portugal	No interests declared	
Maria Isabel Borba Vieira	Alternate (to CHMP representative)	Portugal	No interests declared	
Silviu Istrate	Member	Romania	No interests declared	
Alexandrina Preda	Alternate	Romania	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Lukas Slovak	Member	Slovakia	No interests declared	
Katarina Vavrová	Alternate	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	
Suzana Vidic	Alternate	Slovenia	No participation in final deliberations and voting on:	2.11.5., 2.11.6., 2.11.7., 2.11.8. & 2.11.12.
Sol Ruiz	Member (CHMP co-opted member)	Spain	No interests declared	
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	
Lisbeth Barkholt	Member	Sweden	No interests declared	
Maria Luttgen	Alternate	Sweden	No restrictions applicable to this meeting	
Bernd Gänsbacher	Member	Clinicians' Representative	No interests declared	
Vacant	Alternate	Clinicians' Representative		
Alessandro Aiuti	Member	Clinicians' Representative	No participation in discussions, final deliberations and voting on:	2.11.9. & 2.13.1.
Alessandra Renieri	Alternate	Clinicians' Representative	No restrictions applicable to this meeting	
Kerstin Sollerbrant	Member	Patients' Representative	No interests declared	
vacant	Alternate	Patients' Representative		
Kieran Breen	Member	Patients' Representative	No interests declared	
Roland Pochet	Alternate	Patients' Representative	No interests declared	
Catherine Milne	Observer/Alternate	EDQM	No interests declared	
Declan Noone	Expert	European Haemophilia Consortium	No interests declared	
Marja van de Bovenkamp	Expert	CBG-MEB (NL)	No interests declared	
Johannes Ovelgonne	Expert	SAWP (NL)	No interests declared	
Filip Josephson	Expert	CHMP (ES)	No interests declared	
Esther Rincón	Expert	AEMPS (ES)	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Susana Rojo	Expert	AEMPS (ES)	No interests declared	
Raquel Martin	Expert	AEMPS (ES)	No interests declared	
Pablo de Filipe	Expert	AEMPS (ES)	No interests declared	
Johanna Lähteenvuo	Expert	FIMEA (FI)	No interests declared	
Hanna Kankkonen	Expert	FIMEA (FI)	No interests declared	
Pauliina Lehtolainen-Dalkilic	Expert	FIMEA (FI)	No interests declared	
Karri Penttilä	Expert	FIMEA (FI)	No interests declared	
Elina Asikanius	Expert	FIMEA (FI)	No interests declared	
Jurgen Scherer	Expert	PEI (DE)	No interests declared	
Beate Mosl	Expert	PEI (DE)	No interests declared	
Paolo Gasparini	Member	Clinicians' Representative	No interests declared	
Mencia De Lemus Belmonte	Alternate	Patients' Representative	No restrictions applicable to this meeting	
Federica Chiara	Alternate	Patients' Representative	No interests declared	
Juliane Rau	Expert	PEI (DE)	No interests declared	
Attila Sebe	Expert	PEI (DE)	No interests declared	
Andrea Laslop	Expert - via Webex	AGES AT	No interests declared	
Brigitte Mueller	Expert - via Webex	AGES (AT)	No interests declared	
Harald Bernsteiner	Expert	AGES (AT)	No interests declared	
Melanie Ramberger	Expert	AGES (AT)	No interests declared	
Christine Vaculik	Expert - via Webex	AGES AT	No interests declared	
Jakob Paur	Expert	AGES (AT)	No interests declared	
Manfred Schuster	Expert	AGES (AT)	No restrictions applicable to this meeting	
Tjerk Feenstra	Expert	AGES AT	No interests declared	
Florian Stampfer	Expert	AGES (AT)	No interests declared	
Lothar Bergmann	Expert	SAG (DE)	No restrictions applicable to this meeting	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
A representative from the European Commission attended the meeting				
Meeting run with support from relevant EMA staff				