Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 17-19 July 2024

Chair: Ilona Reischl; Vice-Chair: Kieran Breen

17 July 2024, 14:00 – 18:30, Webex
18 July 2024, 09:00 – 18:30, Webex
19 July 2024, 09:00 – 13:00, Webex

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 ongoing 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 17-19 July 2024. See July 2024 CAT minutes (to be published post September 2024 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 17-19 July 2024 meeting

1.3. Adoption of the minutes

CAT minutes for 19-22 June 2024 meeting

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

2.4.1. Obecabtagene autoleucel - PRIME - Orphan - EMEA/H/C/005907

Autolus GmbH; Treatment of patients with relapsed or refractory B cell precursor acute lymphoblastic leukaemia (ALL)

Scope: Day 120 list of questions

Action: for adoption
2.4.2. Mozafancogene autotemcel - PRIME - Orphan - EMEA/H/C/005537

Rocket Pharmaceuticals B.V.; Treatment of paediatric patients with Fanconi anaemia type A
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

No items

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. Companion diagnostics

2.10.1. Initial consultation

No items

2.10.2. Follow-up consultation

No items

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

2.11.1. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0047

Bristol-Myers Squibb Pharma EEIG
Rapporteur: Rune Kjeken
Scope: Quality, request for supplementary information

**Action:** for adoption

Request for supplementary information adopted on 24.05.2024.

### 2.11.2. Abecma - Idecabtagene vicleucel - Orphan - EMEA/H/C/004662/II/0048

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: Quality, request for supplementary information

**Action:** for adoption

Request for supplementary information adopted on 21.06.2024.

### 2.11.3. Alofisel - Darvadstrocel - Orphan - EMEA/H/C/004258/II/0051/G

Takeda Pharma A/S

Rapporteur: Maria Luttgen, PRAC Rapporteur: Gabriele Maurer

Scope: Efficacy, request for supplementary information

A grouped application comprised of 4 Type II Variations, as follows:

(C.I.4): Update of sections 4.8 and 5.1 of the SmPC in order to update the safety information, based on pooled safety data from the two phase 3 controlled studies (ADMIRE-CD & ADMIRE-CD II) and to update efficacy information based on final results from study ADMIRE-CD II, listed as an obligation in the Annex II. ADMIRE-CD II (Cx601-0303) is a Phase III randomised double blind, placebo controlled study to assess efficacy and safety of Cx601, adult allogeneic expanded adipose-derived stem cells (eASC) for the treatment of complex perianal fistula(s) in patients with Crohn’s disease. The Annex II is updated accordingly. In addition, the MAH took the opportunity to introduce minor changes to the PI, including to section 4.2 of the SmPC and to the Package Leaflet.

3 x (C.I.13): Submission of interim results from studies Darvadstrocel-3003 and Alofisel-5003 (INSPIRE) and final results from study Darvadstrocel-3002 to support the benefit-risk assessment of darvadstrocel based on all new available clinical data.

The RMP version 8.0 has also been submitted.

**Action:** for adoption

### 2.11.4. Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/II/0037/G

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli

Scope: Quality

**Action:** for adoption
Request for supplementary information adopted on 21.06.2024, 15.03.2024.

2.11.5. Hemgenix - Etranacogene dezaparvovec - Orphan - EMEA/H/C/004827/II/0014/G

CSL Behring GmbH

Rapporteur: Silke Dorner

Scope: Quality, request for supplementary information

**Action:** for adoption

2.11.6. Imlygic - Talimogene laherparepvec - EMEA/H/C/002771/II/0067/G

Amgen Europe B.V.

Rapporteur: Maija Tarkkanen

Scope: Clinical

A grouped application as follows:

Type II (C.1.4): Update of sections 4.2, 4.8 and 5.1 of the SmPC in order to update paediatric information based on the paediatric study 20110261, which was previously submitted in procedure II/0063. This is a phase 1, multicentre, open-label study of talimogene laherparepvec in paediatric subjects with advanced non-CNS tumours that were amenable to direct injection in the clinical setting. The Package Leaflet is updated accordingly. In addition, the MAH took the opportunity to introduce minor editorial and formatting changes to the Product Information.

Type IA (A.6): To change the ATC Code of Antineoplastic cell and gene therapy from L01XX51 to L01XL02.

**Action:** for adoption

2.11.7. Upstaza - Eladocagene exuparvovec - Orphan - EMEA/H/C/005352/II/0022/G

PTC Therapeutics International Limited

Rapporteur: Joseph DeCourcey

Scope: Quality, opinion

**Action:** for adoption

2.11.8. Yescarta - Axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0077

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: Quality, request for supplementary information

**Action:** for adoption
2.12. **Extension applications**

No items

2.13. **Other Post-Authorisation Activities**

2.13.1. **Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/MEA/007.1**

Bristol-Myers Squibb Pharma EEIG  
**Rapporteur:** Concetta Quintarelli  
**Scope:** Pharmacovigilance  

MAH’s response to MEA 007 [LTFU study (GC LTFU 001)] RSI as adopted in January 2024. Long-term follow-up of safety and efficacy for all paediatric and adult subjects exposed do a GM T cell therapy in Bristol-Myers Squibb sponsored, or Bristol Myers Squibb alliance partner sponsored, clinical trials in accordance with Health Authorities’ guidance for long-term (up to 15 years) follow-up of subjects treated with gene therapy products.  

**Action:** for adoption

2.13.2. **Breyanzi - Lisocabtagene maraleucel / Lisocabtagene maraleucel - EMEA/H/C/004731/REC/016.1**

Bristol-Myers Squibb Pharma EEIG  
**Rapporteur:** Concetta Quintarelli  
**Scope:** Quality  

**Action:** for adoption

2.13.3. **Zolgensma - Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750/LEG/024**

Novartis Europharm Limited  
**Rapporteur:** Emmely de Vries  
**Scope:** Non-clinical  

From PSUSA-00010848-202305  

Study reports for 2170026 & 222018:  
- Study no. 2170026 (OAV101: Single Dose Intravenous or Intracerebroventricular Germline Transduction and Integration Study in Neonatal FVB/NCrl Mice with up to a 24-Week Evaluation Period).  
- Study no. 2220183: AAV Vector expression in cynomolgus macaque gonadal tissue of scAAV9-CB-GFP and scAAV9-CB-mCherry 28 days after administration Molecular Localization Report.  

**Action:** for adoption
2.14. **GMP and GCP inspections requests**

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**

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. **Autologous CD34 positive cells**

For regeneration purposes, to replace damaged tissue in blood and other tissues (adipogenic, osteogenic, chondrogenic, myogenic and angiogenic)

Scope: ATMP scientific recommendation

**Action:** for adoption

4.2.2. **Autologous antigenic tumor fragments isolated from patient’s circulating cancer cells**

For treatment of cancer patients suffering from blood cancer

Scope: ATMP scientific recommendation

**Action:** for adoption
4.2.3.  **Autologous antigenic tumor fragments isolated from patient’s circulating cancer cells**

For treatment of cancer patients suffering from cancer of epithelial origin

Scope: ATMP scientific recommendation

**Action:** for adoption

4.2.4.  **Autologous antigenic tumor fragments isolated from patient’s circulating cancer cells**

For treatment of cancer patients suffering from melanoma

Scope: ATMP scientific recommendation

**Action:** for adoption

4.2.5.  **Autologous antigenic tumor fragments isolated from patient’s circulating cancer cells**

For treatment of cancer patients suffering from sarcoma

Scope: ATMP scientific recommendation

**Action:** for adoption

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

4.3.1.  **Implantable device 3D bioprinted with autologous microfat and hydrogel bioink**

For treatment of breast reconstruction, soft tissue repair

Scope: ATMP scientific recommendation

**Action:** for adoption

4.4.  **Finalisation of procedure**

4.4.1.  **Autologous adipose-derived stromal vascular fraction cells**

For chronic pain relief

Scope: European Commission raised comments. ATMP scientific recommendation

**Action:** for adoption

4.4.2.  **Double stranded DNA targeting patient specific tumour neo-antigens**

For treatment of non small cell lung cancer
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4.4.9. Synthetic double-stranded RNA oligonucleotide conjugated to GalNAc aminosugar residues

For treatment of primary hyperoxaluria

Scope: European Commission raised no comments. ATMP scientific recommendation

**Action:** for adoption

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: 08-11.07.2024
- Appointment of CAT Peer Reviewers: 17-19.07.2024
- SAWP first reports: 26.06.2024
- CAT Peer Reviewer comments (NC/C): 30.08.2024
- CAT Peer Reviewer comments (Q): 04.09.2024
- Discussion at SAWP: 02-05.09.2024
- Discussion at CAT and feedback to SAWP: 11-13.09.2024

5.1.2. Scientific advice procedures starting at the next SAWP meeting

**Timetable:**
- Start of procedure at SAWP: dd.mm.202y
- Appointment of CAT Peer Reviewers: dd.mm.202y
- SAWP first reports: dd.mm.202y
- CAT Peer Reviewer comments: dd.mm.202y
- Discussion at SAWP: dd.mm.202y
- Discussion at CAT and feedback to SAWP: dd.mm.202y

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

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: 08-11.07.2024
SAWP recommendation: 05.09.2024
CAT recommendation: 13.09.2024
CHMP adoption of report and final recommendation: 19.09.2024

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

No items

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

Action: for information

7.1.2. Vote by proxy

Action: for information

7.1.3. Update on CAT Timeschedule

Action: for information

7.1.4. Strategic Review and Learning meeting under the Hungarian Presidency of the European Union

CAT: Viola Bardoczy
Scope: Draft agenda
Action: for discussion
Note: Date for the SRLM under the Hungarian Presidency: 18-20 November 2024 (changed from date announced in the June CAT meeting)

7.2. Coordination with EMA Scientific Committees

No items

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

7.3.1. GVP Module XVI on risk minimisation measures and its Addendum II

Action: for information (presentation following written consultation of CAT concluded on 4 July 2024)

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

Scope: Status update from drafting group
Action: for information

Following CAT members/experts are contributing to the drafting group discussions on the NAS status for ATMPs: M Timon, B Bonamassa, H Suila, I Reichl, J Scherer, M van de

Committee for Advanced Therapies (CAT)
EMA/CAT/328264/2024
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: Ilona Reischl

Scope: Feedback from the teleconference of 27.06.2024

**Action:** for information

7.6. **CAT work plan**

7.6.1. **CAT work plan 2025**

CAT: Ilona Reischl, Claire Beuneu, Rune Kjeken, Jan Mueller-Berghaus, Mencia de Lemus Belmonte, Maria Lüttgen, Olga Kholmanskikh, Dariusz Sladowski

Scope: Tour de table on progress of 2024 CAT workplan activities and discuss timelines and next steps for 2025 CAT workplan

**Action:** for information

7.6.2. **Guideline on quality, non-clinical and clinical requirements for investigational ATMPs in clinical trials**

CAT: Ilona Reischl

Scope: Status update: feedback from the quality and non-clinical drafting groups

**Action:** for information

7.7. **Planning and reporting**

No items

7.8. **Others**

No items
8. Any other business

8.1. Update to CxMPs on IRIS for Regulatory Procedures

Scope: To update the impacted committees on further procedures to be implemented in IRIS by the end of 2024 (PSURs, PAMs, Line extensions, Renewals, Annual Reassessments, PASS, Referrals)

Action: for information

Date of next CAT meeting:

13-16 August 2024 (written procedure)
9. Explanatory notes

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

For a list of acronyms and abbreviations, see:
List of abbreviations used in EMA human medicines scientific committees and CMDh documents, and in relation to EMA’s regulatory activities

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 Post-authorisation activities (section 2.11-2.13) and any ATMP related inspection requests (section 2.14).

New applications (sections 2.1. to 2.9.)

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 2.4) 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 2.3)). Section 2.6 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.

*New applications (section 2.7.)*

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.

*Withdrawal of applications (section 2.8.)*

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.

*Re-examination procedures (new applications) under article 9(2) of regulation no 726/2004 (section 2.9.)*

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.

*Companion diagnostics (section 2.10)*

This section lists applications for initial and follow-on consultation of companion diagnostics.

*Post-authorisation activities (section 2.11-2.13.)*

Section 2.11 lists type II variations, including extension of indication applications and re-examination procedures for type II variations for which the applicant has requested re-examination of the opinion previously issued by the CHMP. Section 2.12 list extension application according to Annex I of Reg. 1234/2008 and section 2.13 includes all other post-authorisation activities concerning authorised ATMPs that are not covered elsewhere in the agenda such as post-authorisation measures, 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.

*GMP and GCP Inspections Issues (section 2.14.)*

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).
**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/