9 September 2026
EMA/CAT/203216/2026
Human Medicines Division

# Committee for Advanced Therapies (CAT)

Draft agenda for the meeting on 09-11 September 2026

Chair: Ilona Reischl-Kok; Vice-Chair: Kieran Breen

09 September 2026, 14:00 – 18:30

10 September 2026, 09:00 – 18:30

11 September 2026, 09:00 – 13:00

**Health and safety information**

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

**Disclaimers**

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the CAT meeting reports once the procedures are finalised.

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

**Note on access to documents**

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

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# 1. Introduction

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

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the CAT plenary session to be held 09-11 September 2026. See September 2029 CAT minutes (to be published post October 2026 CAT meeting).

## 1.2. Adoption of agenda

CAT agenda for 09-11 September 2026 meeting

## 1.3. Adoption of the minutes

CAT minutes for 15-17 July 2026 meeting

CAT minutes for 10-14 August 2026 meeting – written procedure

# 2. Evaluation of ATMPs

## 2.1. Opinions

No items

## 2.2. Oral explanations

No items

## 2.3. Day 180 list of outstanding issues

### 2.3.1. Zopapogene imadenovec – Orphan - EMEA/H/C/006508

FGK Representative Service GmbH; Treatment of respiratory papillomatosis in adults

Scope: Day 180 list of questions

**Action:** for adoption

## 2.4. Day 120 list of questions

### 2.4.1. Lunsotogene parvec - Orphan - EMEA/H/C/006802

***Accelerated assessment***

Regeneron Ireland Designated Activity Company; Treatment of biallelic OTOF variant-associated hearing loss in adults and children

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. 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. Aucatzyl - Obecabtagene autoleucel - EMA/VR/0000358721

Autolus GmbH

Rapporteur: Berendina Maria van den Hoorn

Scope: Quality, opinion

**Action:** for adoption

### 2.11.2. Casgevy - Exagamglogene autotemcel - Orphan - EMA/VR/0000356719

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Attila Sebe, PRAC Rapporteur: Bianca Mulder

Scope: Clinical, request for supplementary information

Update of sections 4.8 and 5.1 of the SmPC in order to update clinical safety and efficacy information based on final results from study CTX001-111 (in subjects with transfusion-dependent β-thalassemia (TDT)) and study CTX001-121 (in subjects with severe sickle cell disease (SCD)) as well as interim results from study CTX001-131 (long-term follow-up in subjects with either TDT or severe SCD), listed as specific obligations in the Annex II. The Package Leaflet is updated accordingly. The RMP version 2.0 has also been submitted. In addition, the MAH took the opportunity to introduce additional changes to the PI.

**Action:** for adoption

### 2.11.3. Libmeldy - Atidarsagene autotemcel - Orphan - EMA/VR/0000334917

Orchard Therapeutics (Netherlands) B.V.

Rapporteur: Emmely de Vries, PRAC Rapporteur: Dirk Mentzer

Scope: Clinical, request for supplementary information

A grouped application consisting of:

C.12: Submission of the final report from study 201222 listed as a category 3 study in the RMP. This is a Phase I/II clinical trial of haematopoietic stem cell gene therapy for the treatment of metachromatic leukodystrophy (MLD).

C.12: Submission of the final report from study CUP 207394 listed as a category 3 study in the RMP. This is a gene therapy protocol using autologous haematopoietic stem cells for a patient with metachromatic leukodystrophy (MLD).

C.12: Submission of the final report from studies CUP 206258 and HE 205029 listed as category 3 studies in the RMP. These are Expanded Access Programs (EAP) for hematopoietic stem cell gene therapy OTL-200 in subjects with early-onset metachromatic leukodystrophy (MLD).

The RMP version 4.1 has also been submitted.

**Action:** for adoption

### 2.11.4. Luxturna - Voretigene neparvovec – Orphan - EMA/VR/0000357435

Novartis Europharm Limited

Rapporteur: Sol Ruiz, PRAC Rapporteur: Dirk Mentzer

Scope: Clinical, opinion

Submission of the final report from study SPKRPE-PASS, following the request from PRAC in

PSUSA/00010742/202507. This is a post-authorisation, multicenter, longitudinal, observational safety registry study for patients treated with voretigene neparvovec in US.

**Action:** for adoption

## 2.12. Extension applications

No items

## 2.13. Other Post-Authorisation Activities

### 2.13.1. Tecartus - Brexucabtagene autoleucel - EMA/R/0000355892

Kite Pharma EU B.V.

Rapporteur: Attila Sebe, PRAC Rapporteur: Bianca Mulder

Scope: Renewal - 1 year, opinion

**Action:** for adoption

### 2.13.2. Breyanzi - Lisocabtagene maraleucel - EMA/R/0000355073

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Concetta Quintarelli, Co-Rapporteur: Claire Beuneu, PRAC Rapporteur: Dirk Mentzer

Scope: Renewal - 5-year, request for supplementary information

**Action:** for adoption

### 2.13.3. Abecma - Idecabtagene vicleucel - EMA/PAM/0000360921

Bristol-Myers Squibb Pharma EEIG

Rapporteur: Rune Kjeken

Scope: PAM, opinion

**Action:** for adoption

### 2.13.4. Aucatzyl - Obecabtagene autoleucel - EMA/PAM/0000359117

Autolus GmbH

Rapporteur: Berendina Maria van den Hoorn

Scope: PAM, request for supplementary information

**Action:** for adoption

### 2.13.5. Casgevy - Exagamglogene autotemcel - EMA/PAM/0000266941

Vertex Pharmaceuticals (Ireland) Limited

Rapporteur: Attila Sebe

Scope: PAM, request for supplementary information

**Action:** for adoption

### 2.13.6. Yescarta - Axicabtagene ciloleucel - EMA/PAM/0000361254

Kite Pharma EU B.V.

Rapporteur: Attila Sebe

Scope: PAM, request for supplementary information

**Action:** for adoption

### 2.13.7. Zemcelpro - Dorocubicel / Allogeneic umbilical cord-derived CD34- cells, non-expanded - EMA/PAM/0000313184

Cordex Biologics International Limited

Rapporteur: Emmely de Vries

Scope: PAM, opinion

**Action:** for adoption

## 2.14. Companion diagnostics - initial consultation

No items

## 2.15. Companion diagnostics – Follow-up consultation

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

### 4.1.1. Primary human pancreatic islets of Langerhans loaded in a polyvinylidene fluoride (PVDF) membrane-based macroencapsulation device

Treatment of Type 1 diabetes mellitus with recurrent severe hypoglycaemia and/or impaired awareness of hypoglycaemia; Preservation of endogenous insulin secretion following total pancreatectomy

Scope: ATMP scientific recommendation

**Action:** for nomination of CAT coordinator

### 4.1.2. 3D bio-printed bionic pancreas, composed of islets of Langerhans, a non-viable, printable porcine-derived hydrogel matrix, plus vascular prostheses and stents

Treatment of chronic pancreatitis; Brittle diabetes mellitus type 1

Scope: ATMP scientific recommendation

**Action:** for nomination of CAT coordinator

## 4.2. Day 30 ATMP scientific recommendation

### 4.2.1. Autologous CAR-T lymphocytes anti-CEACAM6

Treatment of pancreatic ductal adenocarcinoma (PDAC) PNM I/II

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.2. Recombinant adeno-associated viral vector containing codon-optimised expression cassette to drive the expression of the SQ form of a B-domain deleted human coagulation APC-resistant factor VIII

Treatment of adults with severe or moderate haemophilia A (congenital FVIII deficiency) without anti-AAV-LK03 antibodies

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.3. Adeno-associated virus 8 (AAV8)-based gene therapy vector expressing B domain-deleted human factor VIII (hFVIII)

Treatment of haemophilia A (HA)

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.4. Autologous, colorectal, tumour infiltrating, tumour-reactive lymphocytes T (TILs)

Treatment of colorectal cancer with a single metastatic lesion

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.5. Single-stranded, non-replicating, recombinant adeno-associated virus serotype 9 (AAV9) encoding a micro-dystrophin gene

Treatment of Duchenne muscular dystrophy

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.6. Micronized autologous adipose tissue

Adjunctive local treatment of refractory gastrointestinal fistulas to support tissue repair and fistula closure

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.7. Extracellular vesicles derived from human induced pluripotent stem cells

Intended for the treatment of organ fibrosis

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.8. Human trophoblast stem cell-derived extracellular vesicles suspended in cross-linked hyaluronic acid hydrogel carrier

Intended for the regeneration and repair of periodontal and peri-implant oral tissues; suppression of senescence-associated secretory phenotype (SASP) in aged and damaged oral tissue cells; proposed use in periodontitis, peri-implantitis, post-extraction wound healing, and oral soft tissue regeneration

Scope: ATMP scientific recommendation

**Action:** for adoption

### 4.2.9. Human trophoblast stem cell-derived extracellular vesicles impregnated into a resorbable surgical matrix scaffold

Intended for the regeneration of periodontal, alveolar bone and peri-implant tissues following oral surgery, implantology, bone grafting and surgical extraction; suppression of post-surgical senescence-associated inflammation and promotion of tissue repair at surgical sites

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

No items

## 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

Timetable:

| | |
|---|---|
| - Start of procedure at SAWP: | 28.09-01.10.2026 |
| - Appointment of CAT Peer Reviewers: | 07-09.10.2026 |
| - SAWP first reports: | 19.10.2026 |
| - CAT Peer Reviewer (NC & C) comments: | 23.10.2026 |
| - CAT Peer Reviewer (Q) comments: | 28.10.2026 |
| - Discussion at SAWP: | 26-29.10.2026 |
| - Discussion at CAT and feedback to SAWP: | 04-06.11.2026 |

## 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

# 6. Pre-Authorisation Activities

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

## 6.1. Paediatric investigation plans

No items

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

No items

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

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

| Timetable for assessment: | |
|---|---|
| Procedure start: | 31.08-03.09.2026 |
| SAWP recommendation: | 01.10.2026 |
| CAT recommendation: | 09.10.2026 |
| CHMP adoption of report and final recommendation: | 15.10.2026 |

### 6.3.2. Month 1 – Discussion of eligibility

### 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

**Action:** for information

### 7.1.2. Nominated proxy

Action: for information

### 7.1.3. CAT Strategic Review & Learning meeting (SRLM) under the Irish presidency

Scope: Preparation for the meeting

CAT: Richard Carroll

**Action**: for information

## 7.2. Coordination with EMA Scientific Committees

### 7.2.1. Data protection notice for committees, Working Parties (WPs), Scientific Advisory Groups (SAGs) and Ad-Hoc Expert Groups (AHEGs)

Scope: Confirmation that the Data Protection Notice (DPN) for CXMP has been updated and published on the EMA website.

**Action**: for information

### 7.2.2. CAR-T passport

Scope: Feedback from an informal meeting between GoCART consortium and EMA

**Action**: for discussion

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

No items

## 7.4. Cooperation with the EU regulatory network

### 7.4.1. GMO proposal in Biotech Act

Scope: Presentation by the European Commission

European Commission Representative

**Action**: for information

## 7.5. Cooperation with international regulators

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

Scope: Agenda of the ATMP cluster of 24.09.2026

CAT: Ilona Reischl-Kok

**Action:** for information

## 7.6. CAT work plan

No items

## 7.7. Planning and reporting

No items

## 7.8. Others

### 7.8.1. DrugRadar

Scope: App tracking latest EMA and FDA approvals and late-stage clinical trials

Developer: Bernd Gansbacher

**Action**: for information

# 8. Any other business

No items

Date of next CAT meeting:

07-09 October 2026

# 9. Explanatory notes

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

**Abbreviations in Committee CMD documents and in relation to EMA regulatory activities**

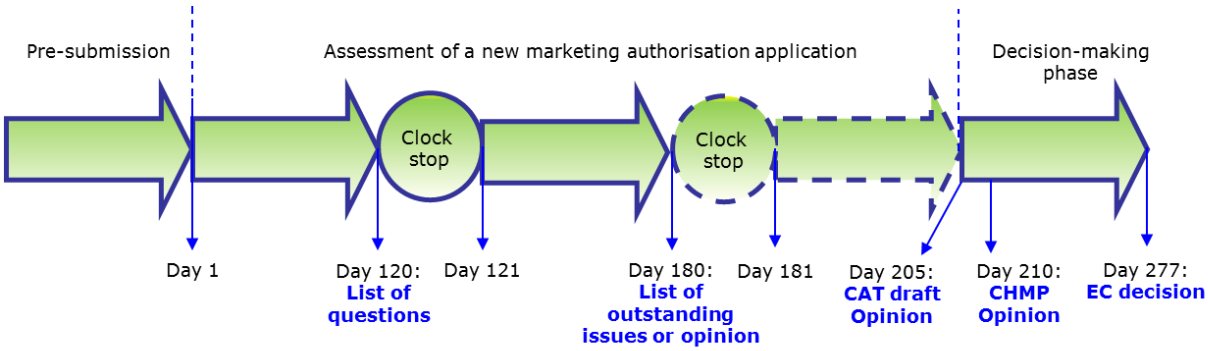
## Evaluation of ATMPs (section 2)

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.

### *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 2.11-2.13.)*

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.

### *Companion diagnostics (section 2.14. & 2.15.)*

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

## 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/