EMA
EUROPEAN MEDICINES AGENCY

CAT workplan 2026

Adopted by the committee on 23 January 2026

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Human Medicines Division

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Introduction by the CAT Chair

Ilona Reischl-Kok

For 2026, CAT will focus on the provision of scientific and regulatory guidance and training. In horizontal activities CAT will contribute the ATMP-specific perspective, supporting other Committees and decision makers.

While specific topics are singled out in the workplan, important other activities will continue, such as communication within the network, exchange with international regulators, identifying regulatory challenges for ATMPs and initiating potential paths to solutions, monitoring new legislations for their impact on the ATMP field, participating in scientific conferences to spread regulatory knowledge and interaction with stakeholders in general.

The CAT will continue its responsibilities in supporting the development and authorisation of ATMPs, and will work to embedding ATMP expertise in the future regulatory framework.

The activities outlined in this workplan have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme.

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Workplan structure

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Evaluation activities for human medicines:

- Pre-authorisation activities
- Initial evaluation activities
- Post-Authorisation activities

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Evaluation activities for human medicines

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Evaluation activities for human medicines

Pre-authorisation activities

Guideline on requirements for investigational ATMPs in clinical trials

This guideline provides guidance to ATMP developers to understand the requirements for ATMPs during clinical development. The aim is to facilitate the development of ATMPs and the preparation of EU clinical trial applications.

Key objectives:

Following the finalisation of the guideline, training of assessors and stakeholders will be organised.

Activities in 2026:
Organise a training for assessors of ATMP clinical trials
Organise a webinar for the ATMP developers

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

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Pre-authorisation activities

Revision of the Questions and Answers on Gene Therapy

A questions and answers document on matters related to the development of gene therapy medicinal products (EMA/CHMP/GTWP/212377/2008) was published in 2010. This document is in need of revision, to remove topics that are either incorporated in scientific guideline or are no longer relevant, and where needed, to update the responses to reflect the current regulatory position. Additional questions might also be identified.

Key objectives:

Revision of the questions and answers on gene therapies to reflect the current regulatory position. The scope of this documents will be expanded to include all classes of ATMPs

Activities in 2026:
Update existing questions and answers
Include new questions and answers (relevant for all ATMPs)

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

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Pre-authorisation activities

Development of guidance on gene editing

Gene editing is becoming an established technology in ATMP development. Clinical trials with *in vivo* and *in vitro* gene editing products are taking place and the first product based on cells genetically modified by means of *in vitro* gene editing has been authorised. Guidance needs to be developed or updated to help the developers submitting marketing authorisation applications for these innovative products

Key objectives:

To reflect the scientific and regulatory requirements for *in vitro* and *in vivo* gene editing

Activities in 2026:
Develop a reflection paper on *in vivo* gene editing
Prepare a concept paper for the revision of the Guideline for genetically modified cells to update the guidance for *in vitro* gene edited cells.

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

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Evaluation activities for human medicines

Pre-authorisation activities

Scientific consultations involving other decision makers to facilitate optimization of clinical evidence generation in drug development programmes

Clinical evidence generated during drug development is intended to serve different decision making. It is therefore desirable that evidence requirements do address regulatory needs as well as those of other down-stream decision makers.

Key objectives:

- To engage with other decision makers in multi-stakeholder consultations on evidence generation planning.
- To prospectively identify post-licensing evidence needs considering the expected evidence available at time of initial decision making by regulators and HTAs, respectively.

Activities in 2026:	CHMP
Collaborate with the Member State Coordination Group on HTA (HTACG) on evidence requirements and management of uncertainties for different types of developments, to inform prospective evidence planning for development programmes	
Engage with the HTACG on opportunities for collaboration on scientific and methodological guidelines	
Explore with healthcare payers' opportunities for sharing views on prospective evidence planning, focusing on post-licensing evidence needs	

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

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Initial evaluation activities

Real World Data (RWD) in regulatory decision making of ATMPs

RWD are used in the development, authorisation, safety and efficacy follow-up and monitoring of ATMPs. Enhanced analysis of RWD has the potential to further support regulatory decision-making and offers the possibility to provide an additional perspective on the use and performance of medicines in everyday clinical use, complementing the evidence obtained from clinical trials.

Key objectives:

To expand further the use of RWD to support regulatory decision making pre-and post-authorisation

Activities in 2026:
Complete the CAR-T report, disseminate the findings, and ensure that the results remain accessible and reusable for CAT/SAWP/CHMP for ongoing and future applications
Contribute to the study to assess the fitness-for-purpose of real-world data (RWD) sources for Duchenne Muscular Dystrophy: provide input and review the study deliverables including the study report and the end of project multi-stakeholder workshop

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Evaluation activities for human medicines

Initial evaluation activities

Patient and Healthcare Professional involvement in assessment work

The objective is to facilitate engagement of patients and healthcare professionals in benefit/risk evaluation and related activities and reflect their input in CAT assessments.

In addition, to facilitate the collection and use of patient experience data, so their perspectives and preferences can be considered in benefit/risk evaluations and related activities, along the medicine regulatory lifecycle.

Key objectives

Maintain current and explore additional processes to capture and include patient experience data within CAT benefit/risk evaluations.

Explore the role of patient reported outcomes in the development and authorisation of ATMPs

Activities in 2026:	CHMP
Monitor and improve methodologies to capture input to CAT procedures (including participation in oral explanations, written consultations, and engaging with patient and healthcare professional organisations at start of Marketing Authorisation Applications).	
Contribute to the public consultation and finalisation of the patient experience data Reflection Paper	
Initiate the development of recommendations on the inclusion of patient reported outcomes in the development of ATMPs	

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Evaluation activities for human medicines

Initial evaluation activities

Strengthening of the assessment of Companion Diagnostics (CDx)

CDx is essential for defining patients' eligibility for specific treatment with a medicinal product. As part of the conformity assessment of a CDx, the notified body shall seek a scientific opinion on the suitability of the CDx with the concerned medicinal product(s) from the competent authorities before issuing an EU technical documentation assessment certificate or an EU type-examination certificate, or a supplement to them for the CDx. The CAT seeks to strengthen the assessment process.

- **Key objectives:**

- Collaborate in the framework for identification of overarching issues in the assessment of CDx consultation procedures.
- Continued identification of general principles that can be later used for training of assessment teams and to update if needed procedural guidance or assessment templates.

Activities in 2026:	CHMP
Collaborate with the CDx expert group to consolidate the evaluation of consultation procedures across committee members	
Monitor assessments to capture input in CAT procedures at initial MAAs	
Collaborate with MWP on the guideline on biomarker development	

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

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Evaluation activities for human medicines

Post-authorisation activities

Post-authorisation safety and efficacy follow-up and RMP for ATMPs

Post-authorisation follow-up of patients treated with ATMPs is essential to collect data on long-term safety and efficacy of the authorised ATMP. It is essential that appropriate guidance is available to ATMP developers in order that appropriate post-authorisation studies can be planned that allow the generation of information.

Key objectives:

Develop guidance on post-authorisation follow-up and risk management planning for ATMPs, taking into account the need to adapt regulatory requirements of patient follow-up to incremental scientific knowledge and clinical experience

Activities in 2026:

Finalise the revision of the GVP Module V to incorporate ATMP specific information based on the feedback during the public consultation	PRAC
Initiate the drafting of a Questions and Answers (or publication) on the duration of follow-up of patients treated with CAR-T cells	

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

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Annex

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Leads and contributors for the activities

Activity	Lead(s)	Contributor(s)
Guideline on requirements for investigational ATMPs in clinical trials	Ilona Reischl, Claire Beuneu, Rune Kjeken, Olga Kholmanskihk	Silke Dorner, Heli Suila, Denisa Margina, Suzana Vidic, Kieran Breen, Barbara Bonamassa, Joseph deCourcey
Revision of the Questions and Answers on Gene Therapy	Claire Beuneu	Ilona Reischl, Violaine Closson Carella, Marcos Timon, Tineke van den Hoorn, Maria Isabel Vieira, Alessandra Renieri, Joseph deCourcey
Development of guidance on genome editing	Emmely de Vries, Tineke van den Hoorn	Martin Olieksiewicz, Rune Kjeken, Marcos Timon, Alessandra Renieri
Scientific consultations involving other decision makers to facilitate optimization of clinical evidence generation in drug development programmes	Maria Lüttgen	Rune Kjeken, Kerstin Sollerbrant
Real World Data (RWD) in regulatory decision making of ATMPs	Kieran Breen	Rozalina Kulaksazova, Emmely de Vries, Olga Kholmanskikh, Alessandra Renieri, Federica Chiari, Torbjörn Callréus
Patient and Healthcare Professional involvement in assessment work	Kieran Breen	Donatella Capone, Federica Chiara, Kerstin Sollerbrant
Strengthening of the assessment of Companion Diagnostics	Ilona Reischl	Silke Dorner, Heli Suila, Violaine Closson Carella, Jan Mueller-Berghaus, Olga Kholmanskikh, Una Riekstina, Liga Kunrade, Vilma Petrikaitė
Post-authorisation safety and efficacy follow-up and RMP for ATMPs **1. Revision of GVP Module V** **2. Q&A (or publication) on duration of follow-up of patients treated with CAR Ts**	1. Olga Kholmanskikh 2. Rune Kjeken	1. Concetta Quintarelli, Rune Kjeken, Julio Delgado 2. Olga Kholmanskikh, Jan Mueller-Berghaus, Attila Sebe, Violaine Closson-Carella, Charlotte Anderberg

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Main deliverables and achievements of 2025

Activity	Deliverables
Guideline on quality, non-clinical and clinical requirements for investigational ATMPs in clinical trials	Publication of the Guideline for investigational advanced therapy medicinal products in clinical trials Publication on ATMP clinical trials in EU: analysis performed and drafting of manuscript ongoing
Revision of the Questions and Answers on Gene Therapy	New topics identified for inclusion in the revised Q&A
Development of guidance on genome editing	Scientific workshop organised – workshop 16 Sept 2025 Next steps for the development of refection paper / concept paper agreed (drafting to start in 2026)
Enabling the safe and responsible use of Artificial Intelligence in the medicine lifecycle	Training session took place at CAT Strategic Review & Learning meeting in Sept 2025 A group of AI experts at CAT was onboarded
Benefit/Risk methodology and communication	• Final version of the reflection paper on single-arm trials presented to assessors • Key principles for documenting key (un) favourable effects in the effects table were agreed and incorporated into the new overview template.
Real World Data (RWD) in regulatory decision making of ATMPs	CAT contribution to the setting up of the RWD study on CAR T products CAT discussion at the CAT Strategic Review & Learning meeting in April 2025 on the inclusion of patient perspectives in regulatory decision making
Scientific consultation involving other decision makers to facilitate optimisation of clinical evidence generation in drug development programmes	Contribution to a workshop series and publication of the Joint HTAb-regulatory perspectives on understanding evidence challenges, managing uncertainties and exploring potential solutions as outcome of a workshop series between HTA bodies and regulators.
Provision of information to support Joint Clinical Assessments	Support to EMA on the process to provide information on ongoing MAA to support the work of JCA.
Implementation of the medical device regulation and strengthening of the assessment of Companion Diagnostics	Support provided to EMA and CHMP on procedure for the companion diagnostic consultation procedures. Contribution to the development of the guideline on biomarker development.
Post-authorisation safety and efficacy follow-up and RMP for ATMPs	Support to the review and analysis of the comments received during the public consultation on the guideline on safety and efficacy follow-up and risk management of ATMPs and the revision of the GVP module V to include ATMP specific guidance.
Engage with key stakeholders from industry, academia, not-for-profit and patient organisations	No specific meeting with stakeholders took place in 2025.
Offer the best scientific support to international convergence / harmonisation activities related to ATMPs.	CAT supported the activities of the ICH Gene and Cell therapy drafting group, WHO and IPRP. CAT actively collaborated with FDA.

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