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Updates from the Committee for Advanced Therapies

Joint PCWP - HCPWP meeting,

Amsterdam, 1st April 2025

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Committee for Advanced Therapies
European Medicines Agency

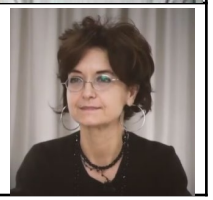
EMA
EUROPEAN MEDICINES AGENCY

The Committee for Advanced Therapies (CAT)

Responsible for assessing the quality, safety and efficacy of advanced therapy medicinal products (ATMPs)

Advanced therapy medicinal products (ATMPs) are medicines for human use that are based on genes, tissues or cells

Patient representatives in CAT

Members	
Kieran Breen, UK Parkinson's Europe Vice-chair, CAT	
Kerstin Sollerbrant, Sweden The Swedish Childhood Cancer Fund	
Alternates	
Mencía de Lemus Belmonte, Spain SMA Europe	
Federica Chiara, Italy Linfa OdV - neurofibromatosis	

Patient representatives in CAT are involved in several projects and activities

- Patients experience data – PED reflection paper
- Activities in the CAT work plan 2025
  - 12 activities in total, patient representatives are involved in 6 of these
  - Shortly mention 3 of them here
- Building trust in PEDs and PROMs in the CAT committee

Activities in the CAT work plan 2025

1. Real world data (RWD) in regulatory decision making 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 objective:

- To expand further the use of RWD including natural history data, patient treatment data, etc. from registries or other valid sources to support regulatory decision making pre-and post-authorisation and in-patient access to ATMPs.

Activities in the CAT work plan 2025 cont.

2. Scientific consultation involving other decision makers to facilitate optimisation 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

• Engage with down-stream decision makers with the aim of improving the clinical evidence generation for ATMPs

• Identify post-licensing evidence needs

Activities in the CAT work plan 2025 cont.

3. Interaction with Stakeholders

Engagement with ATMP developers is important to ensure a mutual understanding of important issues affecting ATMP development, approval and patient access.

Key objectives

- Engage with key stakeholders from industry, academia, not-for-profit and patient organisations.

Activities for 2025

- Contribute to a meeting with Patients' organisations
- And more…..

Building trust in PEDs and PROMs in the CAT committee

- **SRLM**, Strategic Review and Learning Meetings – twice a year
  - Measure what is actually clinically meaningful to patients, and real life consequences
- **SRLM in Leuven, May 17th 2024**
  - *Patient-relevant outcomes to support regulatory decisions on ATMPs*
  - Inherited diseases: Spinal Muscular Atrophy, SMA; Duchenne Muscular Distrophies, DMD and Retinal diseases
- **SRLM in Budapest, Nov 19th 2024**
  - *Patient reported outcome measures in oncology trials and regulatory decision making.*
  - Cancer

Building trust in PEDs and PROMs in the CAT committee cont.

- **SRLM in Warsaw, May 17th 2025**
  - *"Optimising the inclusion of the patient perspective at the CAT",* three parts:

    i. inclusion of PROMs in the assessments (inspired by FDA)
    ii. inclusion of PROMs in the post authorization tools (PASS and PAES)
    iii. procedures and mechanisms to ensure the inclusion of the patient perspective during the assessment procedures

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Thank you

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