



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Patient Health Protection

CAT monthly report of application procedures, guidelines and related documents on advanced therapies

October 2012 meeting

The Committee for Advanced Therapies (CAT) held its 42nd CAT meeting on 11th – 12th October 2012.

The CAT Monthly Report includes statistical data on CAT scientific recommendations on Advanced Therapy Medicinal Product (ATMP) classification, certifications, initial evaluations, CAT contributions to Scientific Advice and Paediatric Investigation Plans, as well as variations, line extensions, renewals.

In addition, the report includes a summary table of the draft opinions issued by the CAT in the current year and a list of adopted guidelines and other public documents.

ATMP Certification Procedure

Further to the CAT recommendation the Agency issued the second certification on the quality data of an ATMP product developed by an SME. The ATMP concerned by the certification is a suspension of autologous expanded smooth muscle derived cells, developed for the proposed therapeutic use: treatment of stress urinary incontinence. This product was previously classified by the CAT as a Tissue Engineered Product.

Scientific recommendation on advanced therapy product classification

Further to consultation with the European Commission, the CAT finalised one scientific recommendation on the following classification of advanced therapy medicinal products (ATMPs).

The following product was classified as a gene therapy medicinal product, combined ATMP:

- Encapsulated cell-based delivery system engineered to deliver human ciliary neurotrophic factor (CNTF) intraocularly after implantation. The product is intended for reducing photoreceptor loss associated with degeneration of the cells of the retina.

CAT received three new ATMP classification requests for which a scientific recommendation will be delivered within 60 days (active review time) after receipt of the final request.

Further information on the ATMP classification procedure can be found at:

[European Medicines Agency - ATMP classification - ATMP classification](#)



Organisation matters

- CAT adopted a revision of its Rules of Procedure (EMA/CAT/454446/2008 rev. 1) related to the calculation of the quorum and majority (article 6). The revisions CAT Rules of Procedure will be published in due course at:
[European Medicines Agency - Committee for Advanced Therapies: Overview](#)
- CAT conducted the election of the Vice-Chair during its 42nd meeting. CAT re-elected Dr Paula Salmikangas as its Vice-Chair for a second three-year term.
- CAT adopted the composition of five *ad-hoc* drafting groups, composed of members from the CAT, former working parties and additional experts to complement expertise amongst the CAT members, to finalise the guidance documents that were already under development by the former Gene Therapy Working Party and Cell-based Products Working Party. A first meeting of one of the drafting groups took place in the margins of the October CAT meeting.
- CAT was informed of the activities and discussions of the Working Party at the EDQM (European Directorate for Quality of Medicines & Health Care) on raw materials for the production of cellular and gene transfer products. A joint EDQM/EMA meeting will be organised with manufacturers of these raw materials and ATMP developers on 3rd and 4th April 2013 in the EDQM premises in Strasbourg, France.
- CAT received feedback from the informal meeting, organised by the Norwegian Medicines Agency, which took place in Oslo on 24th-25th September 2012, on the implementation of the 'Hospital Exemption'.

Other Scientific issues

- On 11th October, CAT held a scientific workshop on 'Reducing the number of laboratory animals used in tissue engineering research'. CAT members/alternates, CAT Interested Parties representatives and experts from Academia had a fruitful exchange of views on the animal testing requirements for cell-based medicinal products. The agenda of the workshop is available at:
[European Medicines Agency - News and Events - Events and meeting calendar](#)
- A meeting of the CAT Focus Group (FG) on non-clinical development of ATMPs was held on 11th October, adjacent to the above mentioned scientific workshop. CAT Focus Groups are convened by CAT with representatives of Interested parties (IPs) to discuss specific topics and propose possible shared solutions to bottlenecks in the development of ATMPs. The CAT-IP FG on non-clinical development of ATMPs started operating in February 2011. A summary of the discussions held by the focus group will be available at:
[European Medicines Agency - Interested Parties to the CAT](#)

Organisations which have not yet registered to become an interested party to the CAT can still do so by completing the form that can be found on the Agency's Website:

[European Medicines Agency - Interested Parties to the CAT](#)

Overview of product-related activities

The Committee discussed ongoing evaluation procedures for ATMPs and other related procedures as summarised in the following tables:

Initial Evaluation of MAA for ATMP					
	2009	2010	2011	2012	Total
Submitted	3	1	2	2	8
Positive draft Opinion	1	0	1 ⁱ	1 ⁱ	3
Negative draft Opinion	1 [*]	0	1	0	2
Withdrawals	1	1	0	0	2

* Application subsequently withdrawn

ⁱ Re-examination opinion (Glybera)

Scientific recommendation on advanced therapy classification					
	2009	2010	2011	2012	Total
Submitted	22	19	12	17	70
Adopted	12	27	12	14	65

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs					
	2009	2010	2011	2012	Total
Submitted	1	0	0	1	2
Adopted	0	1	0	1	2

Scientific advice procedures on ATMPs					
	2009	2010	2011	2012	Total
Discussed*	25	30	36	28	119
Written comments to SAWP	17	15	8	1	41

* Most scientific advices for ATMPs are discussed by the CAT at 2 time points during the SA procedure

Paediatric Investigation Plans (PIP) for ATMPs					
	2009	2010	2011	2012	Total
Discussed*	4	7	6	4	23
Written comments to PDCO	3	1	4	0	8

* PIPs for ATMPs are discussed by the CAT once or twice during the procedure

Upcoming meetings following the October 2012 CAT meeting

The 43rd meeting of the CAT will be held at the Agency on 8th – 9th November 2012.

NOTE:

1. This Monthly Report and other documents can be found on the internet at the following location:
[European Medicines Agency - Committee meeting reports - CAT: Committee meeting reports](#)
2. Specific information related to ATMPs and the activities of the CAT (role, responsibilities and composition) can be found at: [European Medicines Agency - CAT - Committee for Advanced Therapies \(CAT\)](#)

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