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EMA/CAT/256262/2013
Patient Health Protection

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

April 2013 meeting

The Committee for Advanced Therapies (CAT) held its 48th CAT meeting on 18th – 19th April 2013.

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.

Centralised Procedure: Evaluation of MACI concluded

CAT adopted by consensus a positive draft opinion on the marketing authorisation application for MACI. MACI (matrix applied characterised autologous cultured chondrocytes) from Genzyme Europe B.V. is a combined Tissue Engineered Product intended for the repair of symptomatic, full-thickness cartilage defects of the knee (grade III and IV of the Modified Outerbridge Scale) of 3-20 cm^2 in skeletally mature adult patients.

On basis of the draft CAT opinion, CHMP adopted a positive opinion recommending the granting of a marketing authorisation for MACI.

MACI is the first combined ATMP that is recommended for approval in the European Union. More information on the MACI approval can be found here: EMA Press release

Scientific recommendation on advanced therapy product classification

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

The following products were classified as a somatic cell therapy medicinal product:

- Naturally-occurring allogeneic donor lymphocytes enriched for antigen-specific CD4+ and CD8+ T cells using the CliniMACS Cytokine Capture system, intended for the treatment of therapy-refractory infectious and infection-related diseases and pre-emptive and prophylactic treatment of infectious and infection-related diseases.

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- Allogeneic bone marrow derived mesenchymal cells (MSCs) expanded ex vivo, intended for the treatment of acute Graft-versus-Host Disease grades III and IV resistant to first line treatment.
- Human autologous tumour-infiltrating lymphocytes, intended for the treatment of stage III melanoma with one invaded lymph node.

The following product was classified as a gene therapy medicinal product:

- Pseudomonas aeruginosa bacteria genetically modified to secrete oncoproteins of Merkel cell carcinoma, intended for the treatment of Merkel Cell Carcinoma.

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

Scientific Guidelines

CAT adopted the reflection paper on the clinical risks deriving from insertional mutagenesis (EMA/CAT/190186/2012). The reflection paper discusses the factors contributing to genotoxicity of gene therapy vector integration, the strategies to reduce the risk associated with insertional mutagenesis and the assays to evaluate vector oncogenesis at the non-clinical and clinical level.

The work on this reflection paper was initiated by the former Gene therapy working party after a meeting with experts in February 2011 and was finalised by one of the CAT drafting groups.

The reflection paper will be published shortly and can be found at:

European Medicines Agency - ATMP - Gene therapy guidelines

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 Marketing Authorisation Applications (MAA) for ATMP						
	2009	**2010**	**2011**	**2012**	**2013**	**Total**
Submitted MAAs	3	1	2	3	1	10
Positive draft Opinion	1	0	1[ii]	1[ii]	1	4 Corresponding to 3 ATMPs
Negative draft Opinion	1[i]	0	1[ii]	0	0	2 Corresponding to 0 ATMPs*
Withdrawals	1	1[i]	0	0	2	4
Ongoing MAAs						3

i Same product (Cerepro)
ii Same product (Glybera)
* MAAs subsequently withdrawn or re-examinated.

Scientific recommendation on advanced therapy classification						
	2009	**2010**	**2011**	**2012**	**2013**	**Total**
Submitted	22	19	12	17	9	84
Adopted	12	27	12	14	12	79

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs						
	2009	**2010**	**2011**	**2012**	**2013**	**Total**
Submitted	1	0	0	1	1	3
Adopted	0	1	0	1	0	2

Scientific advice procedures on ATMPs						
	2009	**2010**	**2011**	**2012**	**2013**	**Total**
Discussed*	25	30	36	31	6	131

* 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**	**2013**	**Total**
Discussed*	4	7	6	9	2	28

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

Upcoming meetings following the April 2013 CAT meeting

The 49th meeting of the CAT will be held at the Agency on 22nd – 23rd May 2013.

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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