EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Procedure Management and Business Support Division

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

September 2014 meeting

The Committee for Advanced Therapies (CAT) held its 63rd CAT meeting on 18-19 September 2014.

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.

MACI: suspension of marketing authorisation

On 5 September 2014 the marketing authorisation holder closed the EU manufacturing site for MACI (matrix-applied characterised autologous cultured chondrocytes) and MACI will no longer be available in the EU. The closure of the site was due to commercial reasons and the benefit-risk balance of MACI remains positive.

Following the closure of the manufacturing site and because EU legislation requires authorised medicines to have a registered manufacturing site, a review was initiated at the request of the European Commission on 10 September 2014 (under Article 20 of Regulation (EC) No 726/2004) to determine whether the marketing authorisation for MACI should be suspended or revoked.

On 19 September 2014, CAT adopted a draft opinion recommending the suspension of the marketing authorisation of MACI until a new manufacturing site is registered in the EU. The draft CAT opinion was transmitted to the CHMP, who adopted on 25 September 2014 its final opinion recommending the suspension of the marketing authorisation for MACI. The final opinion will now be sent to the European Commission which will issue a legally binding decision.

Scientific recommendation on advanced therapy product classification

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

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

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- Two Adeno-Associated Viral Vector carrying genes to suppress the production of non-functional rhodopsin and to provide a functional rhodopsin, intended for the treatment of autosomal dominant rhodopsin-linked retinitis pigmentosa.

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

- Allogeneic peripheral blood mononuclear cells induced to an early apoptotic state, intended for the prevention of graft versus host disease.
- Autologous mature dendritic cells pulsed with synthetic peptides derived from tumour antigens, intended for the treatment of glioblastoma.

The following products were classified as tissue engineered products:

- Selected and *ex vivo* expanded human cord blood cells, intended for haematopoietic and immunological reconstitution after non-myeloablative conditioning regimens in haematogical malignancies.
- Autologous bone marrow derived mononuclear cells, intended for the treatment of chronic heart disease.

The following product was classified as non ATMPs:

- Killed spores of *Bacillus subtilis* incorporation a non-toxic antigen of *Clostridium difficile*, intended for the prevention of *Clostridium difficile* infections in elderly patients.

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

European Medicines Agency - ATMP classification

CAT Workshop on ATMPs

Feedback was given to the Committee on the successful workshop held on 11 September 2014 in association with the German Society for Transfusion Medicines and Immunohematology (DGTI) and the German Stem Cell Network (GSCN). This workshop, which was held in the margins of 47th Annual meeting of the DGTI, was attended by over 150 participants from academia, hospitals, ATMP manufacturers and authorities. The presentations of this workshop will shortly become available on the EMA Website.

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**	**2014**	**Total**
Submitted MAAs	3	1	2	3	2	2	13
Positive draft Opinion	1	0	1[i]	1[i]	2	0	5 Corresponding to 4 ATMPs
Withdrawals	1	1	0	0	2	0	4
Ongoing MAAs							5

[i] Same product (Glybera)

Variations (Type II) for authorised ATMP							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**Total**
Positive draft Opinion	0	0	1	1	9	4	15

Scientific recommendation on advanced therapy classification							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**Total**
Submitted	22	19	12	17	20	21	116
Adopted	12	27	12	14	23	19	109

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**Total**
Submitted	1	0	0	1	3	0	5
Adopted	0	1	0	1	1	2	5

Scientific advice procedures on ATMPs							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**Total**
Discussed*	25	30	36	31	36	31	189

* 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**	**2014**	**Total**
Discussed*	4	7	6	9	7	5	38

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

Upcoming meetings following the September 2014 CAT meeting

The 64th meeting of the CAT will be held at the Agency on 16-17 October 2014.

NOTE:

1. This Monthly Report, the Agenda of this meeting, the Minutes of the previous CAT meeting 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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