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Procedure Management and Committees Support Division

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

November 2015 meeting

The Committee for Advanced Therapies (CAT) held its 76th CAT meeting on 12 – 13 November 2015.

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.

Scientific recommendation on advanced therapy product classification

Further to consultation with the European Commission, the CAT finalised eight scientific recommendations on the classification of advanced therapy medicinal products.

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

- Autologous bone marrow derived mesenchymal stem cells intended for the treatment of amyotrophic lateral sclerosis.
- Autologous adipose tissue derived mesenchymal stem cells intended for the treatment of amyotrophic lateral sclerosis.
- Allogeneic umbilical cord (human Wharton's jelly) derived mesenchymal stem cells intended for the treatment of amyotrophic lateral sclerosis.

The following products were classified as tissue engineered products:

- Allogeneic mesenchymal precursor cells intended for the treatment of chronic lumbar back pain.
- Autologous expanded mesenchymal stromal cells seeded onto an allogeneic human decellularised trachea scaffold intended for the reconstruction of trachea subsequent to damage or stenosis due to cancer, injury, infection or congenital deformities.
- Hepatocyte-like human embryonic stem cell-derived cells intended for the treatment of inborn liver metabolic diseases like Crigler-Naijar syndrome 1 and for drug-induced acute liver failure such as paracetamol intoxication.

The following product was classified as tissue engineered product, combined ATMP:

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- Autologous expanded viable chondrocytes embedded in a cross linked hydrogel intended for the treatment on articular cartilage defects.

The following product was classified as non-ATMP:

- Haematopoietic progenitor cells, facilitating cells and αβ T cells from mobilized peripheral blood mononuclear cells intended to be used after kidney transplantation.

CAT received 14 new ATMP classification requests for which a scientific recommendation will be delivered within 60 days (active review time).

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

European Medicines Agency - ATMP classification

Organisational, regulatory and methodological matters

- CAT discussed the Good Laboratory Practice (GLP) requirements for non-clinical studies for ATMPs.
- CAT agreed with the development of a CAT-CHMP (Safety Working Party) reflection paper on tumourigenicity studies for cell-based ATMPs.
- CAT discussed and provided input in the two following procedural guidelines: CHMP guideline on conditional marketing authorisation; CHMP guideline on the scientific application and the practical arrangements necessary to implement the procedure for accelerated assessment pursuant to article 14(9) of regulation (EC) 726/2004.
- CAT discussed the following early access initiatives and its application to ATMPs: Adaptive pathway initiative; PRIME.

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**	**2015**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	14
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	7 Corresponding to 6 ATMPs
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	4
Withdrawals	1	1[i]	0	0	2	0	0	4
Ongoing MAAs								3

[i] Same product (Cerepro)
[ii] Same product (Glybera)
[iii] Same product (Heparesc)

Variations (Type II) for authorised ATMP								
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Positive draft Opinion	0	0	1	1	9	4	3	18

Scientific recommendation on advanced therapy classification								
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Submitted	22	19	12	22	20	28	42	165
Adopted	12	27	12	16	23	29	27	146

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs								
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Submitted	1	0	0	1	3	1	1	7
Adopted	0	1	0	1	1	2	1	6

Scientific advice procedures on ATMPs								
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Discussed*	25	30	36	31	36	48	58	264
Number of procedures	17	19	21	19	23	33	40	172

* 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**	**2015**	**Total**
Discussed*	4	7	6	9	7	7	3	43

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

Upcoming meetings following the November 2015 CAT meeting

The 77th meeting of the CAT will be held on 10 – 12 December 2015.

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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