28 January 2016
EMA/68141/2016
Procedure Management and Committees Support Division

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

January 2016 meeting

The Committee for Advanced Therapies (CAT) held its 78th CAT meeting on 21-22 January 2016.

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 eighteen scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Adeno-associated virus serotype 8 vector encoding human ornithine transcarbamylase intended for the treatment of ornithine transcarbamylase deficiency.
- Allogeneic chondrocytes and irradiated genetically modified chondrocytes expressing human TGF-ß1 intended for the treatment of degenerative joint disease.

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

- Autologous bone marrow derived non-haematopoietic stem cells intended for the treatment of rheumatoid arthritis.
- Autologous bone marrow derived non-haematopoietic stem cells intended for the treatment of type I diabetes.
- Autologous bone marrow derived non-haematopoietic stem cells intended for the treatment of type II diabetes.
- Bioartificial liver system intended for the treatment of acute liver failure.

The following products were classified as tissue engineered products:

- Autologous cells of the stromal vascular fraction and autologous adipose derived stem cells intended for the treatment of diabetic foot ulcer.

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- Autologous bone marrow derived non-haematopoietic stem cells intended for the treatment of myocardial infarction.
- Autologous bone marrow derived non-haematopoietic stem cells intended for the treatment of ischemic stroke.
- Autologous peripheral blood-derived total nucleated cells intended for the treatment of critical limb ischemia.
- Co-culture of fibroblasts and keratinocytes as a suspension, intended for the treatment of deep and extensive burns, chronic wound and skin donor sides.
- Co-culture of fibroblasts and keratinocytes as a sheet, intended for the treatment of deep and extensive burns, chronic wound and skin donor sides.
- Co-culture of fibroblasts and keratinocytes seeded on acellular amniotic matrix, intended for the treatment of deep and extensive burns, chronic wound and skin donor sides.
- Co-culture of fibroblasts and keratinocytes seeded on acellular dermal matrix, intended for the treatment of deep and extensive burns, chronic wound and skin donor sides.

The following products were classified as not ATMPs:

- Allograft tendon combined with suture intended for the anterior cruciate ligament reconstruction.
- Human acellular amniotic matrix intended for the treatment of deep and extensive burns, chronic wound and skin donor sides.
- Human acellular dermal matrix intended for the treatment of deep and extensive burns, chronic wound and skin donor sides.
- Transgenic porcine acellular dermal matrix intended for the treatment of deep and extensive burns, chronic wound and skin donor sides.

CAT received 11 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

CAT adopted its work plan for 2016

Highlights from the work plan are the development of guidance documents; contribution to cross-committee projects; work on simplification of procedures and requirement for ATMPs and the organisation of an ATMP assessor training and a scientific workshop on cell-based cancer immunotherapy products.

The CAT work plan will be published shortly on the EMA website.

Organisational matters

- CAT discussed with colleagues from the European Commission, DG Research ATMP-related research priorities that could be considered in future calls funded by Horizon 2020.
- CAT reflected on the interactions between them and the scientific advice working party and how to improve the current process of providing CAT input on scientific advice requests for ATMPs.

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**	**2016**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	0	14
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	0	7 Corresponding to 6 ATMPs
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	4
Withdrawals	1	1[i]	0	0	2	0	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**	**2016**	**Total**
Positive draft Opinion	0	0	1	1	9	4	3	3	21

Scientific recommendation on advanced therapy classification									
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**Total**
Submitted	22	19	12	22	20	28	61	11	195
Adopted	12	27	12	16	23	29	31	18	168

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Submitted	1	0	0	1	3	1	1	0	7
Adopted	0	1	0	1	1	2	1	0	6

Scientific advice procedure for ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Discussed*	25	30	36	31	36	48	63	1	270
Number of procedures	17	19	21	19	23	33	39	7	178

* 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	2016	Total
Discussed*	4	7	6	9	7	7	3	1	44

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

Upcoming meetings following the January 2016 CAT meeting

The 79th meeting of the CAT will be held on 18-19 February 2016.

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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