14 December 2017
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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

December 2017 meeting

The Committee for Advanced Therapies (CAT) held its 99th CAT meeting on 6 – 8 December 2017.

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.

CAT recommends the granting of the marketing authorisation for Alofisel

Alofisel is a tissue engineered product containing expanded allogeneic mesenchymal stem cells derived from adipose tissue. Alofisel is indicated for the treatment of complex perianal fistulas in patients with Crohn's disease.

Following an in-depth review of the dossier submitted by the applicant, Tigenix, S.A.U., CAT concluded during its December meeting that a positive benefit risk profile has been demonstrated. CAT adopted the positive draft opinion recommending the granting of the marketing authorisation for Alofisel. The CHMP subsequently adopted the positive opinion for Alofisel during its December 2017 meeting.

Scientific recommendation on advanced therapy product classification

Further to consultation with the European Commission, the CAT finalised 2 scientific recommendations on the classification of advanced therapy medicinal products.

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

- Encapsulated human retinal pigment epithelial cells genetically modified to express human factor IX protein, intended for the treatment of haemophilia B.

The following product was classified as non-ATMP:

- CD1c (BDCA-1) positive myeloid dendritic cells, intended for the treatment of advanced, pre-treated solid tumours.

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Organisational matters

- The CAT adopted the procedural advice on the evaluation of advanced therapy medicinal products. This document is a revision of the procedural advice published in 2011 (EMA/354785/2010), taking into account the experience gained. It describes the procedure and the interactions between EMA, the different committees (CAT, CHMP, PRAC) and the applicants during the centralised evaluation of ATMPs. The document will be published shortly.
- The CAT adopted its work plan for 2018. The plan focusses firstly on the development of scientific guidelines for ATMPs. The plan will be published shortly.

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**	**2017**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	4	19
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	2	11*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	4
Ongoing MAAs										4

*** Corresponding to 9 ATMPs**
[i] Same product (Cerepro)
[ii] Same product (Glybera)
[iii] CAT adopted two negative draft opinions for the same product (Heparesc)

Variations (Type II) for authorised ATMP										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Positive Opinion	0	0	1	1	9	4	3	6	3	27

Scientific recommendation on advanced therapy classification										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Submitted	22	19	12	22	20	28	61	60	46	290
Adopted	12	27	12	16	23	29	31	87	49	286

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Submitted	1	0	0	1	3	1	1	2	2	11
Adopted	0	1	0	1	1	2	1	1	3	10

Scientific advice procedure for ATMPs										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Number of procedures	17	19	21	19	23	33	39	46	56	273

Paediatric Investigation Plans (PIP) for ATMPs										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Number of procedures	3	4	4	8	5	4	3	5	3	39

Prime Eligibility for ATMPs									
	2016	**2017**							**Total**
Discussed	22	16							38
Granted	8	6							14

Upcoming meetings following the December 2017 CAT meeting

- The 100th meeting of the CAT will be held on 17 – 19 January 2018.

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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