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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

November 2018 meeting

The Committee for Advanced Therapies (CAT) held its 109th CAT meeting on 7 – 8 November 2018.

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

The following products were classified as gene therapy medicinal products:

- Codon-optimised human cystic fibrosis transmembrane conductance regulator mRNA complexed with lipid based nanoparticles, intended for the treatment of cystic fibrosis.
- Adeno-associated virus vector containing a human neuronal ceroid lipofuscinosis expression cassette encoding for the soluble lysosomal enzyme tripeptidyl-peptidase 1, intended for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 disease.

The following product was classified as a somatic cell therapy medicinal product:

- Allogeneic Epstein-Barr virus (EBV)-specific cytotoxic T cells, intended for the treatment of refractory / relapsed EBV-associated post-transplant lymphoproliferative disease.

The following products were classified as tissue engineered products:

- Autologous bone marrow derived mesenchymal stem cells, intended for the treatment of ischemic stroke.
- Autologous bone marrow derived mesenchymal stem cells, intended for the regeneration of cartilage, ligamentum, bone and muscle defects.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Organisational matters

- CAT discussed the Guideline on requirements for investigational ATMPs. This guideline is expected to be released for public consultation by end of 2018.
- CAT held a discussion with an invited physician treating haemophilia patients, a patient representative (from the European Haemophilia Consortium) and a patient on gene therapy medical products for the treatment of haemophilia.

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**	**2018**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	4	2	21
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	2	3	14*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	1	5
Ongoing MAAs											2

*** Corresponding to 13 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**	**2018**	**Total**
Positive Opinion	0	0	1	1	9	4	3	6	3	6	33

Scientific recommendation on advanced therapy classification											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Submitted	22	19	12	22	20	28	61	60	46	50	340
Adopted	12	27	12	16	23	29	31	87	49	38	324

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs											
	2009	2010	2011	2012	2013	2014	2015	2016	2017	2018	Total
Submitted	1	0	0	1	3	1	1	2	2	0	11
Adopted	0	1	0	1	1	2	1	1	3	1	11

Scientific advice procedure for ATMPs											
	2009	2010	2011	2012	2013	2014	2015	2016	2017	2018	Total
Number of procedures	17	19	21	19	23	33	39	46	55	49	321

Paediatric Investigation Plans (PIP) for ATMPs											
	2009	2010	2011	2012	2013	2014	2015	2016	2017	2018	Total
Number of procedures	3	4	4	8	5	4	3	5	3	2	41

Prime Eligibility for ATMPs									
	2016	2017	2018						Total
Discussed	22	16	14						52
Granted	8	6	6						20

Upcoming meetings following the October 2018 CAT meeting

- The 110th meeting of the CAT will be held on 5 – 7 December 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)

Thorsten Olski
Head of Scientific Committees Secretariat
Tel.: (+44-20) 3660 7684
AdvancedTherapies@ema.europa.eu