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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 2018 meeting

The Committee for Advanced Therapies (CAT) held its 110th CAT meeting on 5 – 7 December 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:

- Genetically modified bone marrow derived allogeneic mesenchymal stem cells expressing human alpha-1 antitrypsin, intended for the treatment of steroid refractory acute graft-versus-host disease.
- Adeno-associated virus vector containing a transgene that encodes a microRNA targeting huntingtin, intended for the treatment of Huntington's disease.

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

- *Ex vivo* treated human donor haematopoietic stem cells, intended for the treatment of severe combined immunodeficiency.

The following products were classified as tissue engineered products:

- Allogeneic Wharton's jelly derived mesenchymal stem cells on a dermal scaffold, intended for the treatment of epidermolysis bullosa.
- Suspension of human olfactory ensheathing cells and olfactory nerve fibroblasts, intended for the treatment of complete and incomplete spinal cord injuries, aiming to support neuroregeneration.

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Organisational matters

- CAT discussed and adopted the Guideline on requirements for investigational ATMPs. This guideline will be released for public consultation after adoption by CHMP during their January 2019 meeting.
  The guideline provides guidance on the structure and data requirements for a clinical trial application for exploratory and confirmatory trials with advanced therapy investigational medicinal products.

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	3	22
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											3

*** 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	8	35

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	55	345
Adopted	12	27	12	16	23	29	31	87	49	43	329

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	1	12
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	53	325

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	3	42

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

Upcoming meetings following the December 2018 CAT meeting

- The 111th meeting of the CAT will be held on 23 – 25 January 2019.

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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