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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

October 2018 meeting

The Committee for Advanced Therapies (CAT) held its 108th CAT meeting on 10 – 12 October 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 3 scientific recommendations on the classification of advanced therapy medicinal products.

The following product was classified as a gene therapy medicinal product:

- Genetically modified adeno-associated virus 9 expressing short hairpin RNA (shRNA) targeting mutant polyadenylate RNA binding protein nuclear 1 (PABPN1) as well as a codon-optimised shRNA-insensitive wildtype poly(A) binding protein nuclear 1 (PABPN1), intended for the treatment of oculopharyngeal muscular dystrophy.

The following products were classified as non-ATMPs:

- Donor derived CD34+ haematopoetic stem cells and donor derived CD3+ T-cells, intended for the prevention of kidney graft loss.
- Stromal vascular fraction, intended for the regeneration of epithelial fibrosis as a result of vulvar lichen sclerosis.

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.
- The report from the expert meeting held on 18 October 2017 on genome editing technology used in medicinal product development has been published on the EMA website.

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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	45	335
Adopted	12	27	12	16	23	29	31	87	49	33	319

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	41	313

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	12						50
Granted	8	6	6						20

Upcoming meetings following the October 2018 CAT meeting

- The 109th meeting of the CAT will be held on 7 – 8 November 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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