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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

May 2018 meeting

The Committee for Advanced Therapies (CAT) held its 104th CAT meeting on 23 – 25 May 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 4 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as a tissue engineered products:

- Allogeneic foetal neural stem cells intended for the treatment of amyotrophic lateral sclerosis.
- Allogeneic foetal neural stem cells intended for the treatment of spinal cord injuries.
- Autologous adipose cells intended for the treatment of annual fistula.

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

- Exosomes carrying recombinant cystic fibrosis transmembrane conductance regulator mRNA and microRNA-17, intended for the treatment of cystic fibrosis.

Organisational matters

- CAT agreed on a set of Questions and Answers related to the assessment of similarity for ATMPs in the context of the orphan legislation. These Questions and Answers, which accompany the revised Commission Regulation laying down the provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product and definitions of the concepts ‘similar medicinal product’ and ‘clinical superiority’, were published on the website of the European Commission.
- CAT agreed with the outcome of the discussions that where initiated by the European Commission between the GMO Authorities and the Competent Authorities for medicines on the ERA assessment of human genetically modified cells and plasmids in clinical trials.

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- CAT noted the draft guidelines on GCP for ATMPs, which were developed by a drafting group composed on CAT members and members of the GCP inspection group. The draft guidelines will be published by the European Commission for a public consultation.

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	1	20
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	2	0	11*
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	0	4
Ongoing MAAs											5

*** Corresponding to 10 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	3	30

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	22	312
Adopted	12	27	12	16	23	29	31	87	49	16	302

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	26	298

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	1	40

Prime Eligibility for ATMPs									
	2016	**2017**	**2018**						**Total**
Discussed	22	16	8						46
Granted	8	6	1						15

Upcoming meetings following the May 2018 CAT meeting

- The 105th meeting of the CAT will be held on 20 – 22 June 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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