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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

September 2019 meeting

The Committee for Advanced Therapies (CAT) held its 118th meeting on 11 – 13 September 2019.

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

The following products were classified as somatic cell therapy medicinal products:

- Autologous, *ex vivo* expanded, clonal neo-antigen specific tumour infiltrating lymphocytes, intended for the treatment of solid tumours;
- Autologous tumour-infiltrating lymphocytes, intended for the treatment of solid tumours.

The following product was classified as a tissue engineered product:

- CD34+ haematopoietic stem/progenitor cells enriched with normal mitochondria derived from white blood cells from a related donor, intended for the treatment of non-inherited mitochondrial DNA deletion syndromes.

The following products were classified as gene therapy medicinal products:

- Autologous CD34+ cells transduced with lentiviral vector encoding human γ-globinG16D and short-hairpin RNA734, intended for the treatment of moderate to severe Sickle Cell disease;
- Recombinant adeno-associated viral vector serotype 2 encoding the complementary DNA of human Rab escort protein type 1, intended for the treatment of choroideremia.

The following product was classified as not an advanced therapy medicinal product:

- Uncapped, non-coding ribonucleic acid, intended for the treatment of adenoid cystic carcinoma, squamous cell carcinoma of the head and neck, melanoma and squamous cell carcinoma of the skin.

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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**	**2019**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	4	3	0	22
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	2	3	1	15*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	1	0	5
Ongoing MAAs												2

*** Corresponding to 14 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**	**2019**	**Total**
Positive opinion	0	0	1	1	9	4	3	6	3	8	13	48

Scientific recommendation on advanced therapy classification												
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**2019**	**Total**
Submitted	22	19	12	22	20	28	61	60	46	55	35	380
Adopted	12	27	12	16	23	29	31	87	49	43	43	372

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**	**2019**	**Total**
Submitted	1	0	0	1	3	1	1	2	2	1	1	13
Adopted	0	1	0	1	1	2	1	1	3	1	1	12

Scientific advice procedure for ATMPs												
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**2019**	**Total**
Number of procedures	17	19	21	19	23	33	39	46	55	53	45	370

Paediatric Investigation Plans (PIP) for ATMPs												
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**2019**	**Total**
Number of procedures	3	4	4	8	5	4	3	5	3	3	2	44

Prime Eligibility for ATMPs									
	2016	**2017**	**2018**	**2019**					**Total**
Discussed	22	16	14	10					62
Granted	8	6	6	4					24

Upcoming meetings following the September 2019 CAT meeting

- The 119th meeting of the CAT will be held on 9 – 11 October 2019.

NOTE:

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

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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