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Inspections, Human Medicines, Pharmacovigilance and Committees Division

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

April 2019 meeting

The Committee for Advanced Therapies (CAT) held its 114th meeting on 16 – 17 April 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 4 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Recombinant adeno-associated virus (serotype 8) encoding a codon optimised cDNA encoding human retinitis pigmentosa GTPase regulator, intended for the treatment of X-linked retinitis pigmentosa;
- Autologous T-cells transduced with a T-cell receptor targeting human telomerase reverse transcriptase (hTERT), intended for the treatment of various cancer types expressing hTERT.

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

- Allogeneic adult bone marrow derived stem cells transiently transfected with a plasmid encoding the intracellular domain of human Notch-1, intended for the treatment of motor deficits arising from acquired brain injury, including traumatic brain injury, ischaemic stroke and haemorrhagic stroke.

The following product was classified as tissue engineered product:

- Allogeneic cord blood mononuclear cells, intended for the treatment of neurological disorders, autism spectrum disorders, cerebral palsy.

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Organisational, regulatory and methodological matters

- CAT finalised the Questions and Answers document on the use of Out-of-Specification batches of cell/tissue based authorised ATMPs. This document provides practical information to marketing authorisation holders on the implementation of the provision in Section 11.5 of the Guidelines on GMP for ATMPs[1]. The Questions and Answers will be published on the EMA website shortly.
- CAT discussed the agenda of the joint CAT/CTFG[2] Strategic Review and Learning meeting that will be held in Bucharest, Romania on 13 – 14 June under the auspices of the Romanian presidency of the European Union.

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	4	39

[1] https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-4/2017_11_22_guidelines_gmp_for_atmps.pdf
[2] Clinical Trial Facilitation Group

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	17	362
Adopted	12	27	12	16	23	29	31	87	49	43	26	355

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	0	12
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	20	345

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	0	42

Prime Eligibility for ATMPs									
	2016	**2017**	**2018**	**2019**					**Total**
Discussed	22	16	14	4					56
Granted	8	6	6	3					23

Upcoming meetings following the April 2019 CAT meeting

- The 115th meeting of the CAT will be held on 22 – 24 May 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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