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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

July 2019 meeting

The Committee for Advanced Therapies (CAT) held its 117th meeting on 17-19 July 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 product was classified as a tissue engineered product:

- Autologous $CD34^+$ cells, intended for the treatment of no-option critical limb ischemia.

The following products were classified as gene therapy medicinal products:

- Modified Vaccinia Ankara-Bavarian Nordic-Brachyury and recombinant fowlpox virus-Brachyury encoding the human brachyury gene and expressing three co-stimulatory molecules (CD80, CD54 and CD58), intended for the treatment of chordoma
- Messenger ribonucleic acid coding for coiled-coil domain-containing protein 40 (CCDC40) protein, intended for the treatment of primary ciliary dyskinesia (PCD) caused by biallelic mutation in the CCDC40 gene
- Autologous cultured peripheral blood T cells, CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor, intended for the treatment of various types of cancer

Organisational matters

- The Questions and Answers document on the exemption from EU batch release testing for ATMPs imported into the European Union was discussed. The document will be published on the EMA website.

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- The revised procedure for the consultation of environmental competent authorities on genetically-modified organisms with respect to environmental risk assessment for medicinal products for human use submitted for centralised marketing authorisation was presented and agreed by CAT.

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	7	42

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	27	372
Adopted	12	27	12	16	23	29	31	87	49	43	37	366

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	33	358

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	7					59
Granted	8	6	6	4					24

Upcoming meetings following the July 2019 CAT meeting

- The 118th meeting of the CAT will be held on 11 – 13 September 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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