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Human Medicines Division

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

April 2020 meeting

The Committee for Advanced Therapies (CAT) held its 125th meeting on 22 – 24 April 2020.

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[1]

Further to consultation with the European Commission, the CAT finalised 2 scientific recommendations on the classification of advanced therapy medicinal products.

The following product was classified as gene therapy medicinal product:

- Autologous CD34+ cells genetically modified with a lentiviral vector expressing IL2RG gene, intended for the treatment of X-linked severe combined immunodeficiency.

The following product was classified as advanced therapy medicinal product[2]:

- Allogeneic viable Wharton's jelly derived mesenchymal stem cells, intended for the treatment of adrenomyeloneuropathy.

Organisational matters

CAT adopted the public statement on the use of unproven cell therapies. In this statement, which was published on the EMA website on 28 April 2020, CAT warns patients and the general public against using such unregulated cell-based therapies which may not be safe or effective.

[1] It is stressed that the scientific recommendation on advanced therapy classification does not amount to any endorsement of the plausibility of the product, including the mode of action or therapeutic indication(s) claimed by the applicant.
[2] CAT was unable to consider if these products meet the definition of somatic cell therapy or tissue engineering product due to shortcomings in the information provided regarding the claimed mode of action.

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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							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Submitted MAAs	14	1	4	3	2	2	26
Positive draft Opinion	7 [i]	2	2	3	1	1	16*
Negative draft opinions	4 [i,ii,iii]	0	0	0	0	0	4
Withdrawals	4 [ii]	0	0	1	1 [iv]	0	6
Ongoing MAAs							4

*** Corresponding to 15 ATMPs**

[i] One negative draft opinion and two positive draft opinions for Glybera
[ii] Negative draft opinion and withdrawal for Cerepro
[iii] Two negative draft opinion for Heparesc
[iv] Luxceptar

Variations (Type II) for authorised ATMP							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Positive opinion	18	6	3	8	16	11	62

Scientific recommendation on advanced therapy classification							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Submitted	184	60	46	55	70	38	453
Adopted	150	87	49	43	67	32	428

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Submitted	7	2	2	1	2	0	14
Adopted	6	1	3	1	1	1	13

Scientific advice procedure for ATMPs							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Number of procedures	171	46	55	53	56	14	395

Paediatric Investigation Plans (PIP) for ATMPs							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Number of procedures	31	5	3	3	2	1	45

Prime Eligibility for ATMPs							
	2016	**2017**	**2018**	**2019**	**2020**		**Total**
Discussed	22	16	14	16	8		76
Granted	8	6	6	10	2		32

Upcoming meetings following the April 2020 CAT meeting

- The 126th meeting of the CAT will be held on 18 – 20 May 2020.

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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