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Human Medicines Division

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

July 2020 meeting

The Committee for Advanced Therapies (CAT) held its 128th meeting on 15 – 17 July 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 12 scientific recommendations on the classification of advanced therapy medicinal products.

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

- Allogeneic CD34+ enhanced cell suspension derived from umbilical cord blood, intended for the treatment of patients with inherited metabolic disorders (cerebral adrenoleukodystrophy, Hurler syndrome) where haematopoietic stem cell transplant is indicated.

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

- Aggregates of defined size of human embryonic stem cell derived insulin secreting pancreatic beta cells, encapsulated within an encapsulation device, intended for the treatment of type I diabetes mellitus.

The following product was classified as a tissue engineered product:

- Homogenate of antlerogenic stem cells, intended for the treatment of chronic obstructive pulmonary disease, bronchial asthma.

The following products were classified as advanced therapy medicinal products[2]:

[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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- Autologous adipose tissue derived mesenchymal stem cells, intended for the treatment of cartilage lesions;
- Allogeneic viable Wharton's jelly derived mesenchymal stem cells, intended for the treatment of cerebellum syndrome, encephalitis, Krabbe disease, meningitis, meningomyelocele, myelitis, osteoarthritis, spinal and bulbar muscular atrophy.

Organisational matters

- CAT discussed the revision of the Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells, which incorporated the changes made following the comments received during the public consultation.
- CAT agreed to draft, jointly with the Biologics Working Party and the GMP Inspectors Working Group, a document on the principles of GMP, against which manufacturers of viral vectors used in the production of genetically modified cells can be audited by the ATMP manufacturers.
- The European Commission representative informed CAT of the adoption by the European Parliament and the Council of the Regulation (EC) 2020/1043 on the conduct of clinical trials with and supply of medicinal products for human use containing or consisting of genetically modified organisms intended to treat or prevent coronavirus disease (COVID-19).

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	4	28
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							6

*** 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	20	71

Scientific recommendation on advanced therapy classification							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Submitted	184	60	46	55	70	54	469
Adopted	150	87	49	43	67	68	464

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	2	14

Scientific advice procedure for ATMPs							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Number of procedures	171	46	55	53	56	26	406

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	12		80
Granted	8	6	6	10	2		32

Upcoming meetings following the July 2020 CAT meeting

- The 129th meeting of the CAT will be held on 9 – 11 September 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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