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Human Medicines Division

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

March 2021 meeting

The Committee for Advanced Therapies (CAT) held its 135th meeting on 17 – 18 March 2021.

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

The following products were classified as gene therapy medicinal products:

- Two messenger ribonucleic acid (mRNA) active substances, encoding separately for Human Papilloma Virus type (HPV) 16 E6 and HPV16 E7 protein, intended for the treatment of recurrent/metastatic HPV16-positive carcinoma;
- DNA plasmid encoding human transferring gene, intended for the treatment of retinitis pigmentosa;
- Human umbilical cord MSC derived exosomes carrying recombinant hTERT mRNA and protein, hsa-miR-125b-5p, hsa-miR-125b-1-3p, AntimiR-21-5p, intended for the treatment of Acute Respiratory Distress Syndrome and Chronic Obstructive Respiratory Disease;
- Bacteriophage cocktail consisting of four CRISPR-armed phages, intended for the treatment of prophylaxis of bloodstream *E. coli* infection in neutropenic patients with haematological malignancy.

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

- Autologous dendritic cells activated against SARS-COV-2 peptides, intended for the prevention of SARS-COV-2 infection.

[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.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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The following products were classified as advanced therapy medicinal products[2]:

- Allogeneic human mesenchymal stem cells derived from Wharton's jelly, intended for the treatment of:
    - anal fistula
    - androgenic alopecia, unspecified
    - diabetic foot syndrome
    - diseases of muscles and tendons
    - Parkinson's disease;
- Allogeneic human mesenchymal stem cells derived from Wharton's jelly seeded on the dermal scaffold, intended for the treatment of skin ulcers;
- Autologous human mesenchymal stem cells derived from adipose tissue, intended for the treatment of:
    - anal fistula
    - androgenic alopecia, unspecified
    - diseases of muscles and tendons.

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

- Human amniotic membrane, allogeneic, sterile, cryomilled and lyophilized, intended for the treatment of symptoms of osteoarthritis.

Organisational matters

- CAT discussed and agreed the Question and Answer document on principles of GMP for the manufacturing of starting materials of biological origin used to transfer genetic material for the manufacturing of ATMPs.

- CAT discussed and agreed the Question and Answer document related to the assessment of similarity for ATMPs in the context of the orphan legislation.

- The Core SmPC for ATMPs containing genetically modified cells was presented.

Overview of product-related activities

The Committee discussed ongoing evaluation procedures for ATMPs and other related procedures as summarised in the following tables:

[2] CAT was unable to consider if this product meets the definition of somatic cell therapy or tissue engineering product due to shortcomings in the information provided regarding the claimed mode of action.

Initial Evaluation of Marketing Authorisation Applications (MAA) for ATMP								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted MAAs	14	1	4	3	2	8	0	31
Positive draft Opinion	7 [i]	2	2	3	1	3	0	18*
Negative draft opinions	4 [i,ii,iii]	0	0	0	0	0	0	4
Withdrawals	4 [ii]	0	0	1	1[iv]	2[v]	0	8
Ongoing MAAs								6

*** Corresponding to 17 ATMPs**

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

Variations (Type II) for authorised ATMP								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Positive opinion	18	6	3	8	16	27	9	87

Scientific recommendation on advanced therapy classification								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted	184	60	46	55	70	74	27	516
Adopted	150	87	49	43	67	87	21	504

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted	7	2	2	1	1	0	0	14
Adopted	6	1	3	1	1	2	0	14

Scientific advice procedure for ATMPs								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Number of procedures	171	46	55	53	56	61	8	450

Paediatric Investigation Plans (PIP) for ATMPs								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Number of procedures	31	5	3	3	2	1	0	45

Prime Eligibility for ATMPs							
	2016	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Discussed	22	16	14	16	23	6	97
Granted	8	6	6	10	9	1	40

Upcoming meetings following the March 2021 CAT meeting

- The 136th meeting of the CAT will be held on 14 – 16 April 2021.

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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