



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

28 May 2020
EMA/CAT/287339/2020
Human Medicines Division

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

May 2020 meeting

The Committee for Advanced Therapies (CAT) held its 126th meeting on 18 – 20 May 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 classification¹

Further to consultation with the European Commission, the CAT finalised 11 scientific recommendations on the classification of advanced therapy medicinal products.

The following product was classified as a gene therapy medicinal product:

- Recombinant adeno-associated viral vector containing the human beta-sarcoglycan gene, intended for the treatment of limb-girdle muscular dystrophy type 2E.

The following product was classified as a gene therapy medicinal product, combined ATMP:

- Gene-activated matrix based on octocalcium phosphate and a plasmid expressing the VEG-A gene, intended for various bone healing indications.

The following products were classified as advanced therapy medicinal products²:

- Allogeneic viable Wharton's jelly derived mesenchymal stem cells, intended for the treatment of drug resistant epilepsy, Behçet disease, choroideremia, foetal alcohol syndrome, frontotemporal dementia, progressive bulbar palsy, vitelliform macular degeneration.

The following products were classified as non-ATMP:

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.



- Recombinant chimeric vesicular stomatitis virus carrying the envelop glycoprotein of the visceral non-neurotropic strain of the lymphocytic choriomeningitis virus, intended for the treatment of solid tumours, including non-small cell lung carcinoma;
- Leuco-platelet enriched plasma, intended for the treatment of ulcers and chronic wounds.

Organisational matters

CAT received feedback on the [EMA initiatives](#) to support the development of medicines against 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	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	17	68

Scientific recommendation on advanced therapy classification							
	2009-2015	2016	2017	2018	2019	2020	Total
Submitted	184	60	46	55	70	50	465
Adopted	150	87	49	43	67	43	439

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	20	401

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	9	77
Granted	8	6	6	10	2	32

Upcoming meetings following the May 2020 CAT meeting

- The 127th meeting of the CAT will be held on 17 – 19 June 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 \(CAT\)](#)

Florence Deleska

Head of Meeting Secretariat

Tel.: +31 (0)88 781 7375

AdvancedTherapies@ema.europa.eu