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Human Medicines Division

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

June 2020 meeting

The Committee for Advanced Therapies (CAT) held its 127th meeting on 17 – 19 June 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 13 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Recombinant adeno-associated viral vector containing the gene for the human cyclic nucleotide gated channel subunit beta 3 (CNGB3) protein, intended for the treatment of achromatopsia caused by mutations in the CNGB3 gene;
- Genetically modified *Lactococcus lactis* strain engineered to secrete human pro-insulin and interleukin-10, intended for the treatment of clinical recent-onset diabetes mellitus;
- Autologous CD34+ cell transduced with a lentiviral vector encoding a modified gamma-globin gene, intended for the treatment of sickle cell disease and beta-thalassaemia;
- Autologous haematopoietic stem cells transduced with a lentiviral vector encoding the human alpha galactosidase gene, intended for the treatment of Fabry disease;
- Autologous haematopoietic stem cells transduced with a lentiviral vector encoding the human glucocerebrosidase gene, intended for the treatment of Gaucher disease.

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

- Allogeneic viable Wharton's jelly derived mesenchymal stem cells, intended for the treatment of COVID-19 infections, optic atrophy, ichthyosis follicularis with alopecia and photophobia

[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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(IFAP) syndrome, bone marrow transplant rejection, secondary bone marrow transplant failure/secondary graft failure, progressive supranuclear palsy, multiple system atrophy.

Organisational matters

- The CAT was informed about the European Commission's initiative related the application of the GMO legislation to medicinal products containing GMOs intended to treat or prevent coronavirus disease and the European Commission's reflection process on the future of the legislation on medicinal products.

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	3	27
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							5

*** 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	19	70

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	56	452

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	27	408

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 June 2020 CAT meeting

- The 128th meeting of the CAT will be held on 15 – 17 July 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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