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Human Medicines Division

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

September 2020 meeting

The Committee for Advanced Therapies (CAT) held its 129th meeting on 9 – 11 September 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 three 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 (serotype 8) carrying an optimised gene for human cyclic nucleotide gated channel subunit alpha 3 (CNGA3) protein, intended for the treatment of achromatopsia caused by mutations in the CNGA3 gene;
- Autologous naïve regulatory T cells transduced with a lentiviral vector encoding for a Chimeric Antigen Receptor (CAR) to recognize the HLA-A*02 antigen, intended for the prevention of immune-mediated graft rejection in HLA-A*02 mismatched renal transplantation;
- Live-attenuated, genetically modified *Mycobacterium bovis* expressing the gene coding for listeriolysin from *Listeria monocytogenes*, intended for treatment of non-muscle invasive bladder cancer.

Organisational matters

- CAT discussed the agenda of the Strategic Review and learning meeting, that will be held under the auspices of the German Presidency of the European Union. During this meeting,

[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.

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which will take place virtually on 22 October 2020, CAT will discuss with PDCO and PRAC topics of common interest.

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	5	29
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							7

*** Corresponding to 15 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 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	64	479
Adopted	150	87	49	43	67	71	467

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	1	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	42	423

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	19		87
Granted	8	6	6	10	3		33

Upcoming meetings following the September 2020 CAT meeting

- The 130th meeting of the CAT will be held on 7 – 9 October 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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