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Human Medicines Division

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

March 2020 meeting

The Committee for Advanced Therapies (CAT) held its 124th meeting on 18 – 20 March 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.

CAT recommends the granting of the marketing authorisation for Zolgensma

Zolgensma is a gene therapy medicinal product that expresses the human survival motor neuron (SMN) protein. The active substance, onasemnogene abeparvovec, is a non-replicating recombinant adeno associated virus serotype 9 (AAV9) based vector containing the cDNA of the human SMN gene. Zolgensma is indicated for the treatment of babies and young children who have a rare, serious inherited condition called spinal muscular atrophy (SMA).

Following an in-depth review of the marketing authorisation application submitted by AveXis EU Ltd., CAT concluded during its March 2020 meeting that a positive benefit risk has been demonstrated for Zolgensma. CAT adopted a positive draft opinion recommending the granting of a conditional marketing authorisation. The CHMP subsequently adopted a positive opinion for Zolgensma during its March 2020 meeting.

Further information can be found here.

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.

[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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The following products were classified as gene therapy medicinal products:

- Allogeneic CRISPR/Cas9-mediated genetically modified CAR T cells targeting CD19 antigen, intended for the treatment of CD19+ haematological malignancies;
- Allogeneic CRISPR/Cas9-mediated genetically modified CAR T cells targeting B-cell maturation antigen (BCMA), intended for the treatment of relapsed or refractory multiple myeloma.

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

- Autologous, *ex vivo* expanded, clonal neoantigen specific tumour infiltrating lymphocytes, intended for the treatment of solid tumours.

The following product was classified as a tissue engineered product:

- Human embryonic stem cell-derived otic neural progenitor cells, intended for the treatment of sensorineural hearing loss.

The following product was classified as not an ATMP:

- Micronized autologous adipose tissue particles and costal cartilage powder, intended for the treatment of cartilage defects

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

- Autologous adipose derived mesenchymal stem cell, intended for the treatment of:
    - Amyotrophic lateral sclerosis (ALS)
    - Alopecia
    - Hypertrophic scars
- Allogeneic viable Wharton's jelly derived mesenchymal stem cells, intended for the treatment of:
    - Amyotrophic lateral sclerosis (ALS)
    - Spinal cord injury, drug resistant epilepsy and hypoxia ischemia encephalopathy
    - Huntington's disease
    - Lewy body dementia
    - Secondary progressive multiple sclerosis

Organisational matters

- CAT adopted an amendment to its Rules of Procedures to enable CAT to continue its workings in a virtual emergency setting, as well as to ensure the validity of the various output decisions that are to be adopted by the CAT. The revised CAT Rules of Procedure will be published shortly.

[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.

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 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	9	60

Scientific recommendation on advanced therapy classification							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Submitted	184	60	46	55	70	25	440
Adopted	150	87	49	43	67	30	426

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	13
Adopted	6	1	3	1	1	0	12

Scientific advice procedure for ATMPs							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Number of procedures	171	46	55	53	56	8	389

Paediatric Investigation Plans (PIP) for ATMPs							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Number of procedures	31	5	3	3	2	0	44

Prime Eligibility for ATMPs							
	2016	**2017**	**2018**	**2019**	**2020**		**Total**
Discussed	22	16	14	16	4		72
Granted	8	6	6	10	2		32

Upcoming meetings following the March 2020 CAT meeting

- The 125th meeting of the CAT will be held on 22 – 24 April 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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