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Human Medicines Division

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

June 2021 meeting

The Committee for Advanced Therapies (CAT) held its 138th meeting on 16 – 18 June 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.

CAT recommends the granting of the marketing authorisations for Abecam

During its June 2021 meeting, the CAT adopted a draft opinion recommending the granting of a marketing authorisation to Abecma.

Abecma is a gene therapy medicinal product indicated for the for the treatment of relapsed and refractory multiple myeloma. The active substance of Abecma is idecabtagene vicleucel, a chimeric antigen receptor (CAR)-positive T cell therapy targeting B-cell maturation antigen (BCMA), which is expressed on the surface of normal and malignant plasma cells.

Following an in-depth review of the marketing authorisation application submitted by Celgene Europe BV., CAT concluded that a positive benefit risk has been demonstrated for Abecma. CAT adopted a positive draft opinion recommending the granting of a conditional marketing authorisation. The CHMP subsequently adopted a positive opinion for Abecma during its June 2021 meeting.

Further information on Abecma can be found here.

Scientific recommendation on advanced therapy product classification[1]

Further to consultation with the European Commission, the CAT finalised 5 scientific recommendations on the classification of advanced therapy medicinal products.

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

[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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- Proliferation arrested myelomonocytic leukemic cell line-derived cells with a mature dendritic cell phenotype, intended for the treatment of acute myeloid leukaemia.

The following products were classified as tissue engineered products:

- Allogeneic, expanded, engineered E4ORF1+ human umbilical cord endothelial (CD31+) cells, intended to treat organ vascular niche injuries caused by myeloablative, non-central nervous system penetrating high-dose chemotherapy (HDT) to prevent the development of severe regimen-related toxicities (SRRT) in patients diagnosed with aggressive systemic lymphoma;
- Allogeneic corneal endothelial cells in a confluent monolayer adhering to a cornea-shaped sheet of cross-linked collagen, intended for the treatment of corneal dysfunction.

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

- Allogeneic human Wharton's jelly derived mesenchymal stem cells, intended for the treatment of idiopathic pulmonary fibrosis (IPF), pulmonary fibrosis after COVID-19;
- Ex-vivo expanded autologous cryopreserved Wharton's Jelly derived mesenchymal stem cells, intended for the treatment of bronchopulmonary dysplasia (BPD) for preterm infants.

Organisational matters

- CAT discussed the outcome of the Strategic Review & Learning meeting (SRLM) under the Portuguese presidency of the European Union that took place on 27 May 2021.
- CAT discussed the criteria for comprehensiveness of clinical data in marketing authorisation procedures. These criteria were developed jointly with the CHMP. CAT agreed to apply these criteria for new marketing authorisation applications for ATMPs.
- CAT noted the EMA pilot for patient engagement in the assessment of marketing authorisation applications.
- Feedback was provided on the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) S12 guideline: non-clinical biodistribution studies for gene therapy products definition of gene therapy medicinal products'. This guideline will be published for a four months public consultation.

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	1	33
Positive draft Opinion	7 [i]	2	2	3	1	3	2[vi]	20*
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								5

*** Corresponding to 19 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
[vi] Skysona, Abecma

Variations (Type II) for authorised ATMP								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Positive opinion	18	6	3	8	16	27	17	95

Scientific recommendation on advanced therapy classification								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted	184	60	46	55	70	74	46	535
Adopted	150	87	49	43	67	87	37	520

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	27	469

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	8	99
Granted	8	6	6	10	9	4	43

Upcoming meetings following the June 2021 CAT meeting

- The 139th meeting of the CAT will be held on 14 – 16 July 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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