15 October 2020
EMA/CAT/547273/2010
Human Medicines Division

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

October 2020 meeting

The Committee for Advanced Therapies (CAT) held its 130th meeting on 7 – 9 October 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 authorisations for Libmeldy and Tecartus

During its October 2020 meeting, the CAT adopted two draft opinions recommending the granting of a marketing authorisation to Libmeldy and Tecartus

Libmedly is a gene therapy medicinal product consisting of autologous CD34+ cell enriched population that contains haematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector encoding the human arylsulfatase A gene. Libmeldy is indicated for the treatment of children with the 'late infantile' or 'early juvenile' forms of metachromatic leukodystrophy (MLD).

Following an in-depth review of the marketing authorisation application submitted by Orchard Therapeutics (Netherlands) B.V., CAT concluded that a positive benefit risk has been demonstrated for Libmeldy. CAT adopted a positive draft opinion recommending the granting of a marketing authorisation. The CHMP subsequently adopted a positive opinion for Libmeldy during its October 2020 meeting.

Further information on Libmeldy can be found here

Tecartus is a gene therapy medicinal product consisting of autologous T cells genetically modified *ex vivo* using a retroviral vector encoding an anti-CD19 chimeric antigen receptor (CAR). Tecartus is indicated for the treatment of relapsed or refractory Mantle Cell Lymphoma.

Following an in-depth review of the marketing authorisation application submitted by Kite Pharma EU B.V., CAT concluded that a positive benefit risk has been demonstrated for Tecartus. CAT adopted a

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

positive draft opinion recommending the granting of a conditional marketing authorisation. The CHMP subsequently adopted a positive opinion for Tecartus during its October 2020 meeting.

Further information on Tecartus can be found here

Scientific recommendation on advanced therapy product classification[1]

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

The following products were classified as gene therapy medicinal products:

- Allogeneic CRISPR/Cas9-mediated genetically modified chimeric antigen receptor (CAR) T-cells targeting CD70, intended for the treatment of renal cell carcinoma and haematological malignancies;
- Autologous human T cells genetically modified *ex vivo* with a lentiviral vector encoding a chimeric antigen receptor (CAR) directed against G protein-coupled receptor family C group 5 member D, intended for the treatment of patients with relapsed or refractory multiple myeloma;
- Recombinant adeno-associated viral vector serotype 9 encoding a codon-optimised human neuronal ceroid lipofuscinosis-7 (CLN7) transgene, intended for the treatment of neuronal ceroid lipofuscinosis type 7;
- Recombinant adeno-associated viral vector serotype 9 encoding human ATP7B, intended for the treatment of Wilson disease.

The following products were classified as somatic cell therapy medicinal products:

- Umbilical cord-derived expanded CD34+ cells and umbilical cord-derived non-expanded CD34- cells, intended for the treatment in haematopoietic stem cell transplantation;
- Autologous human endometrial stem cells, intended for treatment of stem cell therapy for ovarian insufficiency includes diminished ovarian reserve, premature ovarian failure, primary ovarian insufficiency and poor ovarian response;
- Irradiated allogeneic induced-pluripotent stem cells expressing pluripotent genes and cancer-specific embryonic neo-antigens, intended for the treatment malignant solid tumours including all epithelial cancers in sub-group type harbouring a stemness mesenchymal-like signature and haematopoietic malignancies;
- Autologous regulatory T lymphocytes with the marker profile of CD3+, CD4+, CD25high, CD127-, FoxP3+, intended for the treatment and prevention of progression of, multiple sclerosis.

Organisational matters

CAT adopted the revision of the Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells. The guideline has been updated to take account of the evolution of science and regulatory experience and includes the scientific principles and

[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.

guidance for recent developments in the area of genetically modified cells, such as genome editing and CAR-T cells.

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	7	31
Positive draft Opinion	7 [i]	2	2	3	1	3	18*
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 17 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	21	72

Scientific recommendation on advanced therapy classification							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Submitted	184	60	46	55	70	68	483
Adopted	150	87	49	43	67	79	475

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	47	428

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	21		89
Granted	8	6	6	10	8		38

Upcoming meetings following the October 2020 CAT meeting

- The Strategic Review and Learning meeting, organised under the German Presidency of the European Union, will take place virtually on 22 October 2020.
- The 131st meeting of the CAT will be held on 4 – 6 November 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