30 January 2020
EMA/CAT/54845/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

January 2020 meeting

The Committee for Advanced Therapies (CAT) held its 122nd meeting on 22 – 24 January 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.

Election of CAT chairperson

The CAT re-elected Martina Schüßler-Lenz as its chair for a second three-year mandate.

Martina Schüßler-Lenz is a medical doctor, certified in internal medicine. She is currently the Deputy Head of the Advanced Therapy Medicinal Products Section at the Paul-Ehrlich Institute (PEI) in Langen, Germany, and has been chair of the CAT since February 2017. Further information can be found here.

CAT will elect a vice-chairperson at the February 2020 CAT meeting.

Scientific recommendation on advanced therapy product classification[1]

Further to consultation with the European Commission, the CAT finalised 10 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as tissue engineered products:

- Autologous chondrocytes in suspension, intended for the treatment of knee joint cartilage lesion;
- Autologous chondrocytes on a fibrinogen carrier, intended for the treatment of knee joint cartilage lesion.

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:

[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.

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
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- Adrenoleukodystrophy;
- Encephalopathy;
- Epilepsy;
- Osteoarthritis;
- Polyneuropathy;
- Spinal muscular atrophy;
- Spinocerebellar ataxia.

The following product was classified as not an ATMP:

- Modulated immune cells, intended for prophylactic use in solid organ transplantation and therapeutic use in autoimmune disease.

Organisational matters

- CAT adopted its Work Plan for 2020. The workplan will be published shortly.

Concerns over unregulated ATMPs

In the light of recent press articles, publications and statements on the websites of FDA and Health Canada, CAT discussed if an update of the EMA public statement on concerns over unregulated medicinal product containing stem cells (published in 2010) is needed. CAT considered that the statement is still valid and is applicable to all types of ATMPs.

Consultation of GMO authorities on the ERA for genetically modified organisms in medicinal products

The new, streamlined procedure for the consultation of environmental competent authorities on genetically modified organisms (GMO) on the environmental risk assessment (ERA) for medicinal products was presented to CAT. An update of section 3.4.2 (related to the ERA) of the European Medicines Agency pre-authorisation advice for users of the centralised procedure will be published shortly.

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	0	15*
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 14 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

Variations (Type II) for authorised ATMP							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Positive opinion	18	6	3	8	16	1	52

Scientific recommendation on advanced therapy classification							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Submitted	184	60	46	55	70	11	426
Adopted	150	87	49	43	67	10	406

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	2	383

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	2		70
Granted	8	6	6	10	0		30

Upcoming meetings following the January 2020 CAT meeting

- The 123nd meeting of the CAT will be held on 19 – 21 February 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)

Thorsten Olski
Head of Scientific Committees Secretariat
Tel.: +31 (0)88 781 7684
AdvancedTherapies@ema.europa.eu