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Human Medicines Division

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

July 2021 meeting

The Committee for Advanced Therapies (CAT) held its 139th meeting on 14 – 16 July 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 concluded positively on the referral for Zynteglo

During its July 2021 meeting, the CAT concluded that the benefit risk balance of Zynteglo, which is indicated for the treatment of beta thalassaemia, is unchanged.

The CAT based its conclusion on the assessment by the Pharmacovigilance and Risk Assessment Committee (PRAC), who concluded that there is no evidence of a link between the treatment with an investigational medicines (bb1111) in a clinical trial for sickle cell disease and the appearance of acute myeloid leukaemia (AML). Although there have been no reports of AML with Zynteglo, both medicines use the same viral vector and there was a concern that the vector may be implicated in the development of the cancer (insertional oncogenesis). Experts from the CAT were involved in the assessment of this signal by the PRAC.

CAT adopted a positive draft opinion on the Article 20 referral procedure for Zynteglo. The CAT draft opinion is forwarded to the Committee for Medicinal Products for Human Use (CHMP) for adoption. More information on this referral can be found here.

Scientific recommendation on advanced therapy product classification[1]

Further to consultation with the European Commission, the CAT finalised 3 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Nanoparticle consisting of non-pseudotyped (bald) lentiviral vector encoding for a CD19 CAR, encapsulated, intended for the treatment of CD19+ B-cell malignancy;

[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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- Autologous T cells genetically modified *ex vivo* using a synthetic chromosome encoding CCR6, IL-2, a truncated version of CD34 and two independent inducible safety switches, intended for treatment of patients with solid tumours for which a draining lymph node can be identified. Initially the product will be developed for colon cancer and urinary bladder cancer.

The following product was classified as advanced therapy medicinal product[2]:

- Allogeneic human Wharton's jelly derived mesenchymal stem cells, intended for the treatment of atherosclerosis of the arteries of the lower extremities.

Organisational matters

- CAT had an initial discussion on the agenda for the Strategic Review & Learning meeting (SRLM) under the Slovenian presidency of the European Union that will take place on 21 October 2021.
- CAT discussed the ATMP section of the draft reflection paper on criteria for the evaluation of new active substance (NAS) status of biological substances. The reflection paper is scheduled to be release for public consultation in October 2021.
- CAT agreed to organise a Stakeholder meeting in the last week of October. At the September meeting, CAT will discuss the topics for the agenda and identify the exact date and time. At that point, the CAT stakeholders will be asked to submit proposals for further agenda points.

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**	**2021**	**Total**
Submitted MAAs	14	1	4	3	2	8	2	34
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								6

*** 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

[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.

[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	40	523

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	34	476

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**

Prime Eligibility for ATMPs							
Discussed	22	16	14	16	23	10	101
Granted	8	6	6	10	9	4	43

Upcoming meetings following the July 2021 CAT meeting

- The 140th meeting of the CAT will be held on 8 – 10 September 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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