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Human Medicines Division

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

October 2021 meeting

The Committee for Advanced Therapies (CAT) held its 141st meeting on 6 – 8 October 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.

Impact of tocilizumab potential shortages on the use of CAR-T cell product in EU

To ensure safe use of CAR-T cell products, the marketing authorisation holders (MAHs) have to ensure that the treatment centres have at least 1 dose of tocilizumab (Roactremra) for each patient as cytokine release syndrome (CRS) management medication prior to treating patients and have access to an additional dose of tocilizumab within 8 hours of each previous dose.

Tocilizumab is also used to treat patients with severe COVID-19 and therefore this has put a stress on the production and supply of tocilizumab, resulting in a shortage.

In view of this unanticipated global challenge, CAT discussed the conditions of use for CAR-T cell-based therapies, to avoid the possible disruption in the treatment of patients with CAR-T cell products.

CAT recommended that MAHs of CAR-T cell-based therapies authorised in EU should submit a type II variation to amend the Product Information and the conditions of the marketing authorisation so that CAR-T cell-based therapies can be used in the EU/EEA also during confirmed tocilizumab shortages. Treating physicians will have to consider alternative suitable treatments for CRS and ensure that such treatments are available to manage this adverse reaction associated with the use of CAR-T cell-based therapies.

Scientific recommendation on advanced therapy product classification[1]

Further to consultation with the European Commission, the CAT finalised 8 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:

- Optimised DNA encoding the sequence of interest COL7A1, intended for the treatment of dystrophic epidermolysis bullosa;
- Recombinant adeno-associated virus serotype HSC 15 (rAAVHSC15) expressing human iduronate-2-sulfatase, intended for the treatment of mucopolysaccharidosis type II (known as Hunter syndrome).

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

- Isolated CD31+ cells, intended for the treatment of erectile dysfunction.

The following product was classified as a tissue engineered product:

- Extracellular matrix and non-viable osteogenic cells derived from human adipose-derived stem cells, associated with hydroxyapatite/beta-tricalcium phosphate (HA/βTCP) particles, intended to stimulate bone regeneration in pathological hypoxic and/or necrotic bone conditions.

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

- Autologous adipose derived mesenchymal stem/stromal cells, intended for the treatment of amyotropthic lateral sclerosis.

The following product does not fulfil the definition of an advanced therapy medicinal product:

- Point-of-care skin cell isolation kit.

Organisational matters

- CAT finalised the agenda of the CAT Stakeholder meeting that will take place on 26 October 2021 from 14.00 to 18.00 on basis of topics proposed by the stakeholders. The second part will be on on Real World Data (RWD) in regulatory decision making of ATMPs.
- CAT discussed its involvement in the revision of the pharmaceutical legislation and identified priority topics where appointed CAT members will be involved in the preparation of concept paper that will guide DG SANTE in identifying new approaches or practical solutions to be reflected in revision of the Directive and Regulation.

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	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
[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	24	102

Scientific recommendation on advanced therapy classification								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted	184	60	46	55	70	74	55	544
Adopted	150	87	49	43	67	87	56	539

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

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs								
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	52	494

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	10	101
Granted	8	6	6	10	9	5	44

Upcoming meetings following the October 2021 CAT meeting

- The joint CAT-CHMP Strategic Review & Learning meeting under the Slovenian presidency will take place on 21 October 2021.
- The CAT-Stakeholders meeting will take place on 26 October 2021.
- The 142nd meeting of the CAT will be held on 3-5 November 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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