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Human Medicines Division

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

September 2021 meeting

The Committee for Advanced Therapies (CAT) held its 140th meeting on 8 – 10 September 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.

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.

The following products were classified as gene therapy medicinal products:

- Recombinant serotype 9 adeno-associated virus encoding a codon-optimised human galactosylceramidase transgene, intended for the treatment of Krabbe disease;
- HEK293 cells transfected with a lentiviral vector to express Wilms' tumour antigen (WT1) and the antigen presenting molecule, cluster of differentiation 1d, intended for the treatment of WT1-expressing tumours.

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

- Autologous population of selected renal cells, intended for the treatment of chronic kidney disease;
- Allogeneic natural killer cells armed with anti-CD20 monoclonal antibody, intended for the treatment of B-Cell Non-Hodgkin lymphoma.

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

- Autologous adipose mesenchymal stem cells, intended for cartilage defects of degenerative origin and for the treatment of osteoarthritis;
- 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 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.

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- rheumatoid arthritis;
- systemic lupus erythematosus;
- systemic sclerosis.

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

- Minimally manipulated autologous pancreatic islets, intended for the treatment of chronic pancreatitis and recurrent acute pancreatitis immediately following pancreatectomy;
- Ribonucleoprotein (RNP), a complex of Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) Cas 9 and sgRNA, delivered by a novel synthetic non-viral vector, for the excision of exon 80 of the human COL7A1 gene, intended for the treatment of recessive dystrophic epidermolysis bullosa.

Organisational matters

- CAT discussed 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 agenda of the CAT Stakeholder meeting that will take place on 26 October 2021 from 14.00 to 18.00. The first part of the agenda will be based on suggestion by the stakeholders, the second part will be on on Real World Data (RWD) in regulatory decision making of ATMPs. The agenda will be finalised at the October CAT meeting.
- CAT provided comments to the WHO white paper: A Regulatory Framework for Cells, Tissues, and Gene Therapies.
- CAT noted the information on the relaunch of the face-to-face scientific committee meetings.

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
[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	20	98

Scientific recommendation on advanced therapy classification								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted	184	60	46	55	70	74	51	540
Adopted	150	87	49	43	67	87	50	533

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	46	488

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 September 2021 CAT meeting

- The 141st meeting of the CAT will be held on 6 – 8 October 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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