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Human Medicines Division

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

December 2020 meeting

The Committee for Advanced Therapies (CAT) held its 132nd meeting on 2 – 3 December 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.

Withdrawal of the marketing authorisation application for autologous human chondrocytes, in vitro expanded

CAT noted the withdrawal of the marketing authorisation application of autologous human chondrocytes, in vitro expanded, which was intended for the repair of cartilage defects of the knee joint.

Scientific recommendation on advanced therapy product classification[1]

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

The following products were classified as gene therapy medicinal products:

- Autologous CD34+ cells transduced with a lentiviral vector encoding human cystinosin, intended for the treatment of cystinosis;
- Delolimogene mupadenorepvec (oncolytic adenovirus expressing two immunostimulatory transgenes (TMZ-CD40L and 4-1BBL)), intended for the treatment of cancer.

The following products were classified as somatic cell 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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- Autologous tumour-infiltrating lymphocytes, intended for the treatment of advanced melanoma;
- Allogeneic cord tissue-derived mesenchymal stromal cells, intended for the treatment of inflammatory and immunological diseases (acute graft-versus-host disease, systemic lupus erythematosus, systemic sclerosis, acute respiratory distress syndrome).

The following products were classified as tissue engineered products and combined ATMPs:

- 3D bio-printed bionic pancreas composed of islets of Langerhans and non-viable printable porcine-derived matrix plus porcine–derived decellularised blood vessel, intended for the treatment of late-chronic pancreatitis;
- 3D bio-printed bionic pancreas composed of insulin- and glucagon-releasing cells and non-viable printable porcine-derived matrix plus porcine–derived decellularised blood vessel, intended for the treatment of brittle diabetes mellitus type I.

Organisational matters

- CAT discussed their work plan for 2021. Adoption of the CAT workplan will take place in the January 2021 CAT meeting.
- CAT received information on the ongoing review of Working Parties.
- An exchange took place between CAT and the European Commission on the revision of the EU legislation on blood, tissues and 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	8	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]	2[v]	8
Ongoing MAAs							6

*** 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
[v] Roctavian, Autologous human chondrocytes, in vitro expanded

Variations (Type II) for authorised ATMP							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Positive opinion	18	6	3	8	16	27	78

Scientific recommendation on advanced therapy classification							
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**Total**
Submitted	184	60	46	55	70	74	489
Adopted	150	87	49	43	67	87	483

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	62	443

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	23		91
Granted	8	6	6	10	8		38

Upcoming meetings following the December 2020 CAT meeting

- The 133rd meeting of the CAT will be held on 20 – 22 January 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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