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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

July 2018 meeting

The Committee for Advanced Therapies (CAT) held its 106th CAT meeting on 18 – 20 July 2018.

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

Further to consultation with the European Commission, the CAT finalised 7 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as tissue engineered products:

- Homogenate of antlerogenic stem cells, intended for the treatment of Recurrent Corneal Erosion Syndrome.
- Homogenate of antlerogenic stem cells, intended as treatment support in spinal cord injuries.
- Dystrophin expressing chimeric cells obtained by *ex vivo* fusion of defective myoblasts from a Duchenne Muscular Dystrophy patient with normal myoblasts, intended for the treatment of Duchenne Muscular Dystrophy.
- Dystrophin expressing chimeric cells obtained by *ex vivo* fusion of allogeneic human myoblasts, intended for the treatment of Duchenne Muscular Dystrophy.

The following products were classified as gene therapy medicinal products:

- Autologous human T-cells genetically engineered to express a chimeric antigen receptor for B-cell maturation antigen, intended for the treatment of relapsed or refractory multiple myeloma.
- Codon-optimized human ornithine transcarbamylase encoding messenger ribonucleic acid, intended for the treatment of ornithine transcarbamylase deficiency.
- Recombinant adeno-associated viral vector encoding the human iduronate-2-sulfatase gene, intended for the treatment of mucopolysaccharidosis type II (Hunter Syndrome).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Withdrawal of an ongoing marketing authorisation application

CAT noted the withdrawal of the marketing authorisation application for Raligize (axalimogene filolisbac), intended for the treatment of cervical cancer. In its letter notifying the Agency of the withdrawal of the application, the company stated that the withdrawal was due to initial concerns expressed by the CAT that the data from the main study would not be sufficient to support the approval of the medicine.

Organisational matters

- CAT adopted the revision of the Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells. The guideline will be published on the EMA website shortly, for public consultation for a period of 12 months.
- CAT noted the documents on the environmental assessment of gene therapy medicinal products in clinical trials. The documents are published on the European Commission website.

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	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	4	1	20
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	2	2	13*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	1	5
Ongoing MAAs											2

*** Corresponding to 12 ATMPs**
[i] Same product (Cerepro)
[ii] Same product (Glybera)
[iii] CAT adopted two negative draft opinions for the same product (Heparesc)

Variations (Type II) for authorised ATMP											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Positive Opinion	0	0	1	1	9	4	3	6	3	5	32

Scientific recommendation on advanced therapy classification											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Submitted	22	19	12	22	20	28	61	60	46	26	316
Adopted	12	27	12	16	23	29	31	87	49	26	312

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Submitted	1	0	0	1	3	1	1	2	2	0	11
Adopted	0	1	0	1	1	2	1	1	3	1	11

Scientific advice procedure for ATMPs											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Number of procedures	17	19	21	19	23	33	39	46	55	30	302

Paediatric Investigation Plans (PIP) for ATMPs											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Number of procedures	3	4	4	8	5	4	3	5	3	2	41

Prime Eligibility for ATMPs									
	2016	**2017**	**2018**						**Total**
Discussed	22	16	10						48
Granted	8	6	4						18

Upcoming meetings following the July 2018 CAT meeting

- The 107th meeting of the CAT will be held on 12 – 14 September 2018.

NOTE:

1. 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

2. 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.: (+44-20) 3660 7684
AdvancedTherapies@ema.europa.eu