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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

February 2019 meeting

The Committee for Advanced Therapies (CAT) held its 112th CAT meeting on 20 – 22 February 2019.

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 11 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Recombinant adeno-associated virus (serotype 5) containing the human retinal guanylate cyclase 1 (GUCY2D) gene, intended for the treatment of inherited retinal disease caused by biallelic mutations in GUCY2D, including Leber congenital amaurosis type 1 (GUCY2D-LCA);
- Recombinant adeno-associated virus (serotype 9) containing the human α-L-iduronidase (hIDUA) gene, intended for the treatment of mucopolysaccharidosis type I;
- Recombinant adeno-associated virus (serotype rh10) containing a transgene encoding a micro ribonucleic acid (miRNA) targeting superoxide dismutase 1 (SOD1) messenger RNA (mRNA), intended for the treatment of amyotrophic lateral sclerosis (ALS) due to mutations in SOD1 gene.

The following products were classified as tissue engineered products:

- Viable autologous adipose-derived regenerative cells combined with whole lipoaspirate, intended for the treatment of progressive hemifacial atrophy (Parry-Romberg syndrome);
- Whole lipoaspirate containing viable autologous adipose-derived regenerative cells, intended for the treatment of burn scars;
- Viable autologous adipose-derived regenerative cells, intended for the treatment of burn scars;

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

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- Autologous cord blood nucleated cells, intended for the treatment of paediatric brain damage, hypoxic-ischemic encephalopathy, cerebral palsy.

The following product was classified as a tissue engineered product and a combined ATMP:

- Cultured autologous adipose-derived stem cells, intended for the treatment of urinary diversion in patients requiring radical cystectomy for the treatment of bladder cancer.

The following product was classified as not an ATMP:

- Whole lipoaspirate containing viable autologous adipose-derived regenerative cells, intended for the treatment of progressive hemifacial atrophy (Parry-Romberg syndrome).

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**	**2019**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	4	3	0	22
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	2	3	0	14*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	1	0	5
Ongoing MAAs												3

*** Corresponding to 13 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**	**2019**	**Total**
Positive opinion	0	0	1	1	9	4	3	6	3	8	1	36

Scientific recommendation on advanced therapy classification												
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**2019**	**Total**
Submitted	22	19	12	22	20	28	61	60	46	55	11	356
Adopted	12	27	12	16	23	29	31	87	49	43	15	344

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**	**2019**	**Total**
Submitted	1	0	0	1	3	1	1	2	2	1	0	12
Adopted	0	1	0	1	1	2	1	1	3	1	0	11

Scientific advice procedure for ATMPs												
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**2019**	**Total**
Number of procedures	17	19	21	19	23	33	39	46	55	53	9	334

Paediatric Investigation Plans (PIP) for ATMPs												
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**2019**	**Total**
Number of procedures	3	4	4	8	5	4	3	5	3	3	0	42

Prime Eligibility for ATMPs									
	2016	**2017**	**2018**	**2019**					**Total**
Discussed	22	16	14	1					53
Granted	8	6	6	0					20

Upcoming meetings following the February 2019 CAT meeting

- The 113th meeting of the CAT will be held on 20 – 22 March 2019.

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