



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

### November 2017 meeting

The Committee for Advanced Therapies (CAT) held its 98<sup>th</sup> CAT meeting on 30 - 31 October 2017.

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 product was classified as a gene therapy medicinal product:

- Adeno-associated virus vector serotype 2/1 encoding human  $\beta$ -hexosaminidase alpha & beta subunits, intended for the treatment of Tay-Sachs disease & Sandhoff disease monosialic ganglioside 2 gangliosidosis.
- Adeno-associated virus vector serotype 8 expressing human low-density lipoprotein receptor, intended for the treatment of hypercholesterolaemia caused by homozygous mutations in the low density lipoprotein receptor (LDLR) gene.

The following products were classified as tissue engineered products:

- Skin substitute composed of an epidermal layer of fully-stratified human keratinocytes and a collagen-rich dermal equivalent containing human dermal fibroblasts, intended for the treatment of patients with acute complex skin loss.
- Freshly isolated CD34+ cells, intended to contribute to the regeneration of soft and hard tissues of temporomandibular joints.
- Freshly isolated autologous dental pulp stem cells, intended for the regeneration of soft and hard tissues of temporomandibular joints.
- Cultured dental pulp stem cells, intended for the regeneration of soft and hard tissues of temporomandibular joints.



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

- Allogeneic cardiopoietic cells derived from adipose tissue derived stem cells, intended to help post-myocardial infarction patients in restoring cardiac function to repair the underlying myocardium damage.

## Organisational matters

- CAT discussed the outcome of the Expert meeting on Genome editing that took place on 18 October 2017. Further discussion on this topic is scheduled during the upcoming CAT Strategic Review and Learning meeting in Estonia.

## 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	Total
Submitted MAAs	3	1	2	3	2	2	1	1	3	18
Positive draft Opinion	1	0	1 <sup>ii</sup>	1 <sup>ii</sup>	2	1	1	2	1	10*
Negative draft opinions	1 <sup>i</sup>	0	1 <sup>ii</sup>	0	0	0	2 <sup>iii</sup>	0	0	4
Withdrawals	1	1 <sup>i</sup>	0	0	2	0	0	0	0	4
Ongoing MAAs										4

\* Corresponding to 9 ATMPs

<sup>i</sup> Same product (Cerepro)

<sup>ii</sup> Same product (Glybera)

<sup>iii</sup> 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	Total
Positive Opinion	0	0	1	1	9	4	3	6	3	27

Scientific recommendation on advanced therapy classification										
	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Submitted	22	19	12	22	20	28	61	60	44	288
Adopted	12	27	12	16	23	29	31	87	47	284

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

	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Submitted	1	0	0	1	3	1	1	2	1	10
Adopted	0	1	0	1	1	2	1	1	3	10

### Scientific advice procedure for ATMPs

	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Number of procedures	17	19	21	19	23	33	39	46	48	265

### Paediatric Investigation Plans (PIP) for ATMPs

	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Number of procedures	3	4	4	8	5	4	3	5	3	39

### Prime Eligibility for ATMPs

	2016	2017								Total
Discussed	22	15								37
Granted	8	5								13

### Upcoming meetings following the November 2017 CAT meeting

- The CAT Strategic Review & Learning meeting will take place in Tallinn, Estonia on 15-17 November 2017 under the auspices of the Estonian Presidency of the Council of the European Union.
- The 99<sup>th</sup> meeting of the CAT will be held on 6 – 8 December 2017.

#### 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](mailto:AdvancedTherapies@ema.europa.eu)