



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

### September 2017 meeting

The Committee for Advanced Therapies (CAT) held its 96<sup>th</sup> CAT meeting on 6 – 8 September 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.

### **CAT expert meeting on Adeno-associated viral vector (AAV)-based gene therapies**

On 6 September 2017, CAT members met with invited experts to discuss various scientific aspects related to AAV-based gene therapy medicinal products. The topics discussed included: clinical grade manufacture, virus-host interactions in animals and humans, immunity, persistence, toxicity and the use of AAV-based medicines in various indications such as haemophilia, ophthalmology, muscle / skeletal disorders and neurological/metabolic diseases.

### **Scientific recommendation on advanced therapy product classification**

Further to consultation with the European Commission, the CAT finalised 1 scientific recommendation on the classification of advanced therapy medicinal products.

The following product was classified as a somatic cell therapy medicinal product:

- Autologous adipose tissue derived mesenchymal stem cells intended for the treatment of chronic wounds.

### **Organisational matters**

- CAT discussed the status of the 2017 CAT work plan topics and reflected on activities for inclusion in the 2018 CAT work plan.
- CAT identified topics for discussion at the CAT Strategic Review and Learning meeting that will take place on 16 – 17 November 2017 in Estonia under the auspices of the Estonian Presidency of the Council of the European Union.



## 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	40	284
Adopted	12	27	12	16	23	29	31	87	35	272

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	41	258

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	2	38

Prime Eligibility for ATMPs										
	2016	2017								Total
Discussed	22	13								35
Granted	8	5								13

## Upcoming meetings following the September 2017 CAT meeting

The 97<sup>th</sup> meeting of the CAT will be held on 4 – 6 October 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 \(CAT\)](#)

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