



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

14 September 2016  
EMA/CAT/612653/2016  
Procedure Management and Committees Support Division

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

### September 2016 meeting

The Committee for Advanced Therapies (CAT) held its 85<sup>th</sup> CAT meeting on 8 – 9 September 2016.

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

The following products were classified as gene therapy medicinal products:

- Adeno-associated viral vector serotype 8 encoding the human glucose-6-phosphatase gene, intended for the treatment of Glycogen storage disease type Ia (von Gierke disease).
- Adeno-associated viral vector serotype 2 encoding the human aromatic L-amino acid decarboxylase gene, intended for the treatment of Parkinson's disease.

The following products were classified as tissue engineered products:

- *Ex-vivo* cultured and expanded human cord blood progenitor cells, intended for the treatment of patients undergoing hematopoietic stem cell transplantation.
- Heterologous human adult liver-derived progenitor cells, intended for the treatment of fibro-inflammatory liver diseases.

### CAT workshop on cell-based cancer immunotherapies

Committee members were asked to disseminate the information on the [CAT workshop on cell-based cancer immunotherapies](#) (to take place at EMA on 15 – 16 November 2016) at their national agencies and to universities and hospitals in their member state.



## 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	Total
Submitted MAAs	3	1	2	3	2	2	1	1	15
Positive draft Opinion	1	0	1 <sup>ii</sup>	1 <sup>ii</sup>	2	1	1	2	9
Negative draft opinions	1 <sup>i</sup>	0	1 <sup>ii</sup>	0	0	0	2 <sup>*</sup>	0	4
Withdrawals	1	1 <sup>i</sup>	0	0	2	0	0	0	4
Ongoing MAAs									2

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

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

<sup>\*</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	Total
Positive draft Opinion	0	0	1	1	9	4	3	4	22

Scientific recommendation on advanced therapy classification									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Submitted	22	19	12	22	20	28	61	52	236
Adopted	12	27	12	16	23	29	31	74	224

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Submitted	1	0	0	1	3	1	1	2	9
Adopted	0	1	0	1	1	2	1	1	7

Scientific advice procedure for ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Discussed*	25	30	36	31	36	48	63	49	318
Number of procedures	17	19	21	19	23	33	39	39	210

\* Scientific advices for ATMPs are discussed by the CAT once or twice during the procedure

Paediatric Investigation Plans (PIP) for ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Discussed*	4	7	6	9	7	7	3	4	47

\* PIPs for ATMPs are discussed by the CAT once or twice during the procedure

Prime Eligibility for ATMPs									
	2016								Total
Discussed	15								15
Granted	4								4

## Upcoming meetings following the September 2016 CAT meeting

The 86<sup>th</sup> meeting of the CAT will be held on 6 – 7 October 2016.

### 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 \(CAT\)](#)

### Nikolaus Križ

Head of Committees Secretariat Service

Tel.: (+44-20) 3660 7016

Fax: (+44-20) 3660 5520

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