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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

### January 2019 meeting

The Committee for Advanced Therapies (CAT) held its 111<sup>th</sup> CAT meeting on 23 – 25 January 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 4 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Bacteriophage capsid containing deoxyribonucleic acid encoding a ribonucleic acid (RNA)-guided nucleases and associated RNA guides, targeting shiga-toxin genes, intended for the treatment of shiga-toxin producing *E.coli* infections.
- Autologous CD34+ haematopoietic cells transduced with a lentiviral vector encoding the CD18  $\beta$ -subunit of human  $\beta 2$  integrin, intended for the treatment of severe leukocyte adhesion deficiency type I.

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

- Allogeneic expanded natural killer cells, intended for the treatment of multiple myeloma.

The following product was classified as a tissue engineered product:

- Allogeneic cultured postnatal thymus tissue-derived product, intended for immune reconstitution in patients with congenital athymia.

### **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 <sup>ii</sup>	1 <sup>ii</sup>	2	1	1	2	2	3	0	14*
Negative draft opinions	1 <sup>i</sup>	0	1 <sup>ii</sup>	0	0	0	2 <sup>iii</sup>	0	0	0	0	4
Withdrawals	1	1 <sup>i</sup>	0	0	2	0	0	0	0	1	0	5
Ongoing MAAs												3

\* Corresponding to 13 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	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	8	353
Adopted	12	27	12	16	23	29	31	87	49	43	4	333

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	4	329

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	0					52
Granted	8	6	6	0					20

### Upcoming meetings following the January 2019 CAT meeting

- The 112<sup>th</sup> meeting of the CAT will be held on 20 – 22 February 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 \(CAT\)](#)

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