



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

### February 2020 meeting

The Committee for Advanced Therapies (CAT) held its 123<sup>rd</sup> meeting on 19 – 21 February 2020.

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.

### Election of CAT vice-chairperson

The CAT re-elected Ilona Reischl as its vice-chair for a second three-year mandate.

### Scientific recommendation on advanced therapy product classification<sup>1</sup>

Further to consultation with the European Commission, the CAT finalised seven 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 5 encoding for the human Retinitis Pigmentosa GTPase Regular (RPGR) gene, intended for the treatment of X-linked retinitis pigmentosa due to defects in RPGR.
- Adeno-associated viral vector serotype 9 encoding for a codon-optimised human aspartylglucosaminidase (AGA) gene, intended for the treatment of aspartylglucosaminuria due to a loss-of-function mutation in the AGA gene.

The following products were classified as advanced therapy medicinal products<sup>2</sup>:

- Allogeneic viable Wharton's jelly derived mesenchymal stem cells, intended for the treatment of:

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<sup>1</sup> It is stressed that the scientific recommendation on advanced therapy classification does not amount to any endorsement of the plausibility of the product, including the mode of action or therapeutic indication(s) claimed by the applicant.

<sup>2</sup> CAT was unable to consider if these products meet the definition of somatic cell therapy or tissue engineering product due to shortcomings in the information provided regarding the claimed mode of action.



- Age-related macular degeneration
- Bone non-union
- Chorioretinal disorders
- Epidermolysis bullosa
- Hypoxic-ischemic encephalopathy

## 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							
	2009-2015	2016	2017	2018	2019	2020	Total
Submitted MAAs	14	1	4	3	2	2	26
Positive draft Opinion	7 <sup>i</sup>	2	2	3	1	0	15*
Negative draft opinions	4 <sup>i,ii,iii</sup>	0	0	0	0	0	4
Withdrawals	4 <sup>ii</sup>	0	0	1	1 <sup>iv</sup>	0	6
Ongoing MAAs							5

**\* Corresponding to 14 ATMPs**

<sup>i</sup> One negative draft opinion and two positive draft opinions for the Glybera

<sup>ii</sup> Negative draft opinion and withdrawal for the Cerepro

<sup>iii</sup> Two negative draft opinion for Heparesc

<sup>iv</sup> Luxceptar

Variations (Type II) for authorised ATMP							
	2009-2015	2016	2017	2018	2019	2020	Total
Positive opinion	18	6	3	8	16	6	57

Scientific recommendation on advanced therapy classification							
	2009-2015	2016	2017	2018	2019	2020	Total
Submitted	184	60	46	55	70	14	429
Adopted	150	87	49	43	67	17	413

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

	2009-2015	2016	2017	2018	2019	2020	Total
Submitted	7	2	2	1	1	0	13
Adopted	6	1	3	1	1	0	12

### Scientific advice procedure for ATMPs

	2009-2015	2016	2017	2018	2019	2020	Total
Number of procedures	171	46	55	53	56	5	386

### Paediatric Investigation Plans (PIP) for ATMPs

	2009-2015	2016	2017	2018	2019	2020	Total
Number of procedures	31	5	3	3	2	0	44

### Prime Eligibility for ATMPs

	2016	2017	2018	2019	2020	Total
Discussed	22	16	14	16	3	71
Granted	8	6	6	8	1	31

## Upcoming meetings following the February 2020 CAT meeting

- The 124<sup>th</sup> meeting of the CAT will be held on 18 – 20 March 2020.

### NOTE:

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](#)

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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