



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

11 December 2019
EMA/CAT/677731/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

December 2019 meeting

The Committee for Advanced Therapies (CAT) held its 121st meeting on 4 – 6 December 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 classification¹

Further to consultation with the European Commission, the CAT finalised 16 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Recombinant adeno-associated viral vector expressing human neuronal ceroid lipofuscinosis type 6 (CLN6) gene, intended for the treatment of CLN6 disease (CLN6 Batten disease).
- Recombinant adeno-associated viral vector expressing human neuronal ceroid lipofuscinosis type 3 (CLN3) gene, intended for the treatment of CLN3 disease (CLN3 Batten disease).

The following products were classified as advanced therapy medicinal products²:

- Allogeneic viable Wharton's jelly derived mesenchymal stem cells, intended for the treatment of:
 - alopecia areata
 - pervasive developmental disorder
 - cerebral infarction
 - developmental delay
 - diabetes

¹ 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.

² 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.



- o muscular dystrophy
- o endometrial atrophy
- o multiple sclerosis
- o optic neuropathy
- o premature ovarian failure
- o retinitis pigmentosa
- o spina bifida
- o spinal cord injury
- o Stargardt's disease.

Organisational matters

- CAT adopted the [Questions and Answers](#) on comparability considerations for advanced therapy medicinal products. The document has been published on the EMA website.
- Feedback was provided on the discussions at the joint CAT-COMP-PDCO Strategic Review and Learning meeting that was held in Helsinki (Finland) on 21 – 22 November 2019 under the auspices of the Finnish presidency of the European Union.
- CAT noted a presentation on the EMA relocation to the permanent building in Amsterdam.

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 | 1 | 23 |
| Positive draft Opinion | 1 | 0 | 1 ⁱⁱ | 1 ⁱⁱ | 2 | 1 | 1 | 2 | 2 | 3 | 1 | 15* |
| Negative draft opinions | 1 ⁱ | 0 | 1 ⁱⁱ | 0 | 0 | 0 | 2 ⁱⁱⁱ | 0 | 0 | 0 | 0 | 4 |
| Withdrawals | 1 | 1 ⁱ | 0 | 0 | 2 | 0 | 0 | 0 | 0 | 1 | 1 ^{iv} | 6 |
| Ongoing MAAs | | | | | | | | | | | | 2 |

* Corresponding to 14 ATMPs

ⁱ Same product (Cerepro)

ⁱⁱ Same product (Glybera)

ⁱⁱⁱ Same product (Heparesc)

^{iv} Luxceptar

| 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 | 16 | 51 |

| 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 | 70 | 415 |
| Adopted | 12 | 27 | 12 | 16 | 23 | 29 | 31 | 87 | 49 | 43 | 67 | 396 |

| 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 | 1 | 13 |
| Adopted | 0 | 1 | 0 | 1 | 1 | 2 | 1 | 1 | 3 | 1 | 1 | 12 |

| 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 | 56 | 381 |

| 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 | 2 | 44 |

| Prime Eligibility for ATMPs | | | | | | | | | | | | |
|-----------------------------|------|------|------|------|--|--|--|--|--|--|--|-------|
| | 2016 | 2017 | 2018 | 2019 | | | | | | | | Total |
| Discussed | 22 | 16 | 14 | 16 | | | | | | | | 68 |
| Granted | 8 | 6 | 6 | 8 | | | | | | | | 28 |

Upcoming meetings following the December 2019 CAT meeting

- The 122nd meeting of the CAT will be held on 22 – 24 January 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 \(CAT\)](#)

Thorsten Olski

Head of Scientific Committees Secretariat

Tel.: +31 (0)88 781 7684

AdvancedTherapies@ema.europa.eu