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Human Medicines Division

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

December 2021 meeting

The Committee for Advanced Therapies (CAT) held its 143rd meeting on 8 – 10 December 2021.

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[1]

Further to consultation with the European Commission, the CAT finalised 4 scientific recommendations on the classification of advanced therapy medicinal products.

The following products fulfil the definition of a gene therapy medicinal product:

- CD 19 CAR T-cells transduced with lentiviral vector, intended for the treatment of adults and children with B-cell non-Hodgkin's lymphoma and acute lymphoblastic leukaemia;
- Recombinant adeno-associated virus, serotype 2, containing human ND4 codon-optimised gene, intended for the treatment of Leber's hereditary optic neuropathy.

The following products fulfil the definition of a somatic cell therapy medicinal product:

- Allogeneic adipose-derived mesenchymal stromal cells, *ex-vivo* expanded, intended for the treatment of osteoarthritis of the knee;
- Allogeneic T-cell precursors, mobilised peripheral blood-derived, *ex vivo* cultured, intended for the treatment of paediatric and adult patients undergoing partially human leucocyte antigen (HLA) compatible allogeneic haematopoietic stem cell transplantation to accelerate adaptive immunological reconstitution.

## Organisational matters

- In the light of the ongoing revision of the pharmaceutical legislation and the preparation of concept papers, a discussion took place on various topics.

[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.

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- CAT adopted the EMA guidance on companion diagnostics (CDx) consultation and CHMP/CAT assessment report CDx template. Both documents will be published for external consultation in January 2022.
- Feedback was provided to CAT on the progress in the development of the Reflection paper on criteria to be considered for the evaluation of new active substance (NAS) status of biological substances.
- CAT discussed and agreed the topics for inclusion in the CAT work plan for 2022. The 2022 work plan is scheduled for adoption at the CAT meeting in January 2022.

## 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** | **2021** | **Total** |
| Submitted MAAs | 14 | 1 | 4 | 3 | 2 | 8 | 3 | 35 |
| Positive draft Opinion | 7 [i] | 2 | 2 | 3 | 1 | 3 | 2[vi] | 20* |
| Negative draft opinions | 4 [i,ii,iii] | 0 | 0 | 0 | 0 | 0 | 0 | 4 |
| Withdrawals | 4 [ii] | 0 | 0 | 1 | 1[iv] | 2[v] | 0 | 8 |
| Ongoing MAAs | | | | | | | | 7 |

*** Corresponding to 19 ATMPs**
[i] One negative draft opinion and two positive draft opinions for the Glybera
[ii] Negative draft opinion and withdrawal for the Cerepro
[iii] Two negative draft opinions for Heparesc
[iv] Luxceptar
[v] Roctavian; Artobend
[vi] Skysona, Abecma

| Variations (Type II) for authorised ATMP | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **2009-2015** | **2016** | **2017** | **2018** | **2019** | **2020** | **2021** | **Total** |
| Positive opinion | 18 | 6 | 3 | 8 | 16 | 27 | 32 | 110 |

| Scientific recommendation on advanced therapy classification | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **2009-2015** | **2016** | **2017** | **2018** | **2019** | **2020** | **2021** | **Total** |
| Submitted | 184 | 60 | 46 | 55 | 70 | 74 | 66 | 555 |
| Adopted | 150 | 87 | 49 | 43 | 67 | 87 | 61 | 544 |

| Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **2009-2015** | **2016** | **2017** | **2018** | **2019** | **2020** | **2021** | **Total** |
| Submitted | 7 | 2 | 2 | 1 | 1 | 0 | 0 | 14 |
| Adopted | 6 | 1 | 3 | 1 | 1 | 2 | 0 | 14 |

| Scientific advice procedure for ATMPs | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **2009-2015** | **2016** | **2017** | **2018** | **2019** | **2020** | **2021** | **Total** |
| Number of procedures | 171 | 46 | 55 | 53 | 56 | 61 | 64 | 506 |

| Paediatric Investigation Plans (PIP) for ATMPs | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **2009-2015** | **2016** | **2017** | **2018** | **2019** | **2020** | **2021** | **Total** |
| Number of procedures | 31 | 5 | 3 | 3 | 2 | 1 | 0 | 45 |

| Prime Eligibility for ATMPs | | | | | | | |
|---|---|---|---|---|---|---|---|
| | **2016** | **2017** | **2018** | **2019** | **2020** | **2021** | **Total** |
| Discussed | 22 | 16 | 14 | 16 | 23 | 14 | 105 |
| Granted | 8 | 6 | 6 | 10 | 9 | 6 | 46 |

## Upcoming meetings following the December 2021 CAT meeting

- The 144th meeting of the CAT will be held on 19 – 21 January 2022.

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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