19 September 2018
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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

## September 2018 meeting

The Committee for Advanced Therapies (CAT) held its 107th CAT meeting on 12 – 14 September 2018.

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.

### CAT recommends the granting of the marketing authorisation for Luxturna

Luxturna (voretigene neparvovec) is a gene therapy medicinal product based on a recombinant adeno-associated viral vector serotype 2 expressing the human retinal pigment epithelium 65 kDa protein (hRPE65) gene. Luxturna is approved for the treatment of adults and children suffering from inherited retinal dystrophy caused by RPE65 gene mutation, a rare genetic disorder which causes vision loss and usually leads to blindness. Luxturna works by delivering a functional RPE65 gene into the cells of the retina through a single retinal injection, which restores the production pathway for the required enzyme thereby improving the patient's ability to detect light.

Following an in-depth review of the marketing authorisation application submitted by Spark Therapeutics Ireland Ltd., CAT concluded during its September 2018 meeting that a positive benefit risk has been demonstrated for Luxturna. CAT adopted a positive draft opinion recommending the granting of the marketing authorisation. The CHMP subsequently adopted a positive opinion for Luxturna during its September 2018 meeting.

Luxturna is indicated for the treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelc RPE65 mutations and who have sufficient viable retinal cells.

Further information can be found here.

### 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 product was classified as a tissue engineered product:

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- Autologous blood-derived endothelial and haematopoietic stem/progenitor cells, intended for the treatment of no-option patients with peripheral arterial disease and critical limb ischemia.

The following products were classified as gene therapy medicinal products:

- Adeno-associated viral vector serotype 2 encoding the channelrhodopsin-2-protein, intended for the treatment of retinitis pigmentosa.
- Combination of 5'-capped single stranded ribonucleic acids encoding one shared tumour-associated antigen, intended for the treatment of malignant melanoma.
- 5' capped single stranded ribonucleic acid encoding tumour-specific neoantigens, intended for the treatment of locally advanced or metastatic tumours.

## Organisational matters

- On 13 September 2018, CAT held a meeting with its Interested Parties. Topics discussed included: guidelines under development, comparability for ATMPs, ATMP action plan, GMO assessment of ATMPs in clinical trials, GCP for ATMPs, CAT interactions with other committees.
- CAT adopted its work plan for 2019.

## 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** | **Total** |
| Submitted MAAs | 3 | 1 | 2 | 3 | 2 | 2 | 1 | 1 | 4 | 1 | 20 |
| Positive draft Opinion | 1 | 0 | 1[ii] | 1[ii] | 2 | 1 | 1 | 2 | 2 | 3 | 14* |
| Negative draft opinions | 1[i] | 0 | 1[ii] | 0 | 0 | 0 | 2[iii] | 0 | 0 | 0 | 4 |
| Withdrawals | 1 | 1[i] | 0 | 0 | 2 | 0 | 0 | 0 | 0 | 1 | 5 |
| Ongoing MAAs | | | | | | | | | | | 1 |

*** Corresponding to 13 ATMPs**
[i] Same product (Cerepro)
[ii] Same product (Glybera)
[iii] 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 | Total |
| Positive Opinion | 0 | 0 | 1 | 1 | 9 | 4 | 3 | 6 | 3 | 6 | 33 |

| Scientific recommendation on advanced therapy classification | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017 | 2018 | Total |
| Submitted | 22 | 19 | 12 | 22 | 20 | 28 | 61 | 60 | 46 | 34 | 324 |
| Adopted | 12 | 27 | 12 | 16 | 23 | 29 | 31 | 87 | 49 | 30 | 316 |

| 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 | Total |
| Submitted | 1 | 0 | 0 | 1 | 3 | 1 | 1 | 2 | 2 | 0 | 11 |
| Adopted | 0 | 1 | 0 | 1 | 1 | 2 | 1 | 1 | 3 | 1 | 11 |

| Scientific advice procedure for ATMPs | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017 | 2018 | Total |
| Number of procedures | 17 | 19 | 21 | 19 | 23 | 33 | 39 | 46 | 55 | 37 | 309 |

| Paediatric Investigation Plans (PIP) for ATMPs | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017 | 2018 | Total |
| Number of procedures | 3 | 4 | 4 | 8 | 5 | 4 | 3 | 5 | 3 | 2 | 41 |

| Prime Eligibility for ATMPs | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|
| | 2016 | 2017 | 2018 | | | | | | Total |
| Discussed | 22 | 16 | 11 | | | | | | 49 |
| Granted | 8 | 6 | 5 | | | | | | 19 |

## Upcoming meetings following the September 2018 CAT meeting

- The 108th meeting of the CAT will be held on 10 – 12 October 2018.

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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