30 June 2011
EMA/CAT/499702/2011

**Monthly Report**

# Committee for Advanced Therapies (CAT)

June 2011 meeting

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 as well as variations, line extensions, renewals.

In addition, the report includes a summary table of the draft opinions issued by the CAT in the current year and a list of adopted guidelines and other public documents.

The Committee for Advanced Therapies (CAT) held its 28th meeting on 16th–17th June 2011.

## Initial applications for marketing authorisation

The CAT adopted one draft negative opinion by consensus on an initial marketing authorisation application (in accordance with Regulation (EC) No. 1394/2007). The draft opinion was transmitted to CHMP for adoption.

*New medicinal products*

**Glybera** (alipogene tiparvovec), from Amsterdam Molecular Therapeutics B.V.

Glybera, an Orphan medicine, is a gene therapy product using an adeno-associated viral vector intended for the treatment of adult patients diagnosed with lipoprotein lipase deficiency demonstrating hyperchylomicronemia or having a history of acute pancreatitis.

On the basis of the opinion of the Committee for Advanced Therapies (CAT) the CHMP recommended not granting a marketing authorisation for this product.

*More information about this opinion is available in a separate question-and answer-document on the Agency's website.*

## Scientific recommendation on advanced therapy classification

Further to consultation with the European Commission, the CAT finalised one scientific recommendation on the following classification of advanced therapy medicinal product (ATMP).

7 Westferry Circus • Canary Wharf • London E14 4HB • United Kingdom
**Telephone** +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7051
**E-mail** info@ema.europa.eu **Website** www.ema.europa.eu

The following product was not classified as an ATMP:

- live recombinant lentiviral vectors, intended for therapeutic vaccination of HIV-1 infected patients.

The CAT delivered its scientific recommendation after consultation with the European Commission within 60 days (active review time) after receipt of the final requests.

The CAT adopted one draft scientific recommendation on classification on advanced therapy medicinal product (ATMP). This procedure will be finalised after consultation with the European Commission within 60 days (active review time).

CAT received three new ATMP classification request for which a scientific recommendation will be delivered within 60 days (active review time) after receipt of the final request.

Further information on the ATMP classification procedure can be found at:

European Medicines Agency - ATMP classification - ATMP classification

## Organisational matters

The Committee discussed during the meeting topics related to:

- CAT work programme: deliverables for 2011 and priorities for 2012
- Outcome of the 4th informal CAT meeting which took place on 31st May – 1st June 2011 under the auspices of the Hungarian Presidency of the EU

## General scientific issues

- Criteria for the assessment of Marketing Authorisation Application for products legally on the market
- Use of DNA transposons as vectors for gene therapy

## 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 MAA for ATMP** | | | | |
|---|---|---|---|---|
| | **2009** | **2010** | **2011** | **Total** |
| Submitted | 3 | 1 | 0 | 4 |
| Positive draft Opinion | 1 | 0 | 0 | 1 |
| Negative draft Opinion | 1* | 0 | 1 | 2 |
| Withdrawals | 1 | 1 | 0 | 2 |

* Application subsequently withdrawn

| **Scientific recommendation on advanced therapy classification** | |
|---|---|

| | 2009 | 2010 | 2011 | Total |
|---|---|---|---|---|
| Submitted | 22 | 19 | 7 | 48 |
| Adopted | 12 | 27 | 5 | 44 |

| **Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs** | | | | |
|---|---|---|---|---|
| | **2009** | **2010** | **2011** | **Total** |
| Submitted | 1 | 0 | 0 | 1 |
| Adopted | 0 | 1 | 0 | 1 |

| **Contribution to scientific advice procedures** | | | | |
|---|---|---|---|---|
| | **2009** | **2010** | **2011** | **Total** |
| Submitted* | 17 | 15 | 4 | 36 |

* Comments from CAT submitted to SAWP

| **Contribution to Paediatric Investigation Plans (PIP) for ATMPs** | | | | |
|---|---|---|---|---|
| | **2009** | **2010** | **2011** | **Total** |
| Submitted* | 3 | 1 | 1 | 5 |

* Comments from CAT submitted to PDCO

## Upcoming meetings following the April 2011 CAT meeting

The 29th meeting of the CAT will be held at the Agency on 14th-15th July 2011.

NOTE:

1. This Monthly Report 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)

Tony Humphreys
Head of Regulatory, Procedural and Committee Support Sector
Tel.: (+44-20) 7418 8583
Fax: (+44-20) 7523 7051
AdvancedTherapies@ema.europa.eu