London, 14th December 2009
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COMMITTEE FOR ADVANCED THERAPIES (CAT)
DECEMBER 2009 MEETING
MONTHLY REPORT

The CAT Monthly Report includes statistical data for the current year on CAT scientific recommendation on 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 11th meeting on 3rd-4th December 2009.

Initial applications for marketing authorisation

The CAT adopted one draft negative opinion by majority 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

Cerepro [adenovirus-mediated Herpes Simplex Virus-thymidine kinase gene (Adv.HSV-tk)], from Ark Therapeutics Ltd, intended for use in conjunction with ganciclovir sodium for the treatment of patients with operable high grade glioma.

The draft opinion was transmitted to CHMP for adoption.

Scientific recommendation on advanced therapy classification

The CAT adopted four scientific recommendations on the following classification of advanced therapy medicinal products (ATMPs).

- The following medicines were classified as gene therapy medicinal product;
  - Product consisting of lentiviral vector expressing the human MYO7A gene, intended for the treatment of retinitis pigmentosa.
  - Product consisting of lentiviral vector expressing the ABCA4 gene, packaged into infectious VS virus envelope, intended for the treatment of retinal disorders.
  - Product consisting of adeno-associated virus (AAV) vector encoding the human N-sulfoglycosamine sulphohydrolase (SGSH) gene, intended for the treatment of congenital, hereditary, and neonatal diseases and abnormalities.

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- Mesenchymal stem cell-derived microvesicles (containing receptors, proteins, lipids, mRNA and microRNA) intended for the treatment of renal diseases/dysfunction, was not considered by CAT as an ATMP.

The CAT delivered its scientific recommendations after consultation with the European Commission in 60 days (active review time) after receipt of the final requests.
Further information on the ATMP classification procedure can be found at:
http://www.ema.europa.eu/htms/human/advanced_therapies/atmp_classification.htm

Certification of quality and non-clinical data for SMEs1 developing ATMPs
The Committee adopted the timetable of the first application for certification of quality data of an ATMP developed by an SME. This procedure is due to start on 18 December 2009 and the CAT is expected to deliver an opinion in 90 days (active time).
The certification system has been designed as an incentive for SMEs[1] to develop ATMPs. Although the certification procedure is a stand-alone evaluation procedure, it aims at facilitating the evaluation of any future application for clinical trial authorisation or a marketing authorisation application (MAA), provided that these applications are based on the same data.
Further information on the ATMP certification procedure can be found at:
http://www.ema.europa.eu/htms/human/advanced_therapies/certification.htm

Organisational matters
The Committee addressed during the meeting topics related to:
- Dossier requirements for new ATMP marketing authorisation applications (MAA): see procedural announcement below.
- Outcome of first meeting held on 27 November 2009 with representatives of CAT, NBOG and NB-MED and discussion on future steps to establish cooperation with notified bodies on issues related to the assessment of combined advanced therapy medicinal products;

General scientific issues
The Committee endorsed the following documents for CHMP adoption in December 2009:
- Draft 'Question and Answers' document on gene therapy, developed by GTWP;
- Draft 'Concept Paper on the Revision of the Note for Guidance on the Quality, Pre-clinical and Clinical Aspects of Gene Transfer Medicinal Products', developed by GTWP;
- Draft 'Concept paper on the development of a guideline on the application of the risk-based approach according to Annex I Part IV of Dir. 2001/83/EC applied to ATMPs', developed by CPWP/GTWP.

PROCEDURAL ANNOUNCEMENT
Change of
Submission of new MAA for ATMPs: change of website location
Applicants who intend to submit a MAA for an ATMP are advised to check:
the correct number of copies of MAA dossier to be sent to Rapporteur and Co-Rapporteur published at:
http://www.ema.europa.eu/pdfs/human/advancedtherapies/dossier_requirements.pdf
and
the dates for submission published at:
http://www.ema.europa.eu/pdfs/human/submission/ATMPs_full_1phase.pdf
More information on the Procedural timetables/Submission dates are published at:
http://www.ema.europa.eu/htms/human/submission/submission.htm

[1] small and medium-sized enterprises

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	**Total**
Submitted	3	3
Ongoing	0	0
Positive draft Opinion	1	1
Negative draft Opinion	1	1
Withdrawals	1	1

Contribution to scientific advice procedures		
	2009	**Total**
Submitted*	17	17

* Comments from CAT submitted to SAWP

Contribution to Paediatric Investigation Plans (PIP) for ATMPs		
	2009	**Total**
Submitted*	3	3

* Comments from CAT submitted to PDCO

Scientific recommendation on advanced therapy classification		
	2009	**Total**
Submitted	22	22
Ongoing	10	10
Adopted	12	12

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

UPCOMING MEETINGS FOLLOWING THE NOVEMBER 2009 CAT MEETING

- The 12th meeting of the CAT will be held at the EMEA on 14th-15th January 2010.

NOTE:

1. This Monthly Report and other documents can be found on the internet at the following location: http://www.ema.europa.eu
2. Specific information related to ATMPs and the activities of the CAT (role, responsibilities and composition) can be found at: http://www.ema.europa.eu/htms/human/advanced_therapies/intro.htm and http://www.ema.europa.eu/htms/general/contacts/CAT/CAT.html
3. **As of 8 December 2009, the URL of the Agency's website and e-mail addresses has changed from 'emea.europa.eu' to 'ema.europa.eu'. Please update your bookmarks and address books accordingly.**

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