EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Monthly Report

Committee for Advanced Therapies (CAT) February 2010 meeting

The CAT Monthly Report includes statistical data on CAT scientific recommendation 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 13th meeting on 11th-12th February 2010.

Scientific recommendation on advanced therapy classification

Further to consultation with European Commission, the CAT finalised four scientific recommendations on the following classification of advanced therapy medicinal products (ATMPs).

The following medicines were classified as gene therapy medicinal products:

- Product consisting of a *Salmonella typhi* strain genetically modified to secrete a fusion protein of the prostate specific antigen (PSA) and a protein leading to an increased antigenicity, intended for the treatment of prostate cancer.
- Genetically modified *Lactococcus lactis* secreting human interleukin-10, intended for the treatment of inflammatory bowel disease.

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Those classifications are the first recommended by the CAT for products consisting of prokaryotic cells (bacteria), genetically modified to contain a recombinant nucleic acid, which express the product (protein) encoded by the inserted genetic sequence using the secretion machinery of the modified prokaryotic cells.
Notwithstanding the above classification, as for all ATMP, a risk based approach may be applied to determine the extent of quality, non-clinical and clinical data to be included in the marketing authorisation application for these products, in accordance with Annex I to Directive 2001/83/EC, as amended.

The following medicine was classified as tissue engineered product:

- Product consisting of autologous osteoprogenitor cells, isolated from bone marrow and expanded in vitro, incorporated, as an integral part, in a 3D biodegradable scaffold, intended for repairing, regenerating and replacing bone defects in odontostomatology and maxillo-facial surgery. This product was classified as a combined tissue engineered product.

The following medicine was not classified as an ATMP:

- Product consisting of naturally occurring antigen-specific CD8+ donor lymphocytes isolated with streptamers, intended for the treatment of infectious diseases.

The CAT delivered its scientific recommendations after consultation with the European Commission within 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

The CAT adopted another three scientific recommendations pending consultation with European Commission. Information on these scientific recommendations will be included in the March 2010 monthly report.

General scientific issues

The Committee addressed during the meeting topics related to:

- Draft Guideline on genetically-modified cells (EMA/CHMP/GTWP/671639/2010)

- 'Workshop on stem-cell based therapies' to be held on 10th May 2010 at the European Medicines Agency: the aim of this event is to discuss the state-of-the-science on stem cell-based therapies bringing together relevant stakeholders (regulatory, academic, and industry scientists)

More information on this workshop and the registration form are available at:
http://www.ema.europa.eu/pdfs/conferenceflyers/EMA_Stemcell_Workshop.pdf

http://www.ema.europa.eu/pdfs/conferenceflyers/EMA_Stemcell_Workshop_Registrationform.pdf

PROCEDURAL ANNOUNCEMENT

Applicants should use the new **Pre-submission request form** that has been released on the Agency's website with any additional supportive documentation required for:
- Intent to submit ATMP classification request
- ATMP Classification Request
- Eligibility for EMEA Procedure (ITF)
- Intent to Submit ATMP Certification
- Request ATMP Certification
- Request Eligibility to Centralised Procedure
- Intent to Submit a MA Application
- Pre-submission Meeting Request (MAA)
- Accelerated Assessment (MAA) Request

With regard to ongoing pre-submission requests, this form should also be used for:
- Notification of Change (administrative request, e.g. change of Applicant and contact details) - Withdrawal of Pre-Submission Request

This form should always be submitted **electronically** to h-cig2@ema.europa.eu except in the following cases:
Requests for Eligibility to the Centralised Procedure, where the pre-submission form should be sent to CPeligibility@ema.europa.eu, together with the relevant additional justification (Annex 1 SPC and Annex 2 Justification for eligibility), as separate Word documents.

Intent to Submit an ATMP classification and **ATMP classification request**, where the pre-submission form should be sent to AdvancedTherapies@ema.europa.eu. For the ATMP classification request, the 'ATMP classification briefing information' should be included as a separate Word document.
These forms can be found at:
http://www.ema.europa.eu/htms/human/advanced_therapies/atmp_classification.htm

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**	**Total**
Submitted	3	1	4
Positive draft Opinion	1	0	1
Negative draft Opinion	1	0	1
Withdrawals	1	0	1

Scientific recommendation on advanced therapy classification			
	2009	**2010**	**Total**
Submitted	22	3	25
Adopted	12	10	22

Contribution to scientific advice procedures			
	2009	**2010**	**Total**
Submitted*	17	5	22

* Comments from CAT submitted to SAWP

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

Contribution to Paediatric Investigation Plans (PIP) for ATMPs			
	2009	**2010**	**Total**
Submitted*	3	0	3

* Comments from CAT submitted to PDCO

UPCOMING MEETINGS FOLLOWING THE JANUARY 2010 CAT MEETING

The 14th meeting of the CAT will be held at the EMEA on 11th-12th March 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

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