EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Monthly Report

Committee for Advanced Therapies (CAT)
May 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 16th meeting on 11th-12th May 2010.

First certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs

Further to the CAT recommendation the Agency issued the first certification on the quality data of an ATMP product developed by an SMEs. This first certification is a milestone for the CAT and the Agency, providing to the SME applicant an in-depth review of the data generated on the ATMP, independent from and prior to the submission of a marketing authorisation application. This is an incentive for the SME, allowing them to further develop their product. A press release is published at: http://www.ema.europa.eu/pdfs/human/cat/32819110en.pdf

Scientific recommendation on advanced therapy classification

- The CAT started 3 new ATMP classification procedures for which a scientific recommendation will be delivered 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

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General scientific issues

The Committee adopted the following document:

- Draft Guideline on the quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells (EMA/CHMP/GTWP/671639/2008)

This guideline defines scientific principles and provides guidance for the development and evaluation of medicinal products containing genetically modified cells intended for use in humans. Its focus is on the quality, safety and efficacy requirements of genetically modified cells developed as medicinal products.

The document, released for public consultation until 30th November 2010 will be published in due course at:

http://www.ema.europa.eu/htms/human/humanguidelines/multidiscipline.htm#gene

The Committee discussed the following topics:

- Reflection paper on quality, non-clinical and clinical issues related to the development of recombinant adeno-associated viral vectors (rAAV)

The European Medicines Agency's Workshop on stem cell-based therapies was held at the Agency on 10th May 2010. The workshop was part of the public consultation process on the 'Reflection paper on stem cell-based medicinal products' that was released on 16th March 2010 for consultation until 30th June 2010.

This reflection paper can be found at: http://www.ema.europa.eu/pdfs/human/cat/57113409en.pdf

More information on the workshop can be found at:
http://www.ema.europa.eu/meetings/conferences/10may10.htm

Organisational matters

The Committee discussed during the meeting topics related to:

- EU GMP Guide: Annex 2 to the Manufacture of Biological Medicinal Substances and Products for Human Use (ENTR/C/8/SF D(2010)380334).
- CAT informal meeting held under the Belgian presidency of the European Union on 30th September-1st October 2010.
- Draft CAT Work programme 2010-2015.
- The Committee agreed with the appointment of Niall MacAleenan as the Chair of the EMA/CAT-Notified Body Coordination group. A summary of the outcomes of the Coordination group meetings of 23rd March and 21st April 2010 can be found in Annex 1.

Hearing with Interested Parties to the CAT

On 11th May 2010, CAT held the second part of a hearing with representatives of large industry associations (EuropaBio, EBE, Eucomed, BHA, BIA). The CAT continued the discussion of the topics identified by the stakeholders such as: interactions between CAT and Notified bodies for the assessment of combined ATMPs, involvement of stakeholders in the development of guidance documents, CAT cooperation with other Regulatory agencies (e.g. FDA), implementation of the 'hospital exemption clause' at member state level, conduct of clinical trials for ATMPs.

Future hearings with other interested parties will be conducted between June and September 2010 in the margins of CAT Plenary meetings.

Organisations which have not yet registered to become an interested party to the CAT can still do so by completing the form that can be found on the Agency's Website:

http://www.ema.europa.eu/htms/human/advanced_therapies/interested_parties.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	1	2

[#] application subsequently withdrawn

Scientific recommendation on advanced therapy classification			
	2009	**2010**	**Total**
Submitted	22	7	29
Adopted	12	14	26

Contribution to scientific advice procedures			
	2009	**2010**	**Total**
Submitted*	17	8	25

* 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	1	1

Contribution to Paediatric Investigation Plans (PIP) for ATMPs			
	2009	**2010**	**Total**
Submitted*	3	1	4

* Comments from CAT submitted to PDCO

UPCOMING MEETINGS FOLLOWING THE MAY 2010 CAT MEETING

The 17th meeting of the CAT will be held at the Agency on 17th-18th June 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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Annex 1:

Summary of the outcomes of the EMA/CAT-Notified Body Coordination group meeting of 23rd March 2010

- On 27th November 2009, at meeting took place between the European Medicines Agency (EMA), the Committee for Advanced Therapies (CAT), the European Commission (DG-Entr - F3), the Notified Bodies Medical Devices EU Coordination Group (NB-MED) and the Notified Body Operation Group (NBOG) representatives. The aim of that meeting was to establish contacts between Notified Bodies for medical devices and the Agency within the context of the evaluation of Advanced Therapy Medicinal products combined with medical devices. It was agreed to establish a formal Coordination group composed of representatives of the institutions / organisation mentioned above, to further discuss practical details of such interactions and to set priorities of involvement.
- The 1st meeting of this Coordination group took place by teleconference on 23rd March 2010.
- During that meeting were identified topics to be developed related to scope of interaction, procedural, terminology, inspection and post-authorisation activities, standards etc… and timelines were agreed upon. The main priority for the remaining of the year is the finalisation of a procedural advice on the consultation of Notified Bodies in accordance with Article 9 of Regulation (EC) No. 1394/2007.
- It was also agreed that at the next meeting which will take place on 21st April 2010 at the EMA, a Chair of the coordination group will be formally elected.

Summary of the outcomes of the EMA/CAT-Notified Body Coordination group meeting of 21st April 2010

- The Coordination group met on 21st April 2010. In view of the travel restrictions following the Icelandic Vulcano eruption, the meeting was held by teleconference.
- Niall MacAlleenan was nominated by consensus as the chair of the coordination group.
- The group discussed and agree on the topics of common interest and agreed on the list of priorities, namely: legal interpretations and clarifications; procedure for notified body consultation; scientific guidance on the evaluation of the medicinal device part in a combined ATMP. Drafting groups, deadlines and deliverables were set.
- The next Coordination group meeting will take place on 2nd June 2010