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Patient Health Protection

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

January 2012 meeting

The Committee for Advanced Therapies (CAT) held its 34th CAT meeting on 11th and 13th January 2012.

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.

CAT Workshop with Stakeholders

On Thursday 12th January 2012, CAT held a workshop entitled: 'Focus Groups, a model for fruitful interactions between CAT and its Interested Parties'. About 200 participants attended this workshop at the EMA premises, and further participants followed the meeting remotely via a video-link.

The one-day workshop was organised by the CAT as part of the Committee's effort to strengthen the dialogue with all of its stakeholders. The main aim of this workshop was to communicate the outcome of the focus group meetings held in 2011 concerning:

- Non-clinical development of Advanced Therapy Medicinal Products (ATMPs);
- A better system to navigate scientific guidance documents on gene therapy and cell-based medicinal products;
- Incentives for Academic, hospitals and trusts developing ATMPs.

The workshop allowed stakeholders involved in the development of ATMPs to:

- Hear signification points collected by the CAT Interested Parties on identified bottlenecks in ATMP development;
- Learn about CAT Focus groups' action plans for 2012;
- Participate in discussions with panellist from the CAT, EMA and Interested Parties.

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For further information see the event programme. The presentations, questions addressed during the panel discussion and the meeting report will be posted on the EMA Website at: http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2011/11/event_detail_000545.jsp&mid=WC0b01ac058004d5c3

Scientific recommendation on advanced therapy product classification

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

The following products was classified as gene therapy medicinal products:

- Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human ABCD1 gene, intended for the treatment of patients with childhood cerebral adrenoleukodystrophy.
- Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta-A-T87Q-globin gene, intended for the treatment of beta-thalassemia major and intermedia and sickle cell anemia

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

CAT received one 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

During its January 2012 meeting, CAT identified topic for discussion for discussion at the 5th Informal CAT meeting that will take place on 24th – 25th May 2012 in Copenhagen under the auspices of the Danish Presidency of the Council of the European Union.

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**	**2012**	**Total**
Submitted	3	1	2	1	7
Positive draft Opinion	1	0	1[i]	0	2
Negative draft Opinion	1*	0	1	0	2
Withdrawals	1	1	0	0	2

* Application subsequently withdrawn
[i] Re-examination opinion (Glybera)

Scientific recommendation on advanced therapy classification					
	2009	**2010**	**2011**	**2012**	**Total**
Submitted	22	19	12	1	54
Adopted	12	27	12	2	53

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs					
	2009	**2010**	**2011**	**2012**	**Total**
Submitted	1	0	0	0	1
Adopted	0	1	0	0	1

Scientific advice procedures on ATMPs					
	2009	**2010**	**2011**	**2012**	**Total**
Discussed*	25	30	36	2	**93**
Written comments to SAWP	17	15	8	0	40

* Most scientific advices for ATMPs are discussed by the CAT at 2 time points during the SA procedure

Contribution to Paediatric Investigation Plans (PIP) for ATMPs					
	2009	**2010**	**2011**	**2012**	**Total**
Submitted*	3	1	4	0	8

* Comments from CAT submitted to PDCO

Upcoming meetings following the January 2012 CAT meeting

The 35th meeting of the CAT will be held at the Agency on 9th – 10th February 2012.

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)

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