25 October 2011
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Monthly Report

Committee for Advanced Therapies (CAT)

October 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 31st meeting on 13th–14th October 2011.

Centralised procedure

Re-examination for Glybera concluded

Following re-examination of its previous negative opinion, the CAT adopted by majority a draft positive opinion, recommending the granting of marketing authorisations under exceptional circumstances for Glybera [Alipogene tiparvovec)], from Amsterdam Molecular Therapies B.V.

Glybera was intended for the treatment of adult patients diagnosed with lipoprotein lipase deficiency demonstrating hyperchylomicronemia or having a history of acute pancreatitis, an indication for which it was designated as an orphan medicinal product in March 2004.

Based on the reanalysis of the data provided by the Applicant during the re-examination procedure and taking into account the recommendation of the Scientific Advisory Group, the CAT concluded that there was currently sufficient evidence of safety and efficacy to recommend approval under exceptional circumstances at this stage.

The CAT considered that the evidence generated by overall efficacy data suggested that Glybera leads to clinical relevant reduction of pancreatitis risk, reduction in hospital admissions and ICU stay, at least in some LPLD patients.

The CAT proposed the following revised indication: ‘Glybera is indicated for adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from at least one pancreatitis episode

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despite dietary fat restrictions. The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein'.

In addition to the restriction of the therapeutic indications the CAT agreed on well defined follow-up specific obligations to allow the post-authorisation follow-up of efficacy, adverse reactions and risk management.

Subsequently, the Committee for Medicinal Products for Human Use (CHMP), taking into account the draft opinion prepared by the CAT, having considered the detailed grounds for the re-examination, recommended the refusal of the granting of the MA under exceptional circumstances.

More information about this re-examination procedure is available in a separate question-and-answers 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 products (ATMPs).

The following product was classified as a somatic cell therapy medicinal product:

- Autologous dendritic cell (DCs) immunotherapy consisting of autologous mature DCs co-electroporated with autologous RCC IVT RNA and synthetic CD40L IVT RNA, intended for the treatment of patients with advanced renal cell carcinoma.

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 two draft scientific recommendations 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 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

The main topics addressed during the October 2011 CAT meeting related to:

- CAT-ESGTC Satellite Workshop on Advanced Therapy Medicinal Product: 'From promise to reality. Regulatory path for translation of research into commercial medicinal products'. More information on this conference, which will take place on 27 October 2011, can be found at: CAT-ESGCT Satellite Workshop 27 Oct 2011. Registration is available via the ESGCT at: ESGCT.

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	2	6
Positive draft Opinion	1	0	1[i]	2
Negative draft Opinion	1[*]	0	1	2
Withdrawals	1	1	0	2

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

Scientific recommendation on advanced therapy classification				
	2009	**2010**	**2011**	**Total**
Submitted	22	19	10	51
Adopted	12	27	9	48

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	6	38

* Comments from CAT submitted to SAWP

Contribution to Paediatric Investigation Plans (PIP) for ATMPs				
	2009	**2010**	**2011**	**Total**
Submitted*	3	1	3	7

* Comments from CAT submitted to PDCO

Upcoming meetings following the October 2011 CAT meeting

The 32nd meeting of the CAT will be held at the Agency on 10th – 11th November 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)

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