EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

27 July 2012
EMA/CAT/497804/2012
Patient Health Protection

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

July 2012 meeting

The Committee for Advanced Therapies (CAT) held its 40th CAT meeting on 12th – 13th July 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 and Paediatric Investigation Plans, 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.

Centralised procedure

Evaluation of Glybera concluded

Following a request from the European Commission, the CAT re-evaluated the medicinal product Glybera in a restricted patient population with severe or multiple pancreatitis attacks. The CAT, confirming its previous opinion of October 2011, adopted by majority a draft positive opinion, recommending the granting of marketing authorisation under exceptional circumstances for Glybera [Alipogene tiparvovec)], from uniQure biopharma B.V. (formerly Amsterdam Molecular Therapies B.V.). Glybera is intended to treat lipoprotein lipase (LPL) deficiency in patients with severe or multiple pancreatitis attacks, despite dietary fat restrictions.

LPL deficiency is an ultra-rare inherited disorder estimated to affect no more than one or two people per million. Glybera was designated as an orphan medicinal product in March 2004.

Based on the reanalysis of the data during the re-examination procedure, 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.

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The CAT proposed the following revised indication: 'Glybera is indicated for adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks 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 (see section 4.4) '.

In addition to the restriction of the therapeutic indication 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 granting of the marketing authorisation under exceptional circumstances.

More information about this procedure is available in a separate Press release at:

European Medicines Agency recommends first gene therapy for approval

Scientific recommendation on advanced therapy product classification

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

The following product was classified as a tissue engineered product:

- Bone marrow derived autologous suspensions of hematopoietic and mesenchymal stem cells depleted from erythrocytes and lymphocytes intended for the treatment of complete or incomplete traumatic spinal cord injury

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

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]	1[i]	3
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	12	65
Adopted	12	27	12	12	63

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs					
	2009	**2010**	**2011**	**2012**	**Total**
Submitted	1	0	0	1	2
Adopted	0	1	0	0	1

Scientific advice procedures on ATMPs					
	2009	**2010**	**2011**	**2012**	**Total**
Discussed*	25	30	36	22	113
Written comments to SAWP	17	15	8	1	41

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

Paediatric Investigation Plans (PIP) for ATMPs					
	2009	**2010**	**2011**	**2012**	**Total**
Discussed*	4	7	6	4	21
Written comments to PDCO	3	1	4	0	8

* PIPs for ATMPs are discussed by the CAT once or twice during the procedure

Upcoming meetings following the June 2012 CAT meeting

The 41st meeting of the CAT will be held at the Agency on 13th – 14th September 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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