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Procedure Management and Business Support Division

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

February 2015 meeting

The Committee for Advanced Therapies (CAT) held its 68th CAT meeting on 19 – 20 February 2015.

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.

Scientific recommendation on advanced therapy product classification

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

The following products were classified as somatic cell therapy medicinal products:

- Adipose derived mesenchymal stem cells intended for the treatment of autoimmune diseases (rheumatoid arthritis and systemic lupus erythematosus).
- Autologous tumour-infiltrating lymphocytes derived from metastatic melanoma intended for the treatment of metastatic melanoma.

The following product was classified as not an ATMP:

- Engineered extracellular matrix proteins produced by human fibroblasts cultured *in vitro* on an absorbable polymer scaffold, intended for surgical or interventional treatment of congenital heart defects, thereby correcting anatomic malformations.

CAT received five new ATMP classification requests for which a scientific recommendation will be delivered within 60 days (active review time) after the receipt of the final request.

Further information on the ATMP classification procedure can be found at:

European Medicines Agency - ATMP classification

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Development of GMP requirements for investigational ATMPs

On request of the European Commission, CAT started to draft a guideline on Good Manufacturing Practice (GMP) requirements for investigational ATMPs. A dedicated drafting group of the CAT has been established, who will work together with members of the GMPD Inspectors Working Group to develop a document reflecting how GMP can be adapted to ATMPs under clinical investigation.

Guideline on the quality, non-clinical and clinical aspects of gene therapy medicinal products

CAT adopted the Guideline on quality, non-clinical and clinical aspects of gene therapy medicinal products. The drafting of this guideline, which is a revision of the Note for Guidance on quality, preclinical and clinical aspect of Gene transfer medicinal products (CPMP/BWP/3088/99, published in 2001), was initiated by the Gene Therapy Working Party and finalised by a CAT Drafting Group, in collaboration with the Biologics Working Party. The guideline will soon be published for external consultation.

CAT involvement in the Benefit Risk and Patient Registries projects

CAT representative were appointed to take part in the Benefit Risk Steering Group and in the Patient Registries Steering Group. This will strengthen the CAT input and cooperation in projects led by other Committees that are relevant and important for the authorisation of ATMPs.

Cooperation with the other Committees and Working Parties

- On request of the PDCO, CAT discussed the acceptable levels of Dimethyl sulfoxide (DMSO) for children. DMSO is used as cryopreservative for cell-based medicines.
- CAT appointed Bernd Gänsbacher as member of the Healthcare Professionals' Organisations Working Party (HCPWP). Bernd Gänsbacher is replacing Olli Tenhunen, who stepped down from this position in January 2015.

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 Marketing Authorisation Applications (MAA) for ATMP							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Submitted MAAs	3	1	2	3	2	2	0	13
Positive draft Opinion	1	0	1[i]	1[i]	2	1	0	6 Corresponding to 5 ATMPs
Withdrawals	1	1	0	0	2	0	0	4
Ongoing MAAs								4

[i] Same product (Glybera)

	Variations (Type II) for authorised ATMP							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Positive draft Opinion	0	0	1	1	9	4	0	15

	Scientific recommendation on advanced therapy classification							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Submitted	22	19	12	17	20	28	6	129
Adopted	12	27	12	14	23	29	4	123

	Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Submitted	1	0	0	1	3	1	0	6
Adopted	0	1	0	1	1	2	0	5

	Scientific advice procedures on ATMPs							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Discussed*	25	30	36	31	36	48	10	216

* 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**	**2013**	**2014**	**2015**	**Total**
Discussed*	4	7	6	9	7	7	0	40

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

Upcoming meetings following the February 2015 CAT meeting

The 69th meeting of the CAT will be held on 19 – 20 March 2015.

NOTE:

1. This Monthly Report, the Agenda of this meeting, the Minutes of the previous CAT meeting 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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