24 July 2015
EMA/CAT/499506/2015
Procedure Management and Business Support Division

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

July 2015 meeting

The Committee for Advanced Therapies (CAT) held its 73rd CAT meeting on 16 – 17 July 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.

Centralised procedure: Re-examination procedure for Heparesc (Allogeneic human heterologous liver cells)

Heparesc is a somatic cell therapy product containing living human liver cells and is intended for the treatment of children with urea cycle disorders. At the CAT meeting in April 2015, the Committee adopted a negative draft opinion for Heparesc. The CAT draft opinion and the CAT assessment were transmitted to the CHMP, who adopted during its June 2015 meeting a negative opinion, recommending the refusal of the marketing authorisation of Heparesc.

On 9 July 2015, the applicant (Cytonet GmbH & Co. KG) submitted to the EMA their request for re-examination of the opinion for Heparesc. CAT appointed a new Rapporteur and Co-Rapporteur for this re-examination procedure. The re-examination procedure will start by the end of August 2015 and CAT and CHMP will finalise the re-examination procedure within 60 days.

For more information on the re-examination procedure, see here.

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 (ATMP).

The following product was classified as gene therapy medicinal product:

- Autologous genetically modified Chimeric Antigen Receptor (CAR+) cells intended for the treatment of various types of cancer.

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

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- Human monocytes-derived suppressive cells, expanded *ex vivo*, intended for the treatment of acute Graft-versus-Host Disease refractory to first-line treatment.

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

European Medicines Agency - ATMP classification

CAT started the work on two new guidance documents

CAT appointed drafting group members to work on two new guidance documents:

- Question and Answer document on minimally manipulated ATMPs and the application of the risk based approach.
- Guideline on requirements for investigational ATMPs.

A concept paper will be drafted in preparation of the Guideline on requirements for investigational ATMPs.

CAT work plan 2015 and 2016

CAT members discussed the progress of the projects included in the CAT work plan for 2015 and agreed timeline for the pending activities. CAT members also discussed topics for inclusion in the CAT work plan for 2016: further discussion and identification of topic leaders will take place at the September CAT meeting.

Registration is open for the joint CAT-ISCT workshop on Challenges and Opportunities for a successful development and approval of ATMPs

On 25 September 2015, a workshop will take place during the International Society for Cellular Therapy (ISCT) Europe 2015 Regional meeting in Seville, Spain. During this workshop, CAT wants to reach out to ATMP developers from academia, hospitals and industry with a programme aiming to provide insight of CAT's considerations and expectations on quality development and manufacturing issues, non-clinical testing and clinical development of ATMPs. The programme includes also a talk on the EMA/CAT support available to ATMP developers and will allow interacting directly with the Regulators in an open forum discussion.

The programme of this Workshop can be found here. For more information and registration, visit the website of ISCT Europe 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	1	14
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	0	6 Corresponding to 5 ATMPs
Negative draft opinions	1[i]	0	1[ii]	0	0	0	1	3
Withdrawals	1	1[i]	0	0	2	0	0	4
Ongoing MAAs								4

[I] Same product (Cerepro)
[ii] 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	22	20	28	13	136
Adopted	12	27	12	16	23	29	15	134

	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	1	6

	Scientific advice procedures on ATMPs							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Discussed*	25	30	36	31	36	48	45	245
Number of procedures	17	19	21	19	23	33	25	157

* 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	1	41

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

Upcoming meetings following the July 2015 CAT meeting

The 74th meeting of the CAT will be held on 17 – 18 September 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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