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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

January 2017 meeting

The Committee for Advanced Therapies (CAT) held its 89th CAT meeting on 18 – 20 January 2017.

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 2 scientific recommendations on the classification of advanced therapy medicinal products.

The following product was classified as a tissue engineered product:

- Bone marrow derived lineage negative heterogenic stem and progenitor cells, intended for the treatment of amyotrophic lateral sclerosis in aduts.

The following product was classified as not an ATMP:

- Banked leukocytes with cancer killing activity, intended for the treatment of metastatic pancreatic ductal adenocarcinoma.

ATMP Certification procedure

CAT adopted a positive opinion on the certification application for an ATMP developed by a micro, small and medium-sized enterprise (SME). The ATMP concerned by this certification application is a gene therapy medicinal product composed of a genetically modified bacterium intended for the treatment of solid malignancies with or without metastases. The certification for this product related to the quality and non-clinical data.

CAT adopted its work plan for 2017

Highlights of the work plan are the development of scientific guidelines, reflections on benefit risk assessment for ATMPs and the use of registries and considerations on gene editing technologies and other approaches for making ATMPs more readily available to patients.

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The work plan will be published shortly on the EMA website.

Organisational matters

- CAT discussed with colleagues from the European Commission, Directorate General Research ATMP related priorities that could be considered for future calls funded by Horizon 2020.
- CAT provided input in the Action plan developed by EMA following the ATMP multi-stakeholders workshop that took place on 27 May 2016.
- CAT discussed the revision of the ATMP guideline on safety and efficacy follow-up and risk management.

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**	**2016**	**2017**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	0	15
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	0	9*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	4
Ongoing MAAs										2

*** Corresponding to 8 ATMPs**
[i] Same product (Cerepro)
[ii] Same product (Glybera)
[iii] CAT adopted two negative draft opinions for the same product (Heparesc)

Variations (Type II) for authorised ATMP										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Positive Opinion	0	0	1	1	9	4	3	6	0	24

Scientific recommendation on advanced therapy classification										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Submitted	22	19	12	22	20	28	61	60	1	245
Adopted	12	27	12	16	23	29	31	87	2	239

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs										
	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Submitted	1	0	0	1	3	1	1	2	0	9
Adopted	0	1	0	1	1	2	1	1	1	8

Scientific advice procedure for ATMPs										
	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Discussed*	25	30	36	31	36	48	63	67	6	342
Number of procedures	17	19	21	19	23	33	39	46	8	225

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

Paediatric Investigation Plans (PIP) for ATMPs										
	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Discussed*	4	7	6	9	7	7	3	4	2	49

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

Prime Eligibility for ATMPs									
	2016	2017							Total
Discussed	22	4							26
Granted	7	1							8

Upcoming meetings following the January 2017 CAT meeting

The 90th meeting of the CAT will be held on 15 – 17 February 2017.

The election of the CAT chair will take place at the start of the February CAT meeting.

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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