23 June 2016
EMA/CAT/358066/2016
Procedure Management and Committees Support Division

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

June 2016 meeting

The Committee for Advanced Therapies (CAT) held its 83rd CAT meeting on 16-17 June 2016.

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.

CAT recommends the granting of a conditional marketing authorisation for Zalmoxis

Zalmoxis contains allogeneic T cells that are genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) and the suicide gene herpes simplex I virus thymidine kinase (HSV-TK Mut2). Zalmoxis is intended as adjunctive treatment in haploidentical haematopoietic stem cell transplantation (HSCT) of adult patients with high-risk haematological malignancies. The T cells are given to transplant patients to help the body fight off infection, enhance the success of the transplant and support long-lasting anti-cancer effects; however T cells can also cause graft-versus-host disease. The suicide gene in Zalmoxis makes the T cells susceptible to ganciclovir or valganciclovir. If the patient develops graft-versus-host disease, ganciclovir/valganciclovir is given, which kills the T cells that have the suicide gene, so preventing further development of the disease.

Following an in-depth review of the dossier submitted by the applicant, MolMed SpA., CAT concluded during its June meeting that a positive benefit risk profile has been demonstrated for Zalmoxis. CAT adopted by majority the positive draft opinion recommending the granting of the conditional marketing authorisation for Zalmoxis. The CHMP adopted during their June 2016 meeting the positive opinion for Zalmoxis.

Further information can be found in the press release.

Scientific recommendation on advanced therapy product classification

Further to consultation with the European Commission, the CAT finalised 4 scientific recommendations on the classification of advanced therapy medicinal products.

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The following products were classified as gene therapy medicinal products:

- Adeno-associated virus (AAV) vector encoding genes from an algae channel rhodopsin, intended for treatment of Retinitis Pigmentosa.

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

- Autologous ex vivo expanded regulatory T lymphocytes, intended for the treatment of Type 1 Diabetes Mellitus.
- Allogeneic Epstein-Barr Virus Cytotoxic T Lymphocytes, intended for the treatment of Epstein-Barr Virus-associated Post Transplant Lymphoproliferative Disorder.

The following product was classified as tissue engineered product:

- Mesenchymal stem cells isolated from autologous bone marrow, intended for the treatment of the following neurological diseases in children: encephalopathy (Hypoxic–ischaemic encephalopathy, immune/autoimmune encephalopathy), epilepsy and spinal cord injury.

CAT received 4 new ATMP classification requests for which a scientific recommendation will be delivered within 60 days (active review time).

Organisational, regulatory and methodological matters

CAT discussed the outcome of the 'Strategic Review and Learning Meeting' that was held on 1 – 2 June 2016 under the auspices of the Dutch Presidency of the Council of the European Union.

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**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	15
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	9 Corresponding to 8 ATMPs
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[*]	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	4
Ongoing MAAs									2

[i] Same product (Cerepro)
[ii] Same product (Glybera)
[*] 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	Total
Positive draft Opinion	0	0	1	1	9	4	3	4	21

Scientific recommendation on advanced therapy classification									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Submitted	22	19	12	22	20	28	61	40	224
Adopted	12	27	12	16	23	29	31	65	215

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Submitted	1	0	0	1	3	1	1	1	8
Adopted	0	1	0	1	1	2	1	1	7

Scientific advice procedure for ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Discussed*	25	30	36	31	36	48	63	34	303
Number of procedures	17	19	21	19	23	33	39	31	202

* 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	2016	Total
Discussed*	4	7	6	9	7	7	3	3	46

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

Prime Eligibility for ATMPs									
	2016								Total
Discussed	6								6

Upcoming meetings following the June 2016 CAT meeting

The 84th meeting of the CAT will be held on 13-15 July 2016.

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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