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Procedure Management and Committees Support Division

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

March 2016 meeting

The Committee for Advanced Therapies (CAT) held its 80th CAT meeting on 22 – 23 March 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 the marketing authorisation for Strimvelis

Strimvelis is a gene therapy medicinal product containing the patient's bone marrow derived (CD34+) cells that are genetically modified to encode for the adenosine deaminase (ADA) enzyme gene. Strimvelis is intended for the treatment of children with severe combined immunodeficiency due to ADA-deficiency (ADA-SCID), who have no matching donor for a stem cell transplant. After infusion, Strimvelis cells will engraft in the bone marrow and secrete pharmacologically active levels of the ADA enzyme. The effect of the product is expected to be life-long.

Following an in-depth review of the dossier submitted by the applicant, GlaxoSmithKline Trading Services, CAT concluded during its March meeting that a positive benefit risk profile has been demonstrated. CAT adopted the positive draft opinion recommending the granting of the marketing authorisation for Strimvelis. The CHMP subsequently adopted the positive opinion for Stimvelis during its March 2016 meeting.

Further information can be found in the press release here.

Scientific recommendation on advanced therapy product classification

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

The following product was classified as a gene therapy medicinal product:

- Irradiated, allogeneic pancreatic tumour cell lines genetically modified to secrete human granulocyte macrophage stimulating factor, intended for the treatment of pancreatic cancer.

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

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- *Ex vivo* expanded autologous Epstein-Barr Virus specific T-cells, intended for the treatment of Epstein-Barr Virus positive malignancies.

The following products were classified as tissue engineered products:

- Human burn eschar and debrided adipose tissue cells as a suspension, intended for the treatment of burns and non-healing wounds.
- Human burn eschar and debrided adipose tissue cells as a sheet, intended for the treatment of burns and non-healing wounds.
- Human burn eschar and debrided adipose tissue cells seeded on acellular amniotic matrix, intended for the treatment of burns and non-healing wounds.
- Human burn eschar and debrided adipose tissue cells seeded on acellular dermal matrix, intended for the treatment of burns and non-healing wounds.
- Co-culture of keratinocytes and human burn eschar and debrided adipose tissue cells seeded on acellular amniotic matrix, intended for the treatment of burns and non-healing wounds.
- Co-culture of keratinocytes and human burn eschar and debrided adipose tissue cells seeded on acellular dermal matrix, intended for the treatment of burns and non-healing wounds.
- Autologous cells of stromal vascular fraction and autologous adipose derived stem cells intended for the treatment of keloid scars.

The following product was classified as not an ATMP:

- Recombinant non-replicative serotype 5 human adenovirus containing sequences coding for the core protein, polymerase protein and selected domains of the envelope protein of hepatitis B virus (genotype D), intended for the treatment of chronic hepatitis B.

CAT received 13 new ATMP classification requests for which a scientific recommendation will be delivered within 60 days (active review time).

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

European Medicines Agency - ATMP classification

Organisational, regulatory and methodological matters

- CAT finalised its proposal of similarity of two active substances for the determination of orphan similarity for ATMPs. This proposal will now be merged with the proposals for chemicals and biologicals and transmitted to the European Commission.
- CAT discussed the organisation of the CAT assessor training (23-24 June 2016) and the Workshop on cell-based cancer immunotherapy products.

ATMP cluster teleconferences with international regulators

CAT welcomed the colleagues from the Japanese Pharmaceutical and Medical Device Agency (PMDA) at the 2-monthly ATMP cluster teleconference calls with US-FDA and Health Canada.

Already from the start of the CAT, regular teleconferences with US-FDA take place to discuss ATMP related aspects. Health Canada joined the ATMP cluster teleconferences in 2012 and from now on,

PMDA will also participate. Because of the confidentiality agreements between the 3 regulatory authorities, confidential (product related) and non-confidential topics can be discussed.

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	1	8 Corresponding to 7 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									3

[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	3	21

Scientific recommendation on advanced therapy classification									
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**Total**
Submitted	22	19	12	22	20	28	61	28	212
Adopted	12	27	12	16	23	29	31	43	193

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	0	6

Scientific advice procedure for ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Discussed*	25	30	36	31	36	48	63	19	288
Number of procedures	17	19	21	19	23	33	39	19	190

* 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	2	45

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

Upcoming meetings following the March 2016 CAT meeting

The 81st meeting of the CAT will be held on 20-21 April 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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