EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

26 May 2016
EMA/CAT/358066/2016
Procedure Management and Committees Support Division

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

May 2016 meeting

The Committee for Advanced Therapies (CAT) held its 82nd CAT meeting on 18 – 20 May 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.

Scientific recommendation on advanced therapy product classification

Further to consultation with the European Commission, the CAT finalised 12 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Live-attenuated, double-deleted *Listeria monocytogenes* expressing human mesothelin intended for the treatment of malignant non-small cell lung cancer.
- Live-attenuated, double-deleted *Listeria monocytogenes* expressing prostate antigens intended for the treatment of malignant prostate cancer.

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

- Allogeneic bone marrow-derived expanded mesenchymal stem cells, intended for the treatment of acute graft-versus-host disease grades III and IV resistant to first line treatment.

The following products were classified as tissue engineered products:

- Concentrate of autologous bone marrow-derived mononuclear cells intended for the Chronic Myocardial Ischemia with left ventricular dysfunction.
- Autologous cultured fibroblast used in the treatment of tissue loss and to close chronic non-closing injuries (such as diabetic foot and venous ulcers) and in the treatment of acne scars.
- Autologous cultured adipose derived mesenchymal stem cells intended for the treatment of non-healing wounds.
- Autologous cultured chondrocytes intended for the treatment of cartilage loss in the knee.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

- Autologous cultured fibroblasts intended for the filling of skin connective tissue loss (skin wounds).
- Autologous cultured keratinocytes intended for the treatment on non-healing wounds, burns and trophic ulcers.
- Autologous cultured muscle-derived stem cells intended for the treatment of faecal and urinary incontinence and of skeletal muscle injury.
- Autologous cultured melanocytes intended for the treatment of vitiligo.

The following product was classified as not an ATMP:

- Extracellular matrix from adipose tissue, intended for the treatment of soft tissue damage (fistula-in-ano, trophic ulcers, burns), cartilage defects as well as large tissue damage after cancer resection.

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

CAT also clarified the legal disclaimer for the ATMP classification reports. The new disclaimer reads: "The present scientific recommendation refers exclusively to the case as presented to the Agency without prejudice to future evaluations by the Agency. It is stressed that the scientific recommendation on advanced therapy classification does not amount to any endorsement of the plausibility of the product, including the mode of action or therapeutic indication(s) claimed by the applicant."

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

European Medicines Agency - ATMP classification

PRIME

CAT contributed, for the first time, to the review of eligibility requests for PRIME for ATMPs. The outcome of PRIME eligibility will be included in the CHMP meeting highlights.

Organisational, regulatory and methodological matters

- CAT discussed the agenda of the upcoming 'Strategic Review and Learning Meeting' that will be held on 1 – 2 June 2016 under the auspices of the Dutch Presidency of the Council of the European Union. This meeting will be held jointly with the Paediatric Committee (PDCO) and the Clinical Trial Facilitation Group (CTFG).

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	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	36	220
Adopted	12	27	12	16	23	29	31	61	211

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	32	301
Number of procedures	17	19	21	19	23	33	39	26	197

* 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 May 2016 CAT meeting

The 83rd meeting of the CAT will be held on 16-17 June 2016.

The Strategic Review and Learning meeting will be held in Utrecht (The Netherlands) on 1-2 June 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)

Sheila Kennedy
Head of Committees Secretariat Service
Tel.: (+44-20) 3660 8508
Fax: (+44-20) 3660 5520
AdvancedTherapies@ema.europa.eu