21 June 2017
EMA/CAT/394234/2017
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

June 2017 meeting

The Committee for Advanced Therapies (CAT) held its 94th CAT meeting on 15 – 16 June 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 14 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Autologous human adipose-derived perivascular stromal cells genetically modified to secrete soluble TRAIL ligand intended for the treatment of TRAIL-sensitive cancers such as Ewing sarcoma and pancreatic ductal adenocarcinoma.
- Replication incompetent adenoviral vector encoding Interleukin 12 with activator ligand, intended for the treatment of patients with recurrent or progressive glioblastoma multiforme.
- Adenovirus-associated viral vector serotype 5 containing CRISPR Cas9 and guide RNAs targeting intron 26 of the CEP290 gene intended for the treatment of Leber Congenital Amaurosis type 10 (LCA10) caused by a homozygous or compound heterozygous intron 26 mutation in the CEP290 gene.

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

- Autologous adipose-derived mesenchymal stem cells intended for the treatment of autoimmune drug resistant epilepsy.
- Cultured autologous adipose-derived mesenchymal stem cells intended for the treatment of autoimmune drug resistant epilepsy.
- Cultured autologous adipose-derived regenerative mesenchymal stem cells intended for the treatment of autoimmune drug resistant epilepsy.

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The following products were classified as tissue engineered products:

- Allogeneic human mesenchymal stem cells derived from Wharton's jelly tissue of umbilical cord intended for the treatment of Chronic obstructive pulmonary disease (COPD).
- Cultured allogeneic Wharton's jelly derived mesenchymal stem cells intended for the treatment of amyotrophic lateral sclerosis.
- Cultured autologous Wharton's jelly derived mesenchymal stem cells intended for the treatment of amyotrophic lateral sclerosis.
- Allogeneic suspension of unexpanded and uncultured human amniotic fluid-derived cells intended for the treatment of chronic, non-healing wounds.
- Human autologous stromal vascular fraction (SVF) intended for the treatment of articular cartilage and bone defects.
- Human autologous adipose-derived stromal/stem cells intended for the treatment of articular cartilage and bone defects.
- Human cultured dermal fibroblasts and human epidermal keratinocytes embedded in/on collagen hydrogel intended for the treatment of partial deep dermal and full thickness burn wounds.

The following product was classified as not an ATMP:

- Resorbable, viscoelastic matrix intended to serve as a temporary resorbable 3-dimensional matrix when combined with cells.
  The classification of the matrix as non-ATMP does not pre-empt the classification of the finished product, which is the cells delivered with the matrix, as an ATMP.

Organisational matters

- CAT discussed and endorsed the GMP for ATMP guideline. This guideline has been developed jointly by the CAT and the GMP-inspectors working group. The European Commission will publish the guideline.
- CAT discussed the preparation of the Expert Meeting on adeno-associated viral (AAV) vectors that will take place on 6 September 2017. During this meeting, CAT will discuss with invited expert aspects related to the quality, non-clinical and clinical development of gene therapy medicinal product based on AAV vectors. This meeting is not open to the public.

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	1	16
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	1	10*
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 9 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	2	26

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	28	272
Adopted	12	27	12	16	23	29	31	87	30	267

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	1	10
Adopted	0	1	0	1	1	2	1	1	2	9

Scientific advice procedure for ATMPs										
	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Number of procedures	17	19	21	19	23	33	39	46	28	245

Paediatric Investigation Plans (PIP) for ATMPs										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Number of procedures	3	4	4	8	5	4	3	5	1	37

Prime Eligibility for ATMPs									
	2016	**2017**							**Total**
Discussed	22	10							32
Granted	8	4							12

Upcoming meetings following the June 2017 CAT meeting

The 95th meeting of the CAT will be held on 12 – 14 July 2017.

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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