24 September 2015
EMA/CAT/632147/2015
Procedure Management and Business Support Division

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

September 2015 meeting

The Committee for Advanced Therapies (CAT) held its 74th CAT meeting on 17 – 18 September 2015.

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 two scientific recommendations on the following classification of advanced therapy medicinal products (ATMP).

The following product was classified as gene therapy medicinal product:

- Adeno-associated virus vector serotype rh10 encoding human factor IX, intended for the treatment of haemophilia B.

The following product was classified as a tissue engineered product:

- Autologous bone marrow derived cells intended for the treatment of peripheral artery disease and critical limb ischemia.

CAT received 9 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 members will contribute to the ADAPT-SMART (Accelerated Development of Appropriate Patient Therapies: a Sustainable, Multi-stakeholder Approach from Research to Treatment-outcomes) project. Further information can be found here: http://adaptsmart.eu/press-release-innovative-medicines-initiative-launches-adapt-smart-an-adaptive-pathways-project-with-32-international-participants/

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

- CAT was informed about the activities of the International Pharmaceutical Regulators Forum (IPRF) cell therapy and gene therapy products working groups. Feedback was provided of the discussion at the meeting of the IPRF gene therapy working group that took place in New Orleans (USA) on 13-16 May 2015. Nicolas Ferry attended this meeting on behalf of CAT / EU. More information on IPRF can be found at the IPRF website.
- CAT discussed the Guideline developed by the International Society for Stem Cell Research (ISSCR) on stem cell science and clinical transformation. The CAT input will be consolidated with the input from other Committees and Working Parties and transmitted to ISSCR.
- CAT was made aware of the recent confidentiality agreements with SwissMedic and WHO. Such confidentiality agreements are already in place with US-FDA, Japan PMDA/MHLW, Health Canada and TGA Australia.

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**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	14
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	0	6 Corresponding to 5 ATMPs
Negative draft opinions	1[i]	0	1[ii]	0	0	0	1	3
Withdrawals	1	1[i]	0	0	2	0	0	4
Ongoing MAAs								4

[i] Same product (Cerepro)
[ii] Same product (Glybera)

Variations (Type II) for authorised ATMP								
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Positive draft Opinion	0	0	1	1	9	4	3	18

Scientific recommendation on advanced therapy classification								
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Submitted	22	19	12	22	20	28	22	145
Adopted	12	27	12	16	23	29	17	136

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs								
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Submitted	1	0	0	1	3	1	1	7
Adopted	0	1	0	1	1	2	1	6

Scientific advice procedures on ATMPs								
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**Total**
Discussed*	25	30	36	31	36	48	49	255
Number of procedures	17	19	21	19	23	33	32	164

* 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**	**Total**
Discussed*	4	7	6	9	7	7	1	41

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

Upcoming meetings following the September 2015 CAT meeting

The 75th meeting of the CAT will be held on 15 – 16 October 2015.

The joint CAT-ISCT workshop on Challenges and Opportunities for a successful development and approval of ATMPs will take place in Seville, Spain on 25 September 2015. For more information and registration, visit the website of ISCT Europe 2015.

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)

Tony Humphreys
Head of Scientific Committee Support
Tel.: (+44-20) 3660 8583
Fax: (+44-20) 3660 5520
AdvancedTherapies@ema.europa.eu