21 April 2017
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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

April 2017 meeting

The Committee for Advanced Therapies (CAT) held its 92nd CAT meeting on 10 – 12 April 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 6 scientific recommendations on the classification of advanced therapy medicinal products.

The following products were classified as gene therapy medicinal products:

- Recombinant adeno-associated virus expressing the thyroxine-binding globulin human uridine diphosphate glycuronosyltransferase 1A1, intended for the treatment of Crigler-Najjar syndrome.
- Implantable continuous glucose monitoring system based on genetically modified cells, to be used as adjunct glucose monitoring in diabetic patients.
- Recombinant oncolytic adenovirus intended for the treatment of pancreatic cancer.

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

- Autologous bone marrow derived mesenchymal stem cells intended for the treatment of coma (brain injury, stroke).
- Banked allogeneic leukocytes for the treatment of pancreatic ductal adenocarcinoma.

  This product was classified by CAT as a non-ATMP in December 2016. The company resubmitted a classification request for this product with additional information. On basis of the new information provided, CAT classified this product as a somatic cell therapy product.

The following product was classified as a tissue engineered product:

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- Allogeneic umbilical cord derived mesenchymal stem cells intended for the treatment of intervertebral disc regeneration

Development of non-substantially manipulated cell-based ATMPs: flexibilities introduced via the application of the risk-based approach.

CAT adopted the document on entitled: *Development of non-substantially manipulated cell-based ATMPs: flexibilities introduced via the application of the risk-based approach*.

The document illustrates some to the possibilities and limitations of the risk-based approach. It provides a further example of how to apply the Guideline on the risk-based approach for a hypothetical ATMP based on cells that have not been subjected to substantially manipulation and that are not intended for the same essential function.

The document will be published shortly.

Organisational matters

CAT discussed the procedure advice on the evaluation of ATMPs. The procedural advice (developed in 2009) has been updated to further clarify the roles of CAT, CHMP and PRAC during the initial evaluation and the re-examination of marketing authorisation applications for ATMPs. The revised document will be adopted jointly by the three involved Committees.

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	0	15
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	0	9*
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 8 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	1	25

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	24	268
Adopted	12	27	12	16	23	29	31	87	14	251

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	18	235

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	9							31
Granted	8	3							11

Upcoming meetings following the April 2017 CAT meeting

The 93rd meeting of the CAT will be held on 10 – 12 May 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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