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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

June 2018 meeting

The Committee for Advanced Therapies (CAT) held its 105th CAT meeting on 20 - 22 June 2018.

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 two CAR-T cell products: Yescarta and Kymriah

Yescarta (axicabtagene ciloleucel) and Kymriah (tisagenlecleucel) are genetically modified T-cells expressing on their surface the Chimeric Antigen Receptor (CAR) that recognises the CD19 antigen that is present on some cancer cells. These autologous immunocellular cancer therapy products will detect and eliminate CD19 expressing cells via the immunological action of the T-cells.

Following an in-depth review of the marketing authorisation applications submitted by Kite Pharma EU B.V. for Yescarta and Novartis Europharm Limited for Kymriah, CAT concluded during its June meeting that a positive benefit risk has been demonstrated for both products. CAT adopted positive draft opinions recommending the granting of the marketing authorisations for both products. The CHMP subsequently adopted positive opinions for Yescarta and Kymriah during its June 2018 meeting.

Yescarta is indicated for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) and primary mediastinal large B-cell lymphoma, after two or more lines of systemic therapy. Kymriah is indicated for the treatment of paediatric and young adult patients (up to 25 years of age) with B-cell acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse, and in adult patients with relapsed or refractory DLBCL after two or more lines of systemic therapy.

Scientific recommendation on advanced therapy product classification

Further to consultation with the European Commission, the CAT finalised 3 scientific recommendations on the classification of advanced therapy medicinal products.

The following product was classified as a tissue engineered product:

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- Human olfactory ensheathing cells and human olfactory nerve fibroblast intended for the treatment of complete spinal cord injury.

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

- CD34+ cells transduced with a lentiviral vector encoding the Fanconi anaemia complementation group A (FANCA) gene, intended for the treatment of Fanconi anaemia type A patients.

The following product was classified as not an ATMP:

- Donor derived CD34+ haematopoetic stem cells and donor derived CD3+ T-cells, intended for the prevention of kidney graft loss.

Organisational matters

- CAT had a first discussion on its Work Plan for 2019.

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**	**2018**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	4	1	20
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	2	2	13*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	0	4
Ongoing MAAs											3

*** Corresponding to 12 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**	**2018**	**Total**
Positive Opinion	0	0	1	1	9	4	3	6	3	4	31

Scientific recommendation on advanced therapy classification											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Submitted	22	19	12	22	20	28	61	60	46	27	317
Adopted	12	27	12	16	23	29	31	87	49	19	305

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Submitted	1	0	0	1	3	1	1	2	2	0	11
Adopted	0	1	0	1	1	2	1	1	3	1	11

Scientific advice procedure for ATMPs											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Number of procedures	17	19	21	19	23	33	39	46	55	30	302

Paediatric Investigation Plans (PIP) for ATMPs											
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**2018**	**Total**
Number of procedures	3	4	4	8	5	4	3	5	3	2	41

Prime Eligibility for ATMPs									
	2016	**2017**	**2018**						**Total**
Discussed	22	16	8						46
Granted	8	6	4						18

Upcoming meetings following the May 2018 CAT meeting

- The 106th meeting of the CAT will be held on 18 – 20 July 2018.

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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