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Human Medicines Division

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

May 2021 meeting

The Committee for Advanced Therapies (CAT) held its 137th meeting on 10 – 12 May 2021.

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 authorisations for Skysona

During its May 2021 meeting, the CAT adopted a draft opinion recommending the granting of a marketing authorisation to Skysona.

Skysona is a gene therapy medicinal product indicated for the treatment of early cerebral adrenoleukodystrophy (CALD). The active substance of Skysona is elivaldogene autotemcel.

Following an in-depth review of the marketing authorisation application submitted by bluebird bio (Netherlands) B.V., CAT concluded that a positive benefit risk has been demonstrated for Skysona. CAT adopted a positive draft opinion recommending the granting of a marketing authorisation. The CHMP subsequently adopted a positive opinion for Skysona during its May 2021 meeting.

Further information on Skysona can be found here.

Scientific recommendation on advanced therapy product classification[1]

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:

- Oncolytic adenovirus, intended for the treatment of histologically and radiologically confirmed progressive neuroendocrine neoplasm (NEN) of gastrointestinal, pancreatic or bronchial origin with multiple liver metastases;

[1] 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.

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- DNA plasmid encoding several neoepitopes from the tumour of a patient, a live wild-type modified vaccinia strain Ankara and a monoclonal antibody against Cytotoxic T-lymphocyte associated protein 4, intended for the treatment of cancer;
- Recombinant adeno-associated virus encoding for the human α-sarcoglycan-protein, intended for the treatment of patients with a confirmed diagnosis of Limb-Girdle muscular dystrophy Type 2D/R3.

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

- Autologous antigen presenting cells loaded with SARS-CoV-2 antigen, intended to be used as vaccines against SARS-CoV-2.

The following product was classified as a tissue engineered product:

- Autologous cultured chondrocytes, intended for the repair of cartilage defects.

The following product was classified as a tissue engineered product and a combined ATMP:

- Autologous mesenchymal stem cells combined with a matrix pre-loaded with BMP2, intended to treat femoral osteochondral lesion (grade III to IV).

Organisational matters

- Feedback was provided on the review of the activities on the EMA working parties.
- CAT noted the publication of the Questions and Answers on the principles of GMP for the manufacturing of starting materials of biological origin used to transfer genetic material for the manufacturing of ATMPs.
- Reduction of the non-Covid related workload: CAT agreed to stop appointing a peer reviewer and abandon the CAT peer review activity for new initial marketing authorisation applications. More information can be found here.

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								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted MAAs	14	1	4	3	2	8	0	32
Positive draft Opinion	7 [i]	2	2	3	1	3	1	19*
Negative draft opinions	4 [i,ii,iii]	0	0	0	0	0	0	4
Withdrawals	4 [ii]	0	0	1	1[iv]	2[v]	0	8
Ongoing MAAs								5

*** Corresponding to 18 ATMPs**

[i] One negative draft opinion and two positive draft opinions for the Glybera

[ii] Negative draft opinion and withdrawal for the Cerepro

[iii] Two negative draft opinions for Heparesc

[iv] Luxceptar

[v] Roctavian; Artobend

Variations (Type II) for authorised ATMP								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Positive opinion	18	6	3	8	16	27	17	95

Scientific recommendation on advanced therapy classification								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted	184	60	46	55	70	74	34	523
Adopted	150	87	49	43	67	87	32	515

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Submitted	7	2	2	1	1	0	0	14
Adopted	6	1	3	1	1	2	0	14

Scientific advice procedure for ATMPs								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Number of procedures	171	46	55	53	56	61	19	461

Paediatric Investigation Plans (PIP) for ATMPs								
	2009-2015	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Number of procedures	31	5	3	3	2	1	0	45

Prime Eligibility for ATMPs							
	2016	**2017**	**2018**	**2019**	**2020**	**2021**	**Total**
Discussed	22	16	14	16	23	8	99
Granted	8	6	6	10	9	4	43

Upcoming meetings following the May 2021 CAT meeting

- The Strategic Review & Learning meeting (SRLM) under the Portuguese presidency of the European Union will be held (virtually) on 27 May 2021.
- The 138th meeting of the CAT will be held on 16 – 18 June 2021.

NOTE:

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

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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