5 May 2022
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Human Medicines Division

CAT quarterly highlights and approved ATMPs [1]

April 2022

This report provides information on ATMP approvals and extension of indications of authorised ATMPs, as well as statistical data on product-related activities (including type II variations, CAT scientific recommendations on Advanced Therapy Medicinal Product (ATMP) classification, certifications, and on CAT contributions to Scientific Advice, Paediatric Investigation Plans and PRIME (priority of medicines) eligibity requests.

The period covered by this report is: January – April 2022.

Advanced therapy medicinal products approvals

During its plenary meeting of January 2022, CAT adopted a positive draft opinion for **Breyanzi** (lisocabtagene maraleucel) for the following indication: treatment of adult with relapsed or refractory diffuse large B cell lymphoma (DLBCL), primary mediastinal large B-cell lymphoma (PMBCL) and follicular lymphoma grade 3B (FL3B), after at least two previous lines of treatments. Based on the assessment of the CAT, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Breyanzi. More information on Breyanzi can be found in the EPAR.

During its plenary meeting of March 2022, CAT adopted a positive draft opinion for **Carvykti** (ciltacabtagene autoleucel) for the following indication: treatment of adult patients with relapsed and refractory multiple myeloma, who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody, and have demonstrated disease progression on the last therapy. Based on the assessment of the CAT, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Carvykti. More information on Carvykti can be found in the Summary of opinion.

Extension of indication of authorised ATMPs

During its plenary meeting of March 2022, CAT adopted an extension of indication for **Kymriah** to include the treatment of adult patients with follicular lymphoma after two or more lines of therapy who

[1] The CAT quarterly statistics replaces the CAT monthly report.

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are refractory or relapsed during or within 6 months after completion of anti-CD20 antibody maintenance or relapsed after autologous haematopoietic stem cell transplantation.

During its plenary meeting of April 2022, CAT adopted an extension of indication for **Yescarta** to include the treatment of adult patients with relapsed or refractory follicular lymphoma after three or more lines of systemic therapy.

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				
	2009-2020	2021	2022	Total
Submitted MAAs	32	3	1	36
Positive draft Opinion	18 [i]	2	2	22*
Negative draft opinions	4 [i,ii,iii]	0	0	4
Withdrawals	8 [ii, iv]	0	0	8
Ongoing MAAs				6

*** Corresponding to 21 ATMPs (see List of authorised 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, Roctavian, Artobend

Variations (Type II) for authorised ATMP				
	2009-2020	2021	2022	Total
Positive opinion	78	32	14	124

Scientific recommendation on advanced therapy classification [2]				
	2009-2020	2021	2022	Total
Submitted	489	66	13	568
Adopted	483	61	16	560

[2] More information on the scientific recommendation on advanced therapy classification and the summaries of ATMP classification can be found on the ATMP classification webpage.
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.

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs[3]				
	2009-2020	2021	2022	Total
Adopted	14	0	0	14

Scientific advice procedure for ATMPs				
	2009-2020	2021	2022	Total
Number of procedures	442	64	16	522

Paediatric Investigation Plans (PIP) for ATMPs				
	2009-2020	2021	2022	Total
Number of procedures	45	0	0	45

PRIME[4] Eligibility for ATMPs				
	2016-2020	2021	2022	Total
Discussed	91	14	1	106
Granted	39	7	0	46

[3] More information on the ATMP certification procedure can be found ATMP certification webpage.
[4] PRIority MEdicines (PRIME) scheme. More information can be found at the PRIME webpage.

List of authorised ATMPs

NAME	Type of ATMP	Authorisation Date	Orphan	PRIME[5]	Comment
Chondrocelect	TEP	5/10/2009	No	No	MA withdrawn July 2016
Glybera	GTMP	25/10/2012	Yes	No	MA not renewed (MA ended Oct. 2017)
MACI	TEP, combined ATMP	27/06/2013	No	No	MA not renewed (MA ended June 2018)
Provenge	CTMP	6/09/2013	No	No	MA withdrawn May 2015
Holoclar	TEP	17/02/2015	Yes	No	
Imlygic	GTMP	16/12/2015	No	No	
Strimvelis	GTMP	26/05/2016	Yes	No	
Zalmoxis	CTMP	18/08/2016	Yes	No	MA withdrawn Oct. 2019
Spherox	TEP	10/07/2017	No	No	
Alofisel	CTMP	23/03/2018	Yes	No	
Yescarta	GTMP	23/08/2018	Yes	Yes	
Kymriah	GTMP	23/08/2018	Yes	Yes	
Luxturna	GTMP	22/11/2018	Yes	No	
Zynteglo	GTMP	29/05/2019	Yes	Yes	MA withdrawn March 2022
Zolgensma	GTMP	18/05/2020	Yes	Yes	
Libmeldy	GTMP	17/12/2020	Yes	No	
Tecartus	GTMP	14/12/2020	Yes	Yes	
Skysona	GTMP	16/07/2021	Yes	Yes	MA withdrawn Nov. 2021
Abecma	GTMP	18/08/2021	Yes	Yes	
Breyanzi	GTMP	4/04/2022	No	Yes	

[5] PRIME (PRIority MEdicines scheme) was set up in March 2016 to provide early and enhanced scientific and regulatory support to medicines that have the potential to significantly address patients' unmet medical needs.

NAME	Type of ATMP	Authorisation Date	Orphan	PRIME [5]	Comment
Carvykti	GTMP	Positive opinion March 2022	Yes	Yes	Commission decision pending

More information on authorised products can be found on: www.ema.europa.eu (type in the product name in the search box)

Abbreviations: ATMP: advanced therapy medicinal product; GTMP: gene therapy medicinal product; CTMP: cell therapy medicinal product; TEP: tissue engineered product; MA: Marketing authorisation