EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Division

CAT quarterly highlights and approved ATMPs

January 2023

This report provides information on Advanced Therapy Medicinal Products (ATMPs) approvals and extension of indications of authorised ATMPs, as well as statistical data on product-related activities.

The period covered by this report is: November 2022 – January 2023.

Advanced therapy medicinal products approvals

During its plenary meeting of December 2022, CAT adopted a positive draft opinion for **Hemgenix** (etranacogene dezaparvovec) for the following indication: treatment of severe and moderately severe Haemophilia B (congenital Factor IX deficiency) in adult patients without a history of Factor IX inhibitors. 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 conditional marketing authorisation for the medicinal product Hemgenix. More information on Hemgenix can be found in the Summary of opinion.

Extension of indication of authorised ATMPs

No extensions of indication of authorised ATMPs in the period covered by this report.

Overview of product-related activities

The Committee discussed ongoing evaluation procedures for ATMPs and other related procedures as summarised in the following tables:

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Initial Evaluation of Marketing Authorisation Applications (MAA) for ATMP					
	2009-2020	**2021**	**2022**	**2023***	**Total**
Submitted MAAs	32	3	1	0	36
Positive draft Opinion	18 [i]	2	6	0	26[#]
Negative draft opinions	4 [i,ii,iii]	0	0	0	4
Withdrawals	8 [ii, iv]	0	1[v]	0	9
Ongoing MAAs					1

 # Corresponding to 25 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

[v] Sitoiganap

Variations (Type II) for authorised ATMP					
	2009-2020	**2021**	**2022**	**2023***	**Total**
Positive opinion	78	32	47	1	158

Scientific recommendation on advanced therapy classification[1]					
	2009-2020	**2021**	**2022**	**2023***	**Total**
Submitted	489	66	51	5	611
Adopted	483	61	46	7	597

Scientific advice procedure for ATMPs					
	2009-2020	**2021**	**2022**	**2023***	**Total**
Number of procedures	442	64	53	6	559

PRIME[2] Eligibility for ATMPs					
	2016-2020	**2021**	**2022**	**2023***	**Total**
Discussed	91	14	10	1	116
Granted	39	7	4	1	50

* Period: January 2023

[1] 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.

[2] PRIority MEdicines (PRIME) scheme. PRIME 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. More information can be found at the PRIME webpage.

List of authorised ATMPs

NAME	Type of ATMP	Authorisation Date	Orphan	PRIME	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	
Carvykti	GTMP	25/05/2022	Yes	Yes	
Upstaza	GTMP	18/07/2022	Yes	No	

NAME	Type of ATMP	Authorisation Date	Orphan	PRIME	Comment
Roctavian	GTMP	24/08/2022	Yes	No	
Ebvallo	CTMP	16/12/2022	Yes	Yes	
Hemgenix	GTMP	Opinion Dec. 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