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Human Medicines Division

CAT quarterly highlights and approved ATMPs

July 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) eligibility requests.

The period covered by this report is: May – July 2022.

Advanced therapy medicinal products approvals

During its plenary meeting of May 2022, CAT adopted a positive draft opinion for **Upstaza** (eladocagene exuparvovec) for the following indication: treatment of patients aged 18 months and older with a clinical, molecular, and genetically confirmed diagnosis of aromatic L-amino acid decarboxylase (AADC) deficiency with a severe phenotype. 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 under exceptional circumstances for the medicinal product Upstaza. More information on Upstaza can be found in the Summary of opinion.

During its plenary meeting of June 2022, CAT adopted a positive draft opinion for **Roctavian** (valoctocogene roxaparvovec) for the following indication: treatment of severe haemophilia A (congenital factor VIII deficiency) in adult patients without a history of factor VIII inhibitors and without detectable antibodies to adeno associated virus serotype 5 (AAV5). 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 Roctavian. More information on Roctavian can be found in the Summary of opinion.

Extension of indication of authorised ATMPs

During its plenary meeting of July 2022, CAT adopted an extension of indication for **Tecartus** to include the treatment of adult patients 26 years of age and above with relapsed or refractory B-cell precursor acute lymphoblastic leukaemia (ALL).

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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	4	24*
Negative draft opinions	4 [i,ii,iii]	0	0	4
Withdrawals	8 [ii, iv]	0	1[v]	9
Ongoing MAAs				3

*** Corresponding to 23 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	Total
Positive opinion	78	32	27	137

Scientific recommendation on advanced therapy classification [1]				
	2009-2020	2021	2022	Total
Submitted	489	66	25	580
Adopted	483	61	26	570

Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs [2]				
	2009-2020	2021	2022	Total
Adopted	14	0	0	14

[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] More information on the ATMP certification procedure can be found ATMP certification webpage.

Scientific advice procedure for ATMPs				
	2009-2020	2021	2022	Total
Number of procedures	442	64	29	535

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

PRIME[3] Eligibility for ATMPs				
	2016-2020	2021	2022	Total
Discussed	91	14	5	110
Granted	39	7	2	48

[3] 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[4]	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	

[4] 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 [4]	Comment
Upstaza	GTMP	Positive opinion May 2022	Yes	No	Commission decision pending
Roctavian	GTMP	Positive opinion June 2022	Yes	No	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