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Human Medicines Division

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

### November 2020 meeting

The Committee for Advanced Therapies (CAT) held its 131<sup>st</sup> meeting on 4 – 6 November 2020.

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.

### **Withdrawal of the marketing authorisation application for Roctavian**

CAT noted the withdrawal by Biomarin International Limited of the marketing authorisation application of Roctavian (valoctogene roxaparovec), which was intended for the treatment of severe haemophilia A. More information can be found [here](#).

### **Scientific recommendation on advanced therapy product classification<sup>1</sup>**

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

The following product was classified as gene therapy medicinal product:

- Recombinant serotype 9 adeno-associated virus encoding a codon-optimised human neuronal ceroid lipofuscinosis-5 transgene intended for the treatment of neuronal ceroid lipofuscinosis type 5.

The following product was classified as an advanced therapy medicinal product<sup>2</sup>:

- Autologous adipose-derived mesenchymal stem cell, intended for the treatment of diabetic foot syndrome.

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<sup>1</sup> 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.

<sup>2</sup> CAT was unable to consider if this product meets the definition of somatic cell therapy or tissue engineering product due to shortcomings in the information provided regarding the claimed mode of action.



## Organisational matters

CAT was informed of the revised policies for declaration of interests for committee members, experts and Management Board's members.

## 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	Total
Submitted MAAs	14	1	4	3	2	8	31
Positive draft Opinion	7 <sup>i</sup>	2	2	3	1	3	18*
Negative draft opinions	4 <sup>i,ii,iii</sup>	0	0	0	0	0	4
Withdrawals	4 <sup>ii</sup>	0	0	1	1 <sup>iv</sup>	1 <sup>v</sup>	7
Ongoing MAAs							7

**\* Corresponding to 17 ATMPs**

<sup>i</sup> One negative draft opinion and two positive draft opinions for the Glybera

<sup>ii</sup> Negative draft opinion and withdrawal for the Cerepro

<sup>iii</sup> Two negative draft opinion for Heparesc

<sup>iv</sup> Luxceptar

<sup>v</sup> Roctavian

Variations (Type II) for authorised ATMP							
	2009-2015	2016	2017	2018	2019	2020	Total
Positive opinion	18	6	3	8	16	25	76

Scientific recommendation on advanced therapy classification							
	2009-2015	2016	2017	2018	2019	2020	Total
Submitted	184	60	46	55	70	76	491
Adopted	150	87	49	43	67	81	477

### Certification of quality and non-clinical data for small and medium-sized enterprises developing ATMPs

	2009-2015	2016	2017	2018	2019	2020	Total
Submitted	7	2	2	1	1	0	14
Adopted	6	1	3	1	1	2	14

### Scientific advice procedure for ATMPs

	2009-2015	2016	2017	2018	2019	2020	Total
Number of procedures	171	46	55	53	56	55	436

### Paediatric Investigation Plans (PIP) for ATMPs

	2009-2015	2016	2017	2018	2019	2020	Total
Number of procedures	31	5	3	3	2	1	45

### Prime Eligibility for ATMPs

	2016	2017	2018	2019	2020	Total
Discussed	22	16	14	16	22	90
Granted	8	6	6	10	8	38

## Upcoming meetings following the November 2020 CAT meeting

- The 132<sup>nd</sup> meeting of the CAT will be held on 2 – 4 December 2020.

#### 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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