



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Procedure Management and Business Support Division

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

June 2015 meeting

The Committee for Advanced Therapies (CAT) held its 72nd CAT meeting on 18 – 19 June 2015.

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.

Refusal of the marketing authorisation for Heparesc (Allogeneic human heterologous liver cells)

Heparesc is a somatic cell therapy product containing living human liver cells and is intended for the treatment of children with urea cycle disorders. At the CAT meeting in April 2015, the Committee adopted a negative draft opinion for Heparesc. The CAT draft opinion and the CAT assessment were transmitted to the CHMP, who adopted during its June 2015 meeting a negative opinion, recommending the refusal of the marketing authorisation of Heparesc.

During its review, the CAT and CHMP considered that the benefits of the treatment were not sufficiently demonstrated, and that therefore, at this point in time, the benefits of Heparesc did not outweigh its risk.

More information will be published on the EMA website.

Scientific recommendation on advanced therapy product classification

CAT received two new ATMP classification requests for which a scientific recommendation will be delivered within 60 days (active review time).

Further information on the ATMP classification procedure can be found at:

[European Medicines Agency - ATMP classification](#)



CAT is organising a joint workshop with ISCT on Challenges and Opportunities for a successful development and approval of ATMPs

On 25 September 2015, a workshop will take place during the International Society for Cellular Therapy (ISCT) Europe 2015 Regional meeting in Seville, Spain. During this workshop, CAT wants to reach out to ATMP developers from academia, hospitals and industry with a programme aiming to provide insight of CAT's considerations and expectations on quality development and manufacturing issues, non-clinical testing and clinical development of ATMPs. The programme includes also a talk on the EMA/CAT support available to ATMP developers and will allow interacting directly with the Regulators in an open forum discussion.

The programme of this Workshop can be found [here](#). For more information and registration, visit the website of [ISCT Europe 2015](#)

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	2010	2011	2012	2013	2014	2015	Total
Submitted MAAs	3	1	2	3	2	2	1	14
Positive draft Opinion	1	0	1 ⁱ	1 ⁱ	2	1	0	6 Corresponding to 5 ATMPs
Withdrawals	1	1	0	0	2	0	0	4
Ongoing MAAs								4

ⁱ Same product (Glybera)

Variations (Type II) for authorised ATMP								
	2009	2010	2011	2012	2013	2014	2015	Total
Positive draft Opinion	0	0	1	1	9	4	0	15

Scientific recommendation on advanced therapy classification								
	2009	2010	2011	2012	2013	2014	2015	Total
Submitted	22	19	12	22	20	28	13	136
Adopted	12	27	12	16	23	29	13	132

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

	2009	2010	2011	2012	2013	2014	2015	Total
Submitted	1	0	0	1	3	1	0	6
Adopted	0	1	0	1	1	2	1	6

Scientific advice procedures on ATMPs

	2009	2010	2011	2012	2013	2014	2015	Total
Discussed*	25	30	36	31	36	48	30	245
Number of procedures	17	19	21	19	23	33	22	156

* Most scientific advices for ATMPs are discussed by the CAT at 2 time points during the SA procedure

Paediatric Investigation Plans (PIP) for ATMPs

	2009	2010	2011	2012	2013	2014	2015	Total
Discussed*	4	7	6	9	7	7	1	41

* PIPs for ATMPs are discussed by the CAT once or twice during the procedure

Upcoming meetings following the June 2015 CAT meeting

The 73rd meeting of the CAT will be held on 16 – 17 July 2015.

NOTE:

1. 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](#)
2. 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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