EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The Committee for Advanced Therapies (CAT)

Dr Kieran Breen

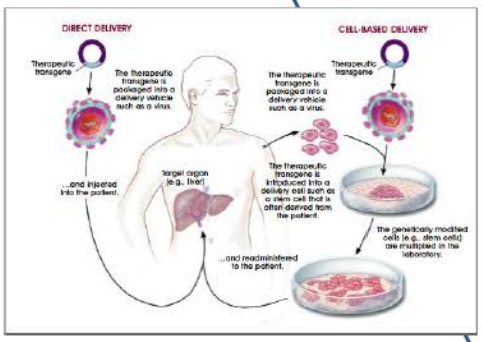
Advanced therapy medicinal products (ATMPs)

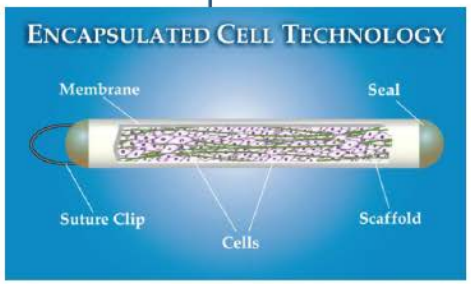
Gene Therapy Medicinal Products

Somatic Cell Therapy Medicinal Products

Tissue Engineering Products

Genetically modified cells






Severe burn victim before and 6 months after treatment with Dermagraft.

medical device + ATMP → combined ATMP

Advanced Therapeutic Medicinal Products

Initial Evaluation of Marketing Authorisation Applications (MAA) for ATMP									
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	15
Positive draft Opinion	1	0	1[II]	1[II]	2	1	1	2	9 Corresponding to 8 ATMPs
Negative draft opinions	1[I]	0	1[II]	0	0	0	2[*]	0	4
Withdrawals	1	1[I]	0	0	2	0	0	0	4
Ongoing MAAs									2

[I] Same product (Cerepro)
[II] Same product (Glybera)
[*] CAT adopted two negative draft opinions for the same product (Heparesc)

Jan – Nov 2016

CAT procedures

Scientific recommendation on advanced therapy classification									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Submitted	22	19	12	22	20	28	61	55	239
Adopted	12	27	12	16	23	29	31	86	236

Scientific advice procedure for ATMPs									
	2009	2010	2011	2012	2013	2014	2015	2016	Total
Discussed*	25	30	36	31	36	48	63	60	329
Number of procedures	17	19	21	19	23	33	39	44	215

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

Jan – Nov 2016

The PRIME programme

Once a candidate medicine has been selected for PRIME, the Agency will:

- appoint a **rapporteur** from the Committee for Medicinal Products for Human Use (CHMP) or from the Committee on Advanced Therapies (CAT) in the case of an advanced therapy to provide continuous support and **help to build knowledge ahead of a marketing-authorisation application;**
- organise a **kick-off meeting** with the CHMP/CAT rapporteur and a multidisciplinary group of experts, so that they provide **guidance on the overall development plan and regulatory strategy;**
- assign a **dedicated contact point;**
- provide **scientific advice at key development milestones**, involving additional stakeholders such as health-technology-assessment bodies, to facilitate quicker access for patients to the new medicine;
- confirm potential for accelerated assessment at the time of an application for marketing authorisation.

	2016
Discussed	21
Granted	7

CAT Workplan 2016 (1)

- Development of a guideline on the quality, non-clinical and clinical requirements for applications for clinical trials for ATMPs with a primarily focus on early trial requirements.
- Development of a Questions and Answers document for minimally manipulated ATMPs and the application of the risk based approach for these products
- Assist the Commission to finalise the GMP requirements for ATMPs
- Assist the COMP/Commission with the revision of the Orphan legislation, with regard to the definition of 'Principal Molecular Structural Features' (PMSF) for ATMPs

CAT Workplan 2016 (2)

- Streamline the process of the CAT-CHMP evaluation of ATMPs including a revision of the Procedural advice guidance on the evaluation of ATMPs
- Organisation of training for assessors who are involved in the review of regulatory applications for ATMPs
- Simplification of procedures and requirements for ATMPs in the post-authorisation phase
- Organisation of a scientific Workshop on cell-based cancer immunotherapy products
- Contribute to EMA activities related to extrapolation (lead committee: PDCO)

Thank you!