EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The Committee for Advanced Therapies (CAT)

Dr Kieran Breen

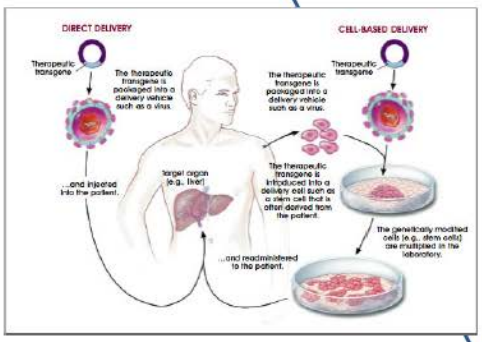
Advanced therapy medicinal products (ATMPs)

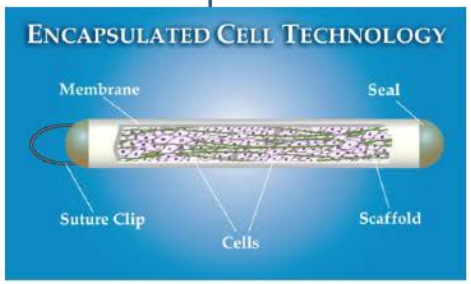
Gene Therapy Medicinal Products

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**	**2017**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	0	15
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	0	9*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	4
Ongoing MAAs										2

*** Corresponding to 8 ATMPs**

[I] Same product (Cerepro)

[ii] Same product (Glybera)

[iii] CAT adopted two negative draft opinions for the same product (Heparesc)

2016 – Strimvelis (Gene therapy for immunodeficiency disease)
- Zalmoxis (Cell therapy for host versus graft disease)

Feb 2017

CAT procedures

Scientific recommendation on advanced therapy classification										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Submitted	22	19	12	22	20	28	61	60	8	252
Adopted	12	27	12	16	23	29	31	87	7	244

Scientific advice procedure for ATMPs										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Discussed*	25	30	36	31	36	48	63	67	15	351
Number of procedures	17	19	21	19	23	33	39	46	12	229

Feb 2017

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.

The PRIME programme

Prime Eligibility for ATMPs									
	2016	**2017**							**Total**
Discussed	22	5							27
Granted	8	2							10

Feb 2017

Additional activities for 2017

- Revision of the Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells
- Reflection on the Benefit-Risk assessment of ATMPs
- CAT discussion on the use of registry data for the approval and post-marketing follow-up of ATMPs (with cross-committee Patient Registry Initiative)
- Scientific and regulatory considerations on gene editing technologies
- Contribution to the European Commission discussions on GMO related issues
- Explore the possibility to set up and define the activities of an *ad hoc* COMP-CAT working group

Thank you!