EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Committee for Advanced Therapies (CAT) update

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Committee for Advanced Therapies (CAT)

- EMA's scientific committee for the evaluation of advanced therapy medicinal products (ATMPs)
  - Provision of scientific recommendations on ATMP classification
  - Contribution to early discussions with developers via ATMP classifications, scientific-advice requests and collaboration with the Agency's Innovation Task Force offers a platform for ATMP-related
  - Contribution to scientific advice for ATMPs
  - Preparation of scientific guidelines in the fields of gene- and cell-therapy and tissue-engineered products
  - Scientific evaluation of quality and non-clinical data for certification procedures
- Leads discussions among national EU authorities and engages with global regulatory authorities on international standardisation discussions.

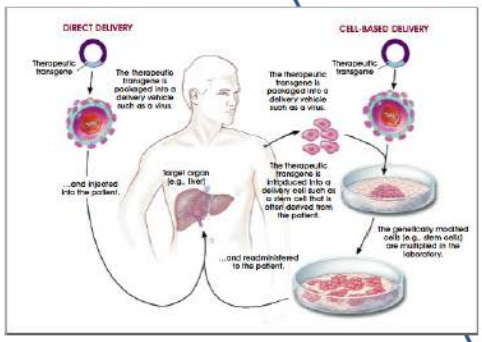
Advanced therapy medicinal products (ATMPs)

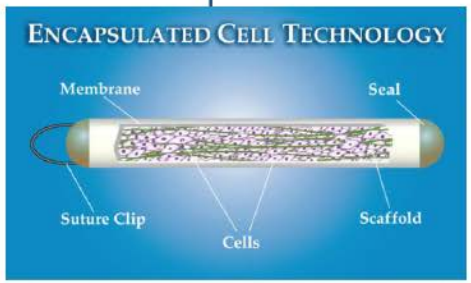
Gene Therapy Medicinal Products

Somatic Cell Therapy Medicinal Products

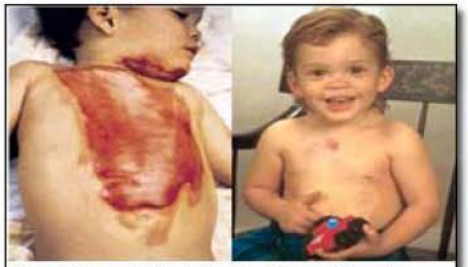
Tissue Engineering Products

Genetically modified cells



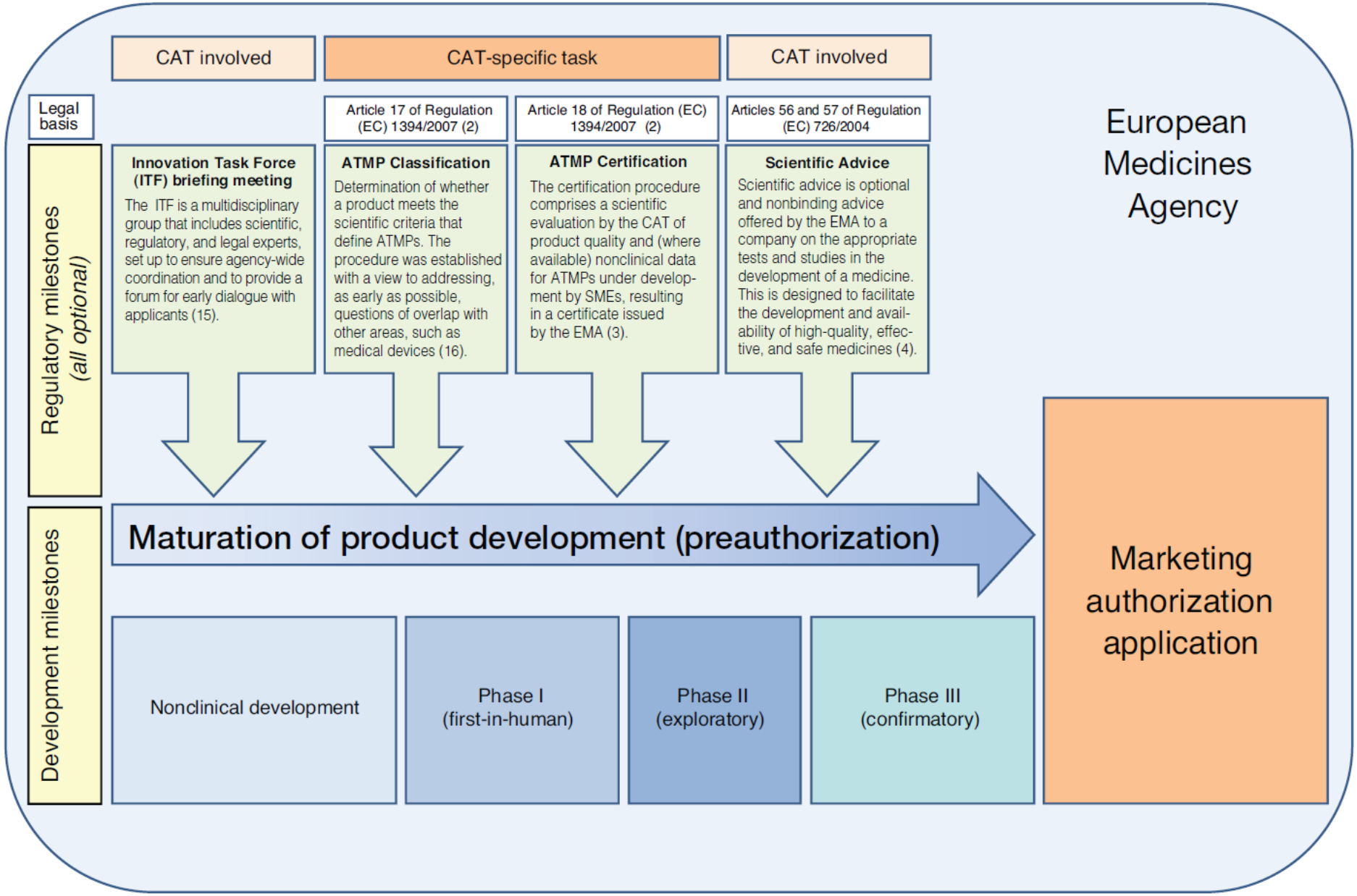


Total Length is 6 mm



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

medical device + ATMP → combined ATMP


CAT involved
CAT-specific task
CAT involved
Legal basis
Article 17 of Regulation (EC) 1394/2007 (2)
Article 18 of Regulation (EC) 1394/2007 (2)
Articles 56 and 57 of Regulation (EC) 726/2004
European Medicines Agency
Regulatory milestones (all optional)
Innovation Task Force (ITF) briefing meeting
The ITF is a multidisciplinary group that includes scientific, regulatory, and legal experts, set up to ensure agency-wide coordination and to provide a forum for early dialogue with applicants (15).
ATMP Classification
Determination of whether a product meets the scientific criteria that define ATMPs. The procedure was established with a view to addressing, as early as possible, questions of overlap with other areas, such as medical devices (16).
ATMP Certification
The certification procedure comprises a scientific evaluation by the CAT of product quality and (where available) nonclinical data for ATMPs under development by SMEs, resulting in a certificate issued by the EMA (3).
Scientific Advice
Scientific advice is optional and nonbinding advice offered by the EMA to a company on the appropriate tests and studies in the development of a medicine. This is designed to facilitate the development and availability of high-quality, effective, and safe medicines (4).
Development milestones
Maturation of product development (preauthorization)
Nonclinical development
Phase I (first-in-human)
Phase II (exploratory)
Phase III (confirmatory)
Marketing authorization application

ATMPs submitted for marketing authorisation

2009	ChondroCelect	Repair damage to the cartilage in the knee	Granted
	Cerepro (2nd)	Glioma	Withdrawn – CAT negative opinion
	Contusugene	Carcinoma of the head and neck	Withdrawn (bankruptcy)
2010	Glybera	Lipoprotein lipase deficiency who have severe or multiple attacks of pancreatitis	Granted
2011	Caomecs	Limbal-stem-cell deficiency (LSCD) - an eye condition in which the patient lacks cells called limbal stem cells, which are found at the edge of the cornea	Withdrawn - CAT negative opinion
	MACI	Repair cartilage defects at the ends of the bones of the knee joint	Granted
	Provenge	Treatment of prostate cancer of using the patient's own immune cells	Granted
2013	Holocar	Ophthalmologicals	Under consideration by CHMP
	Heparesc	Other alimentary tract and metabolism products	Under consideration by CHMP
2014	Zalmoxis	Antineoplastic medicines	Under consideration by CHMP

ATMP pipeline

Scientific recommendation on advanced therapy classification							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**Total**
Submitted	22	19	12	17	20	24	119
Adopted	12	27	12	14	23	20	110

Scientific advice procedures on ATMPs							
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**Total**
Discussed*	25	30	36	31	36	37	195

Public consultation

Reflection paper on classification of advanced therapy medicinal products

Introductory statement on the changes introduced during the revision of this Reflection Paper

CAT is now operating the ATMP classification procedure for more than 5 year and has classified over 100 products based on genes, cells and tissues.

The Reflection Paper on classification of ATMPs has been updated to reflect the current thinking of the CAT on substantial manipulation and non-homologous use (see section 2.2.3).

Additional changes have been implemented throughout the text to clarify the existing concepts, e.g. the demarcation between vaccines against infectious diseases and gene therapy medicinal products (see section 2.2.2) and the Criteria for combined ATMPs (see section 2.2.4).

63 Submissions