EMA Committees: Committee for Advanced Therapies (CAT)

Introduction

The European Medicines Agency (EMA) Committee for Advanced Therapies (CAT) assesses the quality, safety and efficacy of advanced-therapy medicinal products (ATMPs). ATMPs are medicines for human use that are made from genes and cells or tissues and include gene-therapies, somatic-cell therapy medicines, tissue-engineered medicines, and combined-therapies. The following article contains more information on ATMPs and discusses the regulatory concepts and legislation that guide the CAT's activities.

EMA Committee for Advanced Therapies (CAT)

ATMPs can offer potential new treatment opportunities for many diseases such as Alzheimer's disease, cancer, genetic diseases like muscular dystrophy, or burn injuries of the skin.

There are four main groups of ATMPs:

- **Gene-therapy medicines (GTMPs)**
  GTMPs contain genes that lead to a therapeutic effect. GTMPs insert 'recombinant' genes into cells, frequently using a virus as a vector (vehicle used to transfer genetic material to a target cell) to transport the gene. A recombinant gene is a stretch of DNA that is created in the laboratory, bringing together DNA from different sources. When the gene enters the patient's cells, the cells either produce or producing a protein which may help slow down or cure a variety of diseases,

including genetic disorders, cancer, or long-term diseases.

- **Somatic-cell therapy medicines**
  Somatic-cell therapy medicines contain cells (non-sex cells) or tissues that have been manipulated to change their biological characteristics. They can be used to cure, diagnose, or prevent diseases. An example of somatic cell therapy is the use of a patient's manipulated cancer cells to fight remaining cancer cells in the body.
- **Tissue-engineered medicines**
  Tissue-engineered medicines these contain cells or tissues that have been engineered (substantially modified) so they can be used to repair, regenerate, or replace tissue. An example of a tissue-engineered product is artificial skin used to treat patients with burns.
- **Combined ATMPs**
  Combined ATMPs contain one or more medical devices as an integral part of the medicine.

These treatments offer huge potential for patients, because the technologies behind advanced therapies may have positive long-term effects. They may offer an effective treatment for patients and subsequently decrease long-term healthcare management costs.

While they generate huge expectations, advanced therapies are also associated with:

- **New significant risks**, such as
  - Risk of virus infection if a viral vector reactivates
  - Risk of cancer
  - Risk of healthcare professionals and caregivers if accidentally exposed to the treatment
- **Ethical problems**, such as
  - Non-respect of the rights of living donors through

lack of information
- Administration of unproven/unsafe (stem)cell products to patients

Advanced therapies are often developed by small companies or research units in hospitals. The legislation in the EU provides incentives to encourage research and development of advanced therapies by providing fee reductions for scientific advice from the EMA. New regulatory procedures are available to ATMP developers (ATMP classification and ATMP certification).

Regulatory concepts and applicable legislation

Since 2008, all ATMPs are required to use the Centralised Procedure (CP). This ensures that they benefit from the single evaluation and authorisation procedure available in the EU. This makes it easier for companies to market their products and for patients in different Member States to gain access to these products.

ATMPs are complex medicines requiring assessment criteria that go beyond those used in the traditional pharmaceutical field. As an example, for products containing or consisting of genetically modified organisms (GMOs), an environmental risk assessment is required to evaluate the risks to human health and the environment.

The legislation requires that the expertise within the CAT covers all the areas relevant to advanced therapies, including:

- medical devices,
- tissue engineering,
- gene therapy,
- cell therapy,
- biotechnology,

- surgery,
- pharmacovigilance,
- risk-management, and
- ethics.

The CAT prepares a draft opinion on each product. This opinion is sent to the CHMP. Based on the CAT opinion, the CHMP adopts a recommendation on the granting/refusal, variation, suspension, or revocation of a marketing authorisation. The recommendation is then sent to the European Commission for a decision on the marketing authorisation.

Once the products are authorised and marketed, the EMA carries out further assessment of their safety and effectiveness. The EMA also gives scientific support to companies to help them design systems to monitor the safety of these medicines.

Companies that make ATMPs must be able to trace all their products from where they are manufactured to the hospitals or establishments where they are delivered to patients. Hospitals are also required to be able to trace all patients that receive them. The systems in place should allow full traceability, through anonymous coding systems in the case of external donors:

- At the tissue establishment: link between donor and donation.
- At the manufacturing site: link between donation and product.
- At the hospital or practice office: link between product and the patient.

[glossary_exclude]Further Resources

- European Medicines Agency (2015). *Committee for Advanced Therapies (CAT)*. Retrieved 3 September, 2015 from https://www.ema.europa.eu/en/committees/committee-advanced-therapies-cat

- Bhalerao, N., Bhol, R., Paranjpe, G., Jadhav, S., & Bodkhe, P.(2012) *Tissue engineering*. Retrieved 4 September, 2015, from http://fr.slideshare.net/BhaleraoSudhir/tissue-engineering-12323232
- World Health Organisation (2015). *Health policy and the ethical, legal, and social issues (ELSI) in genomics*. Retrieved 4 September, 2015 from http://www.who.int/genomics/policy/ELSI/en/[/glossary_exclude]

[glossary_exclude]Attachments

- Fact Sheet: Summary of regulatory concepts and legislation, and the role of patient organisations

  Size: 106,232 bytes, Format: .docx

  This factsheet provides an overview of the different regulatory concepts and legislation associated with special medicinal products, and information of the role of patient organisations in these regulatory processes.

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