European Medicines Agency

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Questions and answers on the regulation of advanced therapy medicinal products

On 30 December 2008, new legislation on advanced therapy medicinal products came into force in the European Union[1]. The legislation defines what products are to be considered advanced therapy medicinal products and lays down the rules on how they are to be authorised, supervised and monitored to ensure that they are safe and effective. The legislation also establishes within the European Medicines Agency a new committee dedicated to advanced therapies. The Committee for Advanced Therapies (CAT) plays a central role in the scientific assessment of advanced therapy products.

What are advanced therapy medicinal products?

Advanced therapy medicinal products differ from conventional medicines in that they are based on genes and cells. There are three types of advanced therapy products defined in the legislation: gene therapy products, somatic cell therapy products and tissue engineered products.

- Gene therapy products are used to treat genetic diseases. Many of these products work by inserting genes (DNA) into cells. When the new gene is integrated into a cell's chromosomes, the cell produces or stops the production of a protein which may help slow down or cure a disease.
- Somatic cell therapy products contain cells or tissues that have been manipulated to change their biological characteristics. They can be used to cure, diagnose or prevent a disease. An example of somatic cell therapy is the use of a patient's manipulated cancer cells to fight remaining cancer cells in their body.
- Tissue engineered products contain cells or tissues that have been 'engineered' (modified) so they can be used to repair, regenerate or replace tissue. An example of a tissue engineered product is artificial skin which is used to treat patients with burns.

Why has the new legislation for advance therapies been made?

Advanced therapy products offer groundbreaking new treatment opportunities for many diseases and injuries to the human body. However, evaluating them often requires specific expertise that goes beyond what is needed for conventional medicines. The new legislation will ensure that the expertise is available at the Agency to assess these products.
At the moment, advanced therapies are often developed by small companies or research units in hospitals. The legislation provides incentives to help them continue their research on, and development of, advanced therapies.

How are advanced therapy products regulated?

All advanced therapy products come under the centralised procedure, thus ensuring that they benefit from the single evaluation and authorisation procedure available in the European Union. This makes it easier for companies to market their products and for patients in the different Member States to gain access to these products.
The CAT prepares a draft opinion on the quality, safety and efficacy of each product. This opinion is then sent to the Committee for Medicinal Products for Human Use (CHMP), the committee responsible for human medicines at the Agency. Based on the CAT opinion, the CHMP adopts a

[1] Regulation (EC) No 1394/2007 of the European Parliament and of the Council of 13 November 2007 on advanced therapy medicinal products and amending Directive 2001/83/EC and Regulation (EC) No 726/2004.

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recommendation on the granting, variation, suspension or revocation of a marketing authorisation. The recommendation is then sent to the European Commission for a decision.
Once the products are authorised and marketed in the European Union, the Agency carries out further assessment of their safety and effectiveness.

What special requirements are in the legislation on the safety of advanced therapy products?
Companies that make advanced therapy products are required to be able to trace all their products from where they are manufactured to the hospitals where they are delivered to patients. Hospitals are also required to be able trace all the patients that receive them.

How does the new legislation encourage research in advanced therapy products?
The legislation provides for scientific and financial incentives to encourage research and development in the area of advanced therapies. Companies developing advanced therapy medicinal products can obtain reductions in the fees payable to the Agency of:

- 65% for a request for scientific advice (90% for small and medium-sized companies);
- 50% for an application for a marketing authorisation, in cases where the applicant is a hospital or small/medium-sized company and can prove that its product is of a particular public-health interest.

The Agency also gives scientific support to companies to help them design pharmacovigilance and risk-management systems (systems used to monitor the safety of medicinal products).

What is the role of the CAT?
The CAT's main role is to prepare a draft opinion on each application concerning advanced therapy products submitted to the Agency. However, it has many other responsibilities, such as:

- providing recommendations on the classification of advanced therapy products;
- reviewing data on manufacture and testing in animals of products developed by small companies;
- providing scientific advice;
- providing, at the request of the European Commission, scientific expertise and advice for any initiatives related to the development of innovative medicines and therapies.

What is the composition of the CAT?
The CAT brings together scientific expertise in advanced therapies from across the European Union. The Committee is composed of:

- five members of the CHMP appointed by the CHMP;
- one member appointed by each Member State, unless the Member State already has a representative among the members appointed by the CHMP;
- two members appointed by the European Commission to represent clinicians;
- two members appointed by the European Commission to represent patients' associations.

The Committee also has alternate members who are nominated to attend meetings on behalf of members who are absent. All members of the Committee are nominated on the basis of their expertise in fields relevant to the assessment of advanced therapy products. The legislation requires that the expertise within the Committee covers all the areas relevant to advanced therapies, including medical devices, tissue engineering, gene therapy, cell therapy, biotechnology, surgery, pharmacovigilance, risk-management and ethics. At least two members and two alternates should have expertise in medical devices. At CAT meetings, members can be accompanied by experts.