Advanced therapy medicinal products

2005/0227(COD) - 16/11/2005 - Legislative proposal

PURPOSE: to propose new rules concerning advanced therapy medicinal products.

PROPOSED ACT: European Parliament and Council Regulation

CONTENT: the overall policy objective is to improve patients? safe access to advanced therapies by increasing the research, development and authorisation of gene therapy, somatic cell therapy, and tissue engineered products.

More specifically, the main objectives are:

? to guarantee a high level of health protection for European patients treated with advanced therapy products;

? to harmonise market access and to improve the functioning of the internal market by establishing a tailored and comprehensive regulatory framework for the authorisation, supervision and post-authorisation vigilance of advanced therapy products;

? to foster the competitiveness of European undertakings operating in this field;

? to provide overall legal certainty, while allowing for sufficient flexibility at technical level, in order to keep the pace with the evolution of science and technology.

The proposal covers all advanced therapy products (gene therapy medicinal products, somatic cell therapy medicinal products, and tissue engineered products) falling within the global scope of the pharmaceutical legislation (Article 2(1) of Directive 2001/83/EC), i.e. intended to be placed on the market in Member States and either prepared industrially or manufactured by a method involving an industrial process.

The main features of the proposal are as follows:

- a centralised marketing authorisation procedure, to benefit from the pooling of expertise at European level and direct access to the EU market;

- a new and multidisciplinary expert Committee (Committee for Advanced Therapies), within the European Medicines Agency (EMEA), to assess advanced therapy products and follow scientific developments in the field;

- tailor-made technical requirements, which are adapted to the particular characteristics of these products;

- strengthened requirements for risk management and traceability;

- a system of low-cost, top-quality scientific advice provided by EMEA;

- special incentives for small and medium-sized enterprises.

- ethical aspects. The proposed Regulation respects fundamental human rights and observes the principles reflected in the Charter of Fundamental Rights of the European Union1. It also takes into account, as appropriate, the Convention for the protection of human rights and dignity of the human being with regard to the application of biology and medicine: Convention on human rights and biomedicine (?Oviedo? Convention). The proposed Regulation does not interfere with national legislation prohibiting or restricting the use of any specific type of human or animal cells, or the sale, supply or use of medicinal products based on such cells. Explicit provisions have been introduced in the proposal to clarify this point.

In addition, the proposal foresees additional, specific incentives:

? a 90% fee reduction for the provision of scientific advice by the EMEA in respect of advanced therapies, regardless of the economic size of the applicant;

? a system of early evaluation and certification of quality and non-clinical safety data by the Agency, independently of any marketing authorisation application, for SMEs developing advanced therapy medicinal products. This system is designed to help SMEs which focus on the early development aspects, but do not conduct the subsequent clinical trials themselves. The certification of ?early-development? data by the Agency should provide an important selling argument to those companies who wish to license out their technology to bigger undertakings.

The Commission is of the opinion that human tissue- and cell- based products should be founded on the philosophy of voluntary and unpaid donation, anonymity of both donor and recipient, altruism of the donor and solidarity between donor and recipient. Voluntary and unpaid tissue and cell donations are a factor which may contribute to high safety standards for tissues and cells, and hence to the protection of human health.

All interested parties (patients associations, industry, hospitals, research community?) have been widely consulted on this proposal, through various means: internet-based consultation, workshops, bilateral meetings, interviews. The proposed Regulation has been the subject of a Commission Impact Assessment, which is attached to the proposal.

For more details concerning the financial implications of this proposal, please refer to the financial statement.