



Medicines in Europe Forum position statement on the

Draft regulation of the European Parliament and Council on advanced therapy medicinal products and amending Directive 2001/83/EC and Regulation (EC) 726/2004

The Medicines in Europe Forum, which represents patients' and other stakeholders' interests, welcomes the plan to create a single regulatory framework for gene therapy, cell therapy and products derived from tissue engineering. The legislative and regulatory texts are currently dispersed, and the lack of a clear legislative framework in some areas may endanger patients.

A welcome medicinal product status. Being "*presented as having properties for treating or preventing disease in human beings; and used in or administered to human beings with a view to restoring, correcting or modifying physiological functions*", products derived from tissue engineering should be defined as medicinal products and be subject to the same regulatory requirements. The draft Regulation is clear on this point, and rightly stipulates that none of these "advanced therapies" should be considered as "medical devices", which are subject to less demanding regulations.

A welcome centralised European marketing authorisation. It is logical to pool European expertise when deciding whether to authorise such drugs. The use of a Regulation rather than a Directive is reasonable, as it will permit rapid application and avoid uncontrolled development of certain therapies.

The Medicines in Europe Forum nevertheless considers that 5 aspects of the draft text are unsatisfactory as they stand. If the draft Regulation is to offer patients the necessary guarantees, the following points must be revisited:

-Incomplete transposition of the Directives on which the draft Regulation is based: several recent Directives concern gene therapy, cell therapy, and tissue engineering, but not all have been effectively transposed by all Member States. In particular:

-Directive 2004/27/EC on medicines for human use (transposition deadline: 30 October 2005);

-Directives 2004/23/EC and 2006/17/EC on the donation, collection, control, transformation, preservation, storage and distribution of human tissues and cells (respective transposition deadlines: 7 April 2006 and 1 November 2006);

-Directive 2005/28/EC concerning the application of good clinical practices to experimental medicines (transposition deadline: 29 January 2006).

The Medicines in Europe Forum considers that patient safety cannot be guaranteed unless these Directives are applied. Member States must be made to respect transposition deadlines.

-Risks of excessive flexibility: according to the European Commission and manufacturers, the techniques used to produce these “advanced therapies” are so special that regulatory requirements must be adaptable at short notice. The Commission insists on the “three-thirds” strategy which served as the foundation for the draft Regulation: one-third legislative framework, one-third comitology, and one-third guidelines.

Thus, article 4 of the draft text states that the European Commission will be able to adapt good practice guidelines governing clinical trials. Likewise, Article 8 states that the Commission will be able to adapt technical requirements for drugs derived from tissue engineering. And Article 24 states that the Commission will be able to adapt the four annexes of the draft Regulation “*to scientific and technical evolution*”.

While acknowledging the need to adapt regulatory texts to scientific advances, without systematic approval from Parliament, the Medicines in Europe Forum wishes to stress that patients’ interests must always be taken into account. The Forum therefore considers that the Regulation should explicitly oblige the Commission to give the reasons for any such adjustments and also to explain the decision-making process (with mandatory consultation with scientific committees).

-Risk of patient non representation: article 21 of the draft Regulation describes the composition of the Committee on advanced therapies, as part of the European Medicines Agency. The plan is to include 2 patient representatives, but the draft text states that “*All members of the Committee for Advanced Therapies shall be chosen for their scientific qualification or experience in respect of advanced therapy medicinal products*”.

The Medicines in Europe Forum is concerned that this will exclude most potential patient representatives.

-Risk of inadequate patient information: the annexes of the Regulation dealing with the summary of product characteristics, labelling, and patient leaflet, as currently worded, are very similar to those for ‘classical’ medicines. It is likely that they will be inappropriate for some advanced therapies. The draft Regulation also states that it is the role of the Commission to adapt the texts. If patients are not included in the process, there is a risk that they will not have access to some important information, even though it is they who are most directly concerned.

The Medicines in Europe Forum considers that information for patients and caregivers must be sufficient to make an informed choice, and that patients must be involved in establishing the relevant demands.

-Incentives for drug companies, but what payback?

Firms marketing advanced therapies will be entitled to multiple incentives such as data protection (8+2+1), orphan drug status, an accelerated assessment procedure, conditional marketing authorisation, specific financial incentives (e.g. a 90% reduction in Agency fees for scientific advice), administrative assistance, etc.

The Medicines in Europe Forum considers that research on advanced therapies should be encouraged, but that a system must be created to monitor the fruits of any publicly funded incentives, not only for the pharmaceutical industry, but also in terms of therapeutic advance for patients.