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PUBLIC CONSULTATION PAPER ON THE REGULATION ON ADVANCED THERAPY MEDICINAL PRODUCTS

TOPICS

2.1 Marketing authorization application requirements for advanced therapy medicinal products

The registration framework underlying the development and registration of gene therapy, somatic cell therapy and tissue engineering medicinal products has several known limitations, related to the already complex documentation that is required for ”conventional non-advaced therapy” products, with the addition of chronic delays in the trial authorization procedure for the clinical testing of advanced products.
It is noted that new models of preclinical and clinical research, as well as the criteria for the definition of specific quality parameters for gene therapy, somatic cell therapy and tissue engineering, are still under discussion and have to be standardized, both at national and Community level.

It would therefore be appropriate:

- to identify a single reference point of contact in the Agency during the whole development process in order to ensure a more efficient communication and interaction between industry and institutions;
- to define some paths that would lead on the one hand to a greater clarity on the adequacy of the documentation to support the request for marketing authorization, and on the other to a simplification of the procedure by reducing development time and costs.

2.2 Requirements for combined advanced therapy medicinal products

Article 9, paragraph 3 of the Regulation (EC) 1394/2007 may be agreed upon only if it ensures sufficient flexibility in the evaluation of the combined advanced therapy medicinal products in case the results of the assessment of the medical device part or of the active implantable medical device by a notified body are not available.
Also in this case, identification of a single reference point of contact for the whole marketing authorization procedure would be appreciated.

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2.3 Hospital exemption

To preserve the principle that drugs must be used only if the safety and efficacy has demonstrated, the hospital exemption should be considered only when no therapeutic alternative is available and when a medicine approved according to Regulation (EC) 1394/2007 for the same indication is lacking.

Moreover, in order to avoid the risk that different rules be applied to industry and to public and private hospitals, competent authorities must define clear and objective criteria and processes to ensure quality and safety standards for the protection of patients.
For this reason, a clarification is sought leading to a homogeneous interpretation and application of Article 28 of the Regulation on Advanced Therapies is given at Member States level .

2.4 Incentives for the development of advanced therapy medicinal products

It is important to extend the application of the incentives already provided for by Regulation (EC) 1394/2007 beyond the transitional period, considering the typical features of advanced therapy medicinal products.
The inclusion of incentives for clinical trials in phase I and II for advanced therapy medicinal products within Community programs (eg the EU Framework Program for Research and Innovation Horizon 2020) would be appreciated.

2.5 Scope and adaption to technical progress

Consideration should be given to how divergent interpretation by Member States of cell therapy for non-homologous use may lead to inclusion or exclusion of a cell product in the Regulation on Advanced Therapy and affect the product development (eg the use of autologous stem cells extracted from adipose tissue to cure diabetes ulcers or for the post-mastectomy reconstruction).