EUROPEAN COMMISSION
HEALTH AND CONSUMERS DIRECTORATE-GENERAL

Health Systems and Products
Medicinal products – authorisations, EMA

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PUBLIC CONSULTATION PAPER

ON THE REGULATION ON ADVANCED THERAPY MEDICINAL PRODUCTS

Deadline for Public Consultation: 31 March 2013

This document does not represent an official position of the European Commission. It is a tool to explore the views of interested parties.

This document is to be read together with Regulation 1394/2007 of the European Parliament and of the Council on advanced therapy medicinal products and amending Directive 2001/83/EC

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1. ABOUT THE CONSULTATION

1.1. What is the purpose of this consultation?

Regulation 1394/2007 of the European Parliament and of the Council on advanced therapy medicinal products and amending Directive 2001/83/EC (hereafter "Advanced Therapy Regulation") requires the Commission to assess the application of the Advanced Therapy Regulation and to produce a report.

With this public consultation, Directorate General for Health and Consumers intends to seek the view of stakeholders regarding the application of the Advanced Therapy Regulation.

1.2. Who is consulted?

All stakeholders dealing with advanced therapy medicinal products. Comments from Small and Medium-sized Enterprises (SMEs) are particularly welcomed.

1.3. How can I contribute?

Contributions should be sent **before 31 March 2013** by e-mail exclusively to:

SANCO-ADVANCEDTHERAPY-REPORT@ec.europa.eu

Contributions will be made publicly available on the 'Pharmaceuticals' website of the Commission once the consultation period is over, unless a specific request for confidentiality is made, in which case only an indication of the contributor will be disclosed. If you do not wish your contribution to be made public, please clearly indicate so.

1.4. What will happen next?

All contributions will be taken into account in the preparation of the Commission report on the application of the Advanced Therapy Regulation.

2. CONSULTATION TOPICS

2.1. Marketing authorisation application requirements for advanced therapy medicinal products.

The Advanced Therapy Regulation provided for adapted requirements in terms of the dossier that applicants must prepare to demonstrate the quality, efficacy and safety of the medicinal products when applying for a marketing authorisation.

The amount of data that must be generated for the submission of a marketing authorisation application is critical to ensure a high level of public health protection. Proportionality of the requirements is also important to facilitate the marketing of advanced therapies.

Please provide your comments on the requirements for marketing authorisation applications set out in the Regulation.

2.2. Requirements for combined advanced therapy medicinal products.

The existence of advanced therapy medicinal products that incorporate one or more medical devices has been recognised and regulated in the Advanced Therapy Regulation. In particular, combined advanced therapy medicinal products are to be authorised by the Commission following the scientific assessment of the European Medicines Agency. The applicant must demonstrate that the essential requirements of the specific legislation on medical devices have been complied with and there is a possibility for the Agency to consult the relevant notified bodies.

No application for a combined advanced therapy medicinal product has been submitted to the European Medicines Agency yet.

Please provide your views on the authorisation procedure foreseen in the Advanced Therapy Regulation for combined advanced therapy medicinal products.

2.3. Hospital exemption.

The Advanced Therapy Regulation empowers Member States to authorise the use of advanced therapy medicinal products in hospitals for individual patients in the absence of a marketing authorisation. The so-called hospital exemption provides for flexibility to address the situation of individual patients; however, a too large application of this exemption may discourage the application for marketing authorisations.

Please provide your views on the application of the hospital exemption.

2.4. Incentives for the development of advanced therapy medicinal products.

Advanced therapies are at the cutting edge of innovation. The full development of the potential of this sector is closely linked to the evolution of scientific knowledge. The Advanced Therapy Regulation provides for a number of incentives to support the development of these products, such as certification for quality and non-clinical data, reduced fees, scientific advice.

Please provide your views on the incentives provided for under the Advanced Therapy Regulation.

2.5. Scope and adaptation to technical progress.

The Advanced Therapy Regulation applies to gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products.

Please provide your views on the scope of the Regulation and in particular as to whether the scope should be modified to take account of technical progress.