EURORDIS
European Organisation for Rare Diseases

CONTRIBUTION TO THE CONSULTATION PAPER AND DRAFT REGULATION ON ADVANCED THERAPIES

EURORDIS - the European Organisation for Rare Diseases - represents 225 rare disease organisations from 24 countries, 16 of which are EU member states, and thereby reflects the voice of an estimated 30 million patients affected by rare diseases in the European Union.

In response to the Commission's invitation to contribute to its proposal for a Community regulatory framework on advanced therapies, EURORDIS is pleased to send its observations on the consultation paper.

EURORDIS and its members welcome the Commission's proposal as an appreciated recognition of the status of advanced therapies, including gene therapy, cell therapy and human tissue engineering. As organisations representing patients affected by rare diseases, 80% of which are of genetic origin, we have always placed considerable hope on the advancement of genomic research and on advanced therapies. Few well-identified patient groups in the EU are at the forefront of innovation in this field.

EURORDIS supports most of the proposed provisions of the future regulatory framework for advanced therapies. In particular, we support and comment the following elements:

- We welcome the compulsory centralised Community marketing authorisation for all advanced therapy products to be dealt with at EMEA level;

- We welcome the creation, within the EMEA, of the Committee for Advanced Therapies (CAT), pooling very specific expertise. A "clearly-defined procedure" is also appreciated, including strict deadlines and economic incentives (such as fee reductions for EMEA procedures) in order to avoid any delays in the marketing authorisation of these products.

- The proposal of including patients' representatives in the CAT, is also very much welcomed. We deplore the absence of patients' involvement at the earliest stage of the drafting of this paper.

- The distinction between "industrial" advanced therapy product and "individual" advanced therapy product, which recognises the peculiarity of single patients, is particularly important for people living with a rare disease. We nevertheless fear that it will often be difficult to distinguish between the exceptions for products produced on an ad-hoc one-off basis and "any advanced therapy product for autologous use which, although being patient specific by

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definition, is manufactured in accordance with a standardised, industrial process".

- We agree on the need for "sufficient flexibility" in establishing technical requirements for advanced therapy products on which there is not yet a longstanding experience and which evolve particularly rapidly, especially compared to classical pharmaceutical products.

- We very much welcome the possibility for advanced therapy products to be designated as orphan products and hence benefit from a 10-year market exclusivity period.

- We entirely support the principles underlying the donation of human tissue and cell but we would prefer a stronger wording than the one of Directive 2004/23/Ec on the nature of this donation, which "has to be" - rather than "should be" as in the Directive - voluntary and unpaid.

Nevertheless, we have some reservations and would like to express our concern mainly on the following two elements, both related to the proposed Risk Management System:

- We totally agree that appropriate and long-term patient follow-up and post-authorisation monitoring are crucial aspects of these products, however we do not agree that the sponsor should be in charge of these aspects. We do agree that the data collected during any clinical study performed after the marketing authorisation have to be reported systematically by the sponsor, but outside of any official study we would prefer a Centralised and Public Risk Management System to perform these tasks.

  Why should the applicant be given the role of managing a "system allowing complete traceability of the patient"? We consider this as being interference from a manufacturer in the private life of patients. We believe this system should be financed through public funds and managed at European level on the basis of reports from doctors and patients to be sent directly to the European institution responsible for pharmacovigilance. This would enhance public support and confidence towards advanced therapies.

- We also fear that the proposed system could generate too many additional regulatory constraints, thereby discouraging the necessary research in the field of advanced therapies. Innovation in this field is particularly needed for treating rare diseases, but also with a view to enhancing European competitiveness in the area of advanced therapies.

Last but not least, we would like to stress the need for appropriate and sufficient funding - within the 7th Framework Programme of Research & Development of the EU - to be dedicated to research in the field of advanced therapies.

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