Responses from the ATMP Manufacturing Community (amc) to the Public Consultation Paper on the Regulation on Advanced Therapy Medicinal Products (Ref. Ares(2012)1540330 - 21/12/2012)

The amc is an association of UK Stakeholders manufacturing ATMPs or sponsoring clinical trials of advanced therapy IMPs. Its over 200 members include a mixture of industry and health professionals, approximately 20% of which come from SMEs. Further details are available on the amc website.
http://www.atmpmanufacture.org/Home/Home.php

This response document was compiled by amc member Dr Drew Hope, Head of Advanced Therapy Quality at Guy's and St Thomas' NHS Foundation Trust, London, UK.

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 views on whether the requirements for marketing authorisation applications set out in the Regulation are proportionate and adequate to ensure a high level of public health.

Response
The amc believes that the marketing authorisation requirements are adequate and proportionate, but also encourages the Commission to pursue the development of, and encourage the use of, alternative strategies such as conditional marketing authorisations and adoptive licensing.

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 whether the procedure foreseen in the Advanced Therapy Regulation to assess compliance with the essential requirements of the medical device legislation is adequate.

Response
No comments

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 medicinal products prepared on a non-routine basis and used in a hospital under the exclusive professional responsibility of a doctor for 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.

Response
The provision of hospital exemptions for authorisation in Article 28 of the Regulation relate to non-routine preparations of ATMPs. In the UK, the MHRA provides licences for the manufacture of Specials for patients where their needs cannot be met by licensed medicinal products. The amc considers that ATMPs that are available from holders of marketing authorisation should be considered as routine preparations. In such circumstances, hospital exemptions should no longer be available, providing further incentive for application for marketing authorisations.
The Commission should provide guidance on the definition of 'non-routine'. For example, when hospital exemptions have been used beyond this definition, then manufacturers and medical practitioners should be obliged to proceed with clinical trials in order to compile data for the submission of their own marketing authorisation application. Such guidance will promote a harmonised approach by Member State Competent Authorities with application of the hospital exemption.

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.

Response
No comments

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.

Response
No comments