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 authorization 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.

Eucomed observes that 6 years after the entry into force of the ATMP regulation only one tissue engineered product has been granted approval and that no combination product has been approved either. Eucomed has ground to believe that, in the case of tissue engineered products, the proportionality of requirements has not been implemented by the Agency. There is a lack of flexibility in applying the rules normally applicable to medicinal products to products which are largely different in nature, mode of action and which are basically always custom made. Past history of products already on the market has not been taken into account. A deep reconsideration of whether these products should remain under the ATMP or not is necessary. As an alternative a set of rules specific to these products should be developed independently from the existing ones for medicinal products and a specific Committee, independent from the CHMP and constituted by experts in the specific sector, should be charged of the evaluation of tissue engineered products.

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.

The comment to point 2.1 above applies as well to this question

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.

We agree on the analysis; however this exemption remains, for the reasons mentioned above, the only way to continue treating patients with safe, effective and well known products which have not gone through the hurdles of an inappropriate legislation.

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.

We would recommend to separate tissue engineered products from the scope of the Regulation in order to allow a specifically tailored regulatory regime to these products which cannot be assimilated to medicinal products