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We thank the European Medicines Agency (EMA) for the opportunity to answer the consultation on implementation of the European regulation 1394/2007 for advanced-therapy medicinal products.

2.1 Marketing authorisation requirements for advanced therapy medicinal products

The requirements set up in the European Regulation for ATMP were slightly adapted from the regulatory framework of Medicinal Products. However, before this regulation was implemented, most of ATMPs were developed under the tissue and cells requirements. There is a large gap between both type of requirements and it is highly challenging to manage to obtain the requested data without putting at risk the success of the ATMP development. For costs reasons, this challenge is practically impossible to achieve when the developer is a SME or non-profit organisation.

When new ATMP developments are started under the European regulation, some inflexible requirements can also quickly jeopardize the progress of the project. For example, extensive implementation of GMP to low number of production units manufactured from human tissue is very difficult to achieve. This may end up in prohibitive costs for poor value quality data. Although it cannot be denied that "the amount of data that must be generated for the submission of a marketing authorisation application is critical", the technical as well as the financial challenges in developing models for assessment of specific quality features of an ATMP as per European regulation may prevent further research. Better integration of the medical device and tissue approach in the regulation would improve the adequacy between regulatory framework and ATMP characteristics in order to ensure patient safety at a reasonable price. The recent examples of absence of reimbursement of pioneered authorised ATMP in most of European countries do not foster confidence in the future of ATMP in Europe, unless changes are considered.

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2.2 Requirements for combined advanced therapy medicinal products

Some regulatory authorities seem to consider combined ATMP with utmost caution and are reluctant to evaluate separately the matrix of an ATMP as a medical device. The reason given for this is that the matrix is not used alone and therefore cannot be evaluated as such. It seems that a regulatory issues is raised here and that further discussion with national authorities is needed in order to clarify this approach.

2.3 Hospital exemptions

The application of hospital exemption has been left to the appreciation of the Member states. To our knowledge, this results in large discrepancies between states. Some of them have implemented a legal framework at the same level as the European regulation, while other countries do not have any particular requirements. Whilst the latter may compromise centralised MA of ATMP, the availability of the products to the population remains limited.

Moreover, the justification of this status remains questionable from a medical point of view, as a potential lowest quality of final products is implicit in this approach.

In order to overcome those drawbacks, a decentralised procedure could be an encouraging perspective susceptible to relaunch ATMP development. It could facilitate the marketing authorisation of ATMP by SME and non-profit organisation on the basis of national requirements while living open further distribution in other European countries and pursuit of partnership.

2.4 Incentives for the development of advanced therapy medicinal products

The incentives mentioned in the European regulations are well intentioned but insufficient to allow ATMP development in SME or non-profit organizations which are the only candidates to this niche markets, for the financial reasons mentioned above.

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