Advanced therapy medicinal products

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The Commission presents a report in accordance with Regulation (EC) No 1394/2007 of the European Parliament and of the Council on advanced therapy medicinal products (ATMP) and takes stock of the situation of ATMPs in the EU and analyses the impact of the Regulation on advanced therapies.

The cornerstone of the Regulation is that a marketing authorisation must be obtained prior to the marketing of ATMPs. The marketing authorisation can only be granted if, after a scientific assessment of the quality, efficacy and safety profile, it is demonstrated that the benefits outweigh the risks.

The Commission states that the contribution of the ATMP Regulation to public health could be measured against two parameters: (1) the extent to which new ATMPs have become available in the EU; and (2) the extent to which authorised ATMPs are efficacious and safe.

Availability of new ATMPs: the report stresses that the regulation of ATMPs has been an important step to protect patients from scientifically unsound treatments. In addition, the ATMP Regulation has created a common framework for the assessment of advanced therapies in the EU. However, it is still the early days of the development of advanced therapies and only four ATMPs have been granted a marketing authorisation .

The much higher activity of the Committee for Advanced Therapies in the area of scientific advice and classification, as well as the high number of clinical trials involving ATMPs, is a signal of a dynamic research sector.

The Commission feels that advanced therapies have the potential to bring major benefits to patients. However, there are still many unknowns and it is therefore important to put in place adequate controls to prevent detrimental consequences for public health.

Requirements for authorisation: the ATMP Regulation protects patients by requiring that an independent review of the ATMP is done by the best available experts in the EU according to high standards of quality, efficacy and safety before the product is made available to patients.

The report states, however, that too burdensome requirements could have detrimental consequences for public health as it could prevent the appearance of valid treatments for unmet medical needs.

Regulation in this area should contribute to creating conditions that facilitate the appearance of new medicinal products, while ensuring a high level of public health protection. It is also important that the regulatory framework is adapted to rapid scientific progress.

Conclusions and recommendations: experience accumulated since the entry into force of the ATMP Regulation shows that some options to help the translation of research into ATMPs available to patients across the EU while maintaining a high level of public health protection can be identified, including:

- clarification of the scope of the ATMP Regulation by fine-tuning the current definitions of ATMPs and by reflecting on the appropriate regulatory framework for new innovative products that many not be captured by existing provisions;
- considering measures to avoid disparities in the classification of ATMPs in the EU;
- clarification of the conditions for the application of the hospital exemption (which allows the use of custom-made ATMPs under controlled conditions in the absence of a marketing authorisation), as well as the role of data obtained therefrom in the context of marketing authorisation procedures. If the hospital exemption became the normal route to market advanced therapies, there would be detrimental consequences for public health ;
- revising the requirements for the authorisation of ATMPs with a view to ensuring that requirements applicable are proportionate and well-adapted to the specific characteristics thereof, having specific consideration to autologous products (in the latter case, the cells/tissues are harvested from a patient, then treated or expanded, and finally they are introduced back into the same patient;
- streamlining the marketing authorisation procedures (scientific evaluation involves up to five committees) which is perceived as too cumbersome, particularly for SMEs and non-for-profit organisations ;
- extending the certification procedure and clarification of the link between the certification and the marketing authorisation procedure ;
- creating a more favourable environment for ATMP developers working in an academic or non-for-profit setting, including by promoting early contacts with the authorities through the application of the fee reduction for scientific advice and by extending the certification scheme to these developers;
- considering possible fee incentives to reduce the financial impact of post-marketing obligations.