

Advanced Therapy Medicinal Products

LERU briefing paper
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Executive summary

Advanced Therapy Medicinal Products (ATMPs) are innovative and complex medicines which can potentially be used to treat a variety of human health issues, including cancers (such as leukemia and melanoma), neurodegenerative diseases (such as Huntington's and Parkinson's diseases), inherited diseases and autoimmune diseases (such as diabetes, multiple sclerosis and rheumatoid arthritis). They are particularly important for severe, rare, or chronic diseases where conventional approaches have proven to be inadequate. The number of patients treated with specific ATMPs, however, is still very low.

Universities already play an important role in ATMP development, but their role could be improved, made larger and more prominent, if specific hurdles were addressed. In this paper, we outline possible ways in which this could be done. These include:

- Improving interactions with, and help provided by, support organisations, such as EMA, industry and other universities, to cover better the whole product development pathway and to ensure support (including financial) at all stages of development.
- Universities should focus on new innovative products that address an unmet medical need and have a high chance of success and on products which are unlikely to be attractive to industry.
- Retaining the Hospital Exemption¹, but harmonising its application, improving its assessment and using it only in product development or for products not suitable for Marketing Authorisation. A registry should be developed to record information on ATMP use under the Hospital Exemption.
- Improving transparency, both in ATMP trials and in the use of the Hospital Exemption, which will help organisations active in this field to learn from others' successes and failures.

LERU hopes that the actions in this paper will lead to the development of new therapeutic options, improved links with the pharmaceutical industry and, most importantly, to better patient access to these innovative medicinal products.

¹ See section 4 for an explanation of the Hospital Exemption

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