The Advanced Therapy Medicinal Product Regulation
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Introduction
Advanced therapy medicinal products (ATMPs) are new innovative, regenerative biological medicinal products that are based on genes, cells and tissues. ATMPs herald revolutionary treatments for a number of diseases where no effective therapy currently exists. However, the lack of an EU-wide regulatory framework in the past led to divergent national approaches which may have impaired the growth of the emerging biotechnology area and led to a reduction in patients’ access to these novel therapies. As a result, the European Union adopted Regulation 1394/2007/EC in November 2007 in order to facilitate and formalise the market authorisation of these products. Subsequently, the regulation came into force on the 30th of December 2008 and now requires all applications for marketing authorisations of such products to be made centrally to the European Medicines Agency (‘the Agency’) in London.

ATMP Definition
The ATMP regulation makes specific provision for three categories of advanced therapy products based on genes (gene therapy), cells (cell therapy) and tissues (tissue engineering), as defined in Part IV of Annex I to Directive 2009/120/EC.

Scope of Regulation
In accordance with the ATMP regulation, the Committee for Advanced Therapies (CAT) was established. As a multi-disciplinary scientific expert committee, it represents all EU member states and EFTA countries, as well as patients and physicians groups and associations. The CAT is responsible for the primary evaluation of ATMPs for final approval by the Agency’s Committee for Medicinal Products for Human Use. Additionally, the CAT operates two new regulatory procedures for companies developing ATMPs namely the “classification procedure” and the “certification procedure”.

The classification procedure aims to determine whether a product based on genes, cells or tissues meets the scientific criteria which define it as an ATMP. The certification procedure is the scientific evaluation of available quality and non-clinical data for ATMPs under development by Small and Medium-sized Enterprises (SMEs). It will determine whether the data generated to date is acceptable in terms of future regulatory compliance and scientific robustness. Overall, it is hoped that this procedure will provide an incentive for SMEs to develop ATMPs.

Given the sheer novelty, complexity and technical specificity of ATMPs, new and unexplored risks to public health and to individual patients may arise beyond those that can be recognised at the time of marketing evaluation. To legislate for this, the ATMP regulation outlines measures to ensure post authorisation follow-up of efficacy and safety in the form of a risk management system. Additionally, a system allowing complete traceability (up to 30 years) of the patients as well as of the product and its starting materials is also required to monitor the safety of the ATMP.

Finally, some ATMPs are excluded from the scope of the legislation. These include local, hospital-produced products for use in individual named patients on a non-routine basis as well as for clinical trials of ATMPs which are regulated by the national authorities. However, national requirements in terms of manufactures authorisation, compliance with GMP, tissue establishment authorisation and the fulfilment of national pharmacovigilance and traceability requirements will still apply.

References