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Scientific and Regulatory Challenges of Advanced Therapy Medicinal Products

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Abstract : Background. Advanced therapy medicinal products (ATMPs) are innovative therapies that mainly target orphan diseases and high unmet medical needs. ATMP includes gene therapy medicinal products (GTMP), somatic cell therapy medicinal products (CTMP), and tissue-engineered therapies (TEP). Since legislation opened the way in 2007, 25 ATMPs have been approved in the EU, which is about the same amount as the U.S. Food and Drug Administration. However, not all of the ATMPs that have been approved have successfully reached the market and retained their approval. Objectives. We aim to understand all the factors limiting the market access to very promising therapies in a systemic approach, to be able to overcome these problems, in the future, with scientific, regulatory and commercial innovations. Further to recent reviews that focus either on specific countries, products, or dimensions, we will address all the challenges faced by ATMP development today. Methodology. We used mixed methods and a multi-level approach for data collection. First, we performed an updated academic literature review on ATMP development and their scientific and market access challenges (papers published between 2018 and April 2023). Second, we analyzed industry feedback from cell and gene therapy webinars and white papers published by providers and pharmaceutical industries. Finally, we established a comparative analysis of the regulatory guidelines published by EMA and the FDA for ATMP approval. Results: The main challenges in bringing these therapies to market are the high development costs. Developing ATMPs is expensive due to the need for specialized manufacturing processes. Furthermore, the regulatory pathways for ATMPs are often complex and can vary between countries, making it challenging to obtain approval and ensure compliance with different regulations. As a result of the high costs associated with ATMPs, challenges in obtaining reimbursement from healthcare payers lead to limited patient access to these treatments. ATMPs are often developed for orphan diseases, which means that the patient population is limited for clinical trials which can make it challenging to demonstrate their safety and efficacy. In addition, the complex manufacturing processes required for ATMPs can make it challenging to scale up production to meet demand, which can limit their availability and increase costs. Finally, ATMPs face safety and efficacy challenges: dangerous adverse events of these therapies like toxicity related to the use of viral vectors or cell therapy, starting material and donor-related aspects. Conclusion. As a result of our mixed method analysis, we found that ATMPs face a number of challenges in their development, regulatory approval, and commercialization and that addressing these challenges requires collaboration between industry, regulators, healthcare providers, and patient groups. This first analysis will help us to address, for each challenge, proper and innovative solution(s) in order to increase the number of ATMPs approved and reach the patients

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