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2023, A Landmark Year in Biomedical Research; A Turning Point in Medical History

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Abstract

The rapid development of knowledge on healthy nutrition, and hygiene practices, as well as the advent of antibiotics and vaccines, has led to increased life expectancy in the recent century. The extended lifespan has brought new challenges for healthcare professionals, including the management of chronic degenerative diseases, malignancies, and autoimmune disorders. Advanced therapeutic medicinal products (ATMPs) have emerged as a promising frontier alongside conventional therapeutic modalities, offering innovative solutions through cell-based therapies, gene therapy, and tissue engineering. Recent years have witnessed remarkable advancements in regenerative medicine and the launching of innovative ATMPs. Numerous ATMPs have been registered and approved by regulatory agencies for the management of different diseases in 2023. The approval of groundbreaking therapies around the world has made 2023 an exceptional year. Novel ATMPs and the development of artificial intelligence (AI) in 2023 will pave the way for the integration of ATMPs and advanced technologies in personalized medicine, early diagnosis and targeted treatments.

Keywords: Cell-Based Therapies, Gene Therapy, Tissue Engineering

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Previously, health practitioners encountered significant challenges including infectious diseases, malnutrition, and poor hygiene while the development of science and widespread knowledge on healthy nutrition and hygiene, as well as the emergence of antibiotics and vaccines, have somewhat addressed these issues and led to an increase in life expectancy. However, the challenges facing healthcare professionals have changed with the increasing life expectancy, such as chronic diseases, malignancies, and autoimmune diseases (1, 2).

Encountering these clinical issues, advanced therapeutic medicinal products (ATMPs) emerged as a promising frontier alongside the conventional therapeutic methods. ATMPs encompass a diverse array of innovative approaches, including cell and cell-derivative-based therapies, gene therapy, and tissue engineering (3, 4).

In recent years, the field of ATMPs has seen significant advancements, with numerous innovative therapies being developed and tested. The year 2023 is known as a landmark year for ATMPs (5), with many groundbreaking therapies approved by the Food and Drug Administration (FDA), offering new hope to patients with complicated conditions. The approval of these therapies in 2023 is a testament to the rapid progress and potential of ATMPs in revolutionizing current medicine. Among numerous ATMPs for different disorders, 34 products have been approved by the FDA by the end of 2023. Seven ATMPs including CASGEVY™ [treatment of sickle cell disease (SCD)], ELEVIDYS™ [treatment of Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene], LANTIDRA™ (donislecel) (treatment of type 1 diabetes), LYFGENIA™ (treatment of SCD), OMISIRGE® (in patients with hematologic malignancies who are planned for umbilical cord blood transplantation following myeloablative conditioning to reduce the time to neutrophil recovery and the incidence of infection), ROCTAVIAN™ (treatment of severe hemophilia A), VYJUVEK® (treatment of wounds in patients with dystrophic epidermolysis bullosa) were approved by FDA in 2023 (6). Among the mentioned products, CASGEVY and LYFGENIA™ are a pair of gene therapies for SCD which work through gene-editing and gene-addition approaches, respectively. CASGEVY™ and LYFGENIA™ are the first cell-based gene therapy products against SCD. Both therapeutic methods use autologous blood stem cells in 12 years and older patients with SCD and recurrent or history of vaso occlusive crises (VOCs) (7). CASGEVY™ was approved

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by the FDA in December 2023 and is known as the first gene editing therapeutic method in humans. This product which is based on CRISPER technology can be directed to cut DNA in targeted areas, enabling the ability to accurately remove, add or replace DNA where it was cut (8-10). Lyfgenia™, the other approved ATMP for SCD, is a revolutionary gene therapy that alters the patient's blood stem cells to generate a gene therapy-based corrected hemoglobin that mimics the function of a specific type of healthy adult hemoglobin unaffected by SCD.

The market authorization for ReColorCell®, the first approved ATMP in Iran, was approved in 2023 by the Iranian FDA (IR-FDA). ReColorCell® is a cell-based therapeutic product containing autologous melanocyte-keratinocyte for patients with vitiligo. This product is available in the form of a cellular suspension and is injected into the epidermal in spots resulting from vitiligo (11-13).

The development of ATMPs has the potential to provide targeted and personalized treatments that can address the underlying pathologies of diseases rather than just managing symptoms. This represents a significant shift towards more effective and sustainable healthcare solutions. In the future, ATMPs are expected to play a key role in the development of precision medicine, where treatments are tailored to individuals based on their genetic makeup and specific health needs. This personalized approach has the potential to improve patient outcomes alongside minimized side effects, reduce healthcare costs, and ultimately lead to a more efficient and effective healthcare system. Overall, the development and integration of ATMPs in medicine hold great promise for the future of healthcare and offer new perspectives on how we can approach and treat diseases. Furthermore, a combination of ATMPs and artificial intelligence (AI) algorithms holds great promise for early detection and treating a wide range of diseases by targeting specific molecular pathways. By utilizing AI and machine learning techniques, researchers can analyze vast amounts of data to identify potential drug targets, optimize vaccine design, and predict patient responses to treatment (14). The integration of AI in molecular targeting and vaccine development has the potential to revolutionize personalized medicine and enhance the efficiency of therapeutic interventions.

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Authors' Contributions

M.V.; Conceived the design of the study. B.S., M.V.; Reviewed literature and drafted the manuscript. All authors read and approved the final manuscript.

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