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US Gene Therapy Market - 2025-2033

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Abstracts

Overview

The US gene therapy market reached US$ 2,812.92 million in 2024 and is expected to reach US$ 21,701.51 million by 2033, growing at a CAGR of 23.5 % during the forecast period 2025-2033.

Gene therapy is a modern medical technique that seeks to cure and prevent genetic disorders by modifying or introducing genetic material into the cells of a patient. This is achieved by using vectors, such as viruses, to deliver functional genes or gene-editing tools to the patient's body. By focusing on the specific genes that cause diseases, scientists aim to rectify or replace faulty genetic information, which could offer enduring solutions.

Gene therapy has immense potential for addressing inherited disorders, cardiovascular diseases, infectious diseases, and cancer. Gene therapy works by replacing or inactivating disease-causing genes. In some cases, gene therapy introduces new genes into the body to treat a specific disease. With gene therapy, healthcare providers deliver a healthy copy of a gene to cells inside the body. This healthy gene replaces a damaged gene, inactivates a mutated gene, or introduces an entirely new gene.

Market Dynamics: Drivers & Restraints

Growing Utilization of Advanced Delivery Vectors

The development and utilization of advanced delivery vectors are one of the most significant factors driving the growth of the US gene therapy market. Delivery vectors are essential for delivering therapeutic genes to specific cells in the body. The efficiency, precision, and safety of these vectors can significantly influence the

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effectiveness of gene therapies and their widespread adoption.

The development of more efficient vectors is enabling gene therapies to be applied to a wider range of diseases, including solid tumors, neurological disorders, and cardiovascular diseases, which were previously difficult to treat due to delivery challenges.

For instance, in October 2024, in San Francisco, Roche applied Dyno Therapeutics' engineered adeno-associated virus (AAV) capsid platform to develop next-generation AAV vectors for gene therapies targeting unspecified neurological diseases, through a collaboration that could generate more than $1 billion for Dyno.

Delivery vectors are critical technologies used to transport genetic material into a patient's cells. Adeno-associated virus (AAV) vectors are among the most commonly used delivery systems due to their ability to target a variety of tissues, including the liver, muscle, and eye. AAV-based gene therapies, such as Zolgensma for spinal muscular atrophy (SMA), are already proving highly effective.

High Cost associated with the Gene Therapies

The high cost of gene therapies is one of the most significant challenges impacting the growth and accessibility of gene therapies in the U.S.. While gene therapies have shown curative potential for many serious diseases, their expensive price tags pose barriers to widespread adoption.

For instance, the gene therapies Zynteglo and Skysona are priced at US$ 2.8 million and US$ 3 million per dose. Moreover, Zolgensma has a reported list price of US$ 2.1 million. The development of gene therapies is also an expensive and lengthy process. Clinical trials for gene therapies often span several years and involve high patient monitoring and expensive regulatory approval procedures.

For instance, Luxturna, a gene therapy for Leber's congenital amaurosis, took over 10 years from development to approval, and its cost is $850,000 per patient. Many gene therapies are personalized treatments that are tailored to the genetic makeup of individual patients, requiring bespoke production for each case. This individualization further increases costs.

For instance, CAR-T therapies such as Yescarta and Kymriah are customized for each patient by collecting and modifying the patient's T-cells, a process that involves

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complex manufacturing and quality control measures. These therapies can cost over $300,000 to $400,000 per patient.

Segment Analysis

The US gene therapy market is segmented based on approach, vector type, technique, and application.

Approach:

The in vivo segment in the approach is expected to dominate the US gene therapy market share

In-vivo gene therapy refers to the direct delivery of genetic material either intravenously (through an IV) or locally to a specific organ (eg, directly into the eye). In-vivo gene therapy works through the help of a vector, which directly inserts functional copies of a gene into target cells to treat a mutated or missing gene.

In vivo delivery of gene therapy has been proven in many areas of research. Some of the currently approved gene therapies deliver genetic material in vivo. Targeted in vivo gene therapy will continue to evolve as scientists continue to refine methods of gene delivery.

Furthermore, key players' strategies, such as investments, agreements, and technological innovations in AI, would propel this market growth. For instance, in June 2024, in the US, 2seventy Bio completed a $40 million asset purchase agreement (APA) with Novo Nordisk, selling its Hemophilia A program. Novo Nordisk also gains rights to 2seventy's MegaTAL in vivo gene editing technology, excluding its use in oncology and certain autoimmune-related cell therapies.

Also, in January 2025, Dyno Therapeutics, a genetic technology company specializing in AI-driven in vivo gene delivery, announced that Roche has exercised its option to license a novel adeno-associated virus (AAV) capsid for a gene therapy program targeting an undisclosed neurological disease. Dyno's platform uses AI and in vivo data to address gene delivery challenges. Overall, as research and development continue and technological evolution in in-vivo delivery methods plays an important role in this segment's growth during the forecast period.

Competitive Landscape

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The major players in the US gene therapy market include Alnylam Pharmaceuticals, Inc., NOVARTIS AG, Sarepta Therapeutics, Inc., Krystal Biotech, Inc., CSL, Bluebird Bio, Inc., SPARK THERAPEUTICS, INC., Ferring, Vertex Pharmaceuticals Incorporated, Amgen, Inc., and Orchard Therapeutics Plc, among others.

Key Developments

In February 2025, a global biotechnology leader in the US, CSL, announced the four-year results from the pivotal HOPE-B study confirming the long-term durability and safety of a one-time infusion of HEMGENIX (etranacogene dezaparvovec-drlb) for adults living with hemophilia B.

In September 2024, uniQure N.V., a leading gene therapy company advancing transformative therapies for patients with severe medical needs, announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to AMT-191, uniQure's investigational gene therapy for the treatment of Fabry disease, a rare, inherited genetic disease.

In April 2024, Ferring Pharmaceuticals announced a strategic agreement with SK pharmteco, a leading contract development and manufacturing organization (CDMO), to scale up commercial production of the drug substance for Adstiladrin (nadofaragene firadenovec-vncg), an FDA-approved intravesical gene therapy for bladder cancer.

Why Purchase the Report?

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Approach Performance & Market Positioning: Analyze product performance, market positioning, and growth potential to optimize strategies.

Real-World Evidence: Integrates patient feedback and data into Approach development for improved outcomes.

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Supply Chain Optimization: Assesses supply chain risks and distribution strategies for an efficient Approach to delivery.

Sustainability & Regulatory Impact: Focuses on eco-friendly practices and evolving regulations in healthcare.

Post-market Surveillance: Uses post-market data to enhance the product's safety and access.

Pharmacoeconomics & Value-Based Pricing: Analyzes the shift to value-based pricing and data-driven decision-making in R&D.

The US gene therapy market report delivers a detailed analysis with 45 key tables, more than 33 visually impactful figures, and 126 pages of expert insights, providing a complete view of the market landscape.

Target Audience 2024

Manufacturers: Pharmaceutical, Medical Device, Biotech Companies, Contract Manufacturers, Distributors, Hospitals.

Regulatory & Policy: Compliance Officers, Government, Health Economists,

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Market Access Specialists.

Vector Type & Innovation: AI/Robotics Providers, R&D Professionals, Clinical Trial Managers, Pharmacovigilance Experts.

Investors: Healthcare Investors, Venture Fund Investors, Pharma Marketing & Sales.

Consulting & Advisory: Healthcare Consultants, Industry Associations, Analysts.

Supply Chain: Distribution and Supply Chain Managers.

Consumers & Advocacy: Patients, Advocacy Groups, Insurance Companies.

Academic & Research: Academic Institutions.

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