

# Cell and Gene Therapies in Rare Disorders Market to Exhibit Rapid Growth Rate From (2024-2034), Asserts DelveInsight

*The Cell and Gene Therapies in Rare Disorders market size was ~2,000 million in 2023 to grow with a significant CAGR during the study period (2020-2034).*

LAS VEGAS, NEVADA, UNITED STATES, June 28, 2024 /EINPresswire.com/ -- DelveInsight's "Cell and Gene Therapies in Rare Disorders Market Insights, Epidemiology, and Market Forecast-2034" report offers an in-depth understanding of the Cell and Gene Therapies in Rare Disorders, historical

and forecasted epidemiology as well as the Cell and Gene Therapies in Rare Disorders market trends in the United States, EU4 (Germany, Spain, Italy, France) the United Kingdom and Japan.

To know in detail about the Cell and Gene Therapies in Rare Disorders market outlook, drug uptake, treatment scenario and epidemiology trends, Click here; [Cell and Gene Therapies in Rare Disorders Market Forecast](#)

Some of the key facts of the Cell and Gene Therapies in Rare Disorders Market Report:

The Cell and Gene Therapies in Rare Disorders market size was valued ~2,000 million in 2023 and is anticipated to grow with a significant CAGR during the study period (2020-2034)

In February 2024, Sangamo Therapeutics disclosed that following a Type D meeting, the US FDA agreed that data from a single, well-conducted study could serve as the primary basis for approving a Biologics License Application (BLA) for isaralgagenecivaparvovec, intended to treat Fabry disease.

In February 2024, REGENXBIO reported that the key phase of the RGX-121 trial for treating Hunter syndrome achieved its primary endpoints with statistically significant results.

Fabry disease, as estimated by the National Institutes of Health Genetics Home Reference, impacts approximately 1 in 40,000 to 60,000 males in the United States.

In 2023, Retinitis Pigmentosa had the highest prevalence among selected indications for Cell and Gene Therapies in Rare Disorders in the United States, while Hunter Syndrome reported the



Cell and Gene Therapies in Rare Disorders Market

lowest number of cases.

Approximately 432,000 prevalent cases of selected indications for Cell and Gene Therapies in Rare Disorders were estimated in the United States in 2023.

In 2023, nearly 8,000 prevalent cases of Fabry disease were reported in the United States.

In the United States, there were approximately 73,300 cases of retinitis pigmentosa among the eligible patient population.

Key Cell and Gene Therapies in Rare Disorders Companies: Pfizer, Sangamo Therapeutics, Orchard Therapeutics/ SR-Tiget, Novartis, TVAX Biomedical, Aivita Biomedical, RHEACELL GmbH & Co, Capricor Therapeutics, and others

Key Cell and Gene Therapies in Rare Disorders Therapies: Fidanacogene elaparvovec, GiroctocogenE fitelparvovec, OTL-103, OAV101, TVI-Brain-1, AV-GBM-1, ABCB5+ MSCs, CAP-1002, and others

The Cell and Gene Therapies in Rare Disorders market is expected to surge due to the disease's increasing prevalence and awareness during the forecast period. Furthermore, launching various multiple-stage Cell and Gene Therapies in Rare Disorders pipeline products will significantly revolutionize the Cell and Gene Therapies in Rare Disorders market dynamics.

### Cell and Gene Therapies in Rare Disorders Overview

Cell and gene therapies are cutting-edge medical technologies with the potential to significantly improve patient care and society at large. New developments in these innovative medicines have the potential to revolutionise medicine and the ability to treat a wide range of incurable diseases.

Get a Free sample for the Cell and Gene Therapies in Rare Disorders Market Report:

[https://www.delveinsight.com/report-store/gene-and-cell-therapies-in-rare-disorder-market?utm\\_source=einpresswire&utm\\_medium=pressrelease&utm\\_campaign=gpr](https://www.delveinsight.com/report-store/gene-and-cell-therapies-in-rare-disorder-market?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=gpr)

### Cell and Gene Therapies in Rare Disorders Epidemiology

The epidemiology section provides insights into the historical, current, and forecasted epidemiology trends in the seven major countries (7MM) from 2020 to 2034. It helps to recognize the causes of current and forecasted trends by exploring numerous studies and views of key opinion leaders. The epidemiology section also provides a detailed analysis of the diagnosed patient pool and future trends.

### Cell and Gene Therapies in Rare Disorders Epidemiology Segmentation:

The Cell and Gene Therapies in Rare Disorders market report proffers epidemiological analysis for the study period 2020–2034 in the 7MM segmented into:

Total Prevalent Cases of Selected Indications for Cell and Gene Therapies in Rare Disorders in the 7MM

Total Indication-wise Eligible Cases for Cell and Gene Therapies in Rare Disorders in the 7MM

Total indication-wise Treated Cases for Cell and Gene Therapies in Rare Disorders in the 7MM

Download the report to understand which factors are driving Cell and Gene Therapies in Rare

## Disorders epidemiology trends @ [Cell and Gene Therapies in Rare Disorders Epidemiology Forecast](#)

### Cell and Gene Therapies in Rare Disorders Drugs Uptake and Pipeline Development Activities

The drugs uptake section focuses on the rate of uptake of the potential drugs recently launched in the Cell and Gene Therapies in Rare Disorders market or expected to get launched during the study period. The analysis covers Cell and Gene Therapies in Rare Disorders market uptake by drugs, patient uptake by therapies, and sales of each drug.

Moreover, the therapeutics assessment section helps understand the drugs with the most rapid uptake and the reasons behind the maximal use of the drugs. Additionally, it compares the drugs based on market share.

The report also covers the Cell and Gene Therapies in Rare Disorders Pipeline Development Activities. It provides valuable insights about different therapeutic candidates in various stages and the key companies involved in developing targeted therapeutics. It also analyzes recent developments such as collaborations, acquisitions, mergers, licensing patent details, and other information for emerging therapies.

### Cell and Gene Therapies in Rare Disorders Therapies and Key Companies

Fidanacogene elaparvovec: Pfizer

GiroctocogenE fitelparvovec: Pfizer/ Sangamo Therapeutics

OTL-103: Orchard Therapeutics/ SR-Tiget

OAV101: Novartis

TVI-Brain-1: TVAX Biomedical

AV-GBM-1: Aivita Biomedical

ABCB5+ MSCs: RHEACELL GmbH & Co

CAP-1002: Capricor Therapeutics

### Cell and Gene Therapies in Rare Disorders Market Strengths

AAVs have emerged as the predominant vectors for delivering genes of interest to target tissues with improved specificity, efficiency, and safety.

Companies involved in rare disease therapy development usually have niche markets, lower competition, higher pricing power and so on.

### Cell and Gene Therapies in Rare Disorders Market Barriers

To ensure development of treatments for rare disease, including gene therapies, biopharmaceutical companies are incentivized with the prospect of Orphan and Fast Track Designations and longer market exclusivity.

Gene therapies are expected to become more relevant for rare diseases with the potential of a paradigm shift from symptomatic alleviation to disease modification and even cure

### Scope of the Cell and Gene Therapies in Rare Disorders Market Report

Study Period: 2020–2034

Coverage: 7MM [The United States, EU5 (Germany, France, Italy, Spain, and the United Kingdom),

and Japan]

Key Cell and Gene Therapies in Rare Disorders Companies: Pfizer, Sangamo Therapeutics, Orchard Therapeutics/ SR-Tiget, Novartis, TVAX Biomedical, Aivita Biomedical, RHEACELL GmbH & Co, Capricor Therapeutics, and others

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Cell and Gene Therapies in Rare Disorders Therapeutic Assessment: Cell and Gene Therapies in Rare Disorders current marketed and Cell and Gene Therapies in Rare Disorders emerging therapies

Cell and Gene Therapies in Rare Disorders Market Dynamics: Cell and Gene Therapies in Rare Disorders market drivers and Cell and Gene Therapies in Rare Disorders market barriers

Competitive Intelligence Analysis: SWOT analysis, PESTLE analysis, Porter's five forces, BCG Matrix, Market entry strategies

Cell and Gene Therapies in Rare Disorders Unmet Needs, KOL's views, Analyst's views, Cell and Gene Therapies in Rare Disorders Market Access and Reimbursement

To know more about Cell and Gene Therapies in Rare Disorders companies working in the treatment market, visit @ [Cell and Gene Therapies in Rare Disorders Treatment Landscape](#)

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## About DelveInsight

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It also offers Healthcare Consulting Services, which benefits in market analysis to accelerate the business growth and overcome challenges with a practical approach.

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