

# Hemophilia Gene Therapy Market By Type (Hemophilia A, Hemophilia B) , By End User (Hospitals, Specialty Clinics, Others) : Global Opportunity Analysis and Industry Forecast, 2024-2033

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## Abstracts

### Hemophilia Gene Therapy Market

The hemophilia gene therapy market was valued at \$0.4 billion in 2023 and is projected to reach \$3.0 billion by 2033, growing at a CAGR of 23.6% from 2024 to 2033.

Hemophilia is a genetic blood disorder that involves improper coagulation of blood due to deficiency or abnormality in certain clotting factors. Gene therapy for this disease is an innovative approach that includes the delivery of an active copy of the dysfunctional gene responsible for the production of clotting factors. The delivered gene induces the patient's cells to produce the clotting factors, preventing the requirement for frequent factor replacement infusions. This treatment remains highly reliable as it cures the root cause of the disease, eliminating the risk of reversion.

Advancements in gene transfer technology have enhanced the reliability and efficacy of gene therapies, thereby propelling the development of the market. In addition, the ability of gene therapy to treat the root cause of hemophilia entirely prevents patients from recurrent factor replacement sessions and augments the market growth significantly. Furthermore, upsurge in government support in the form of funding & incentives for boosting the R&D of healthcare and biotechnology has elevated the availability of gene therapy for patients. This acts as a key driver of the hemophilia gene therapy market. A notable therapeutic approach currently being explored by researchers and doctors is gene editing. Genome editing tools such as zinc finger nucleases and transcription

activator-like effector nucleases are being used to modify a patient's DNA to cure the disease. This approach is poised to be trending in the future due to its highly targeted and fast-curing ability.

However, the high cost of gene therapies prevents several budget-sensitive individuals from undergoing the treatment, thereby hampering the development of the market. For instance, the two new gene therapies approved by the U.S. Food and Drug Administration in 2022-2023 have list prices of \$2.9 million and 3.5 million. On the contrary, rise in strategic collaborations between pharmaceutical firms, research institutions, and regulatory bodies is anticipated to accelerate therapeutic development & reduce the associated expenses. This surge in collaborations & reduction in expenses is expected to present lucrative opportunities for the market growth.

### Segment Review

The hemophilia gene therapy market is segmented into type, end user, and region. On the basis of type, the market is bifurcated into hemophilia A and hemophilia B. Depending on end user, it is divided into hospitals, specialty clinics, and others. Region wise, it is analyzed across North America, Europe, Asia-Pacific, and LAMEA.

### Key Findings

On the basis of type, the hemophilia A segment is expected to dominate the market by 2033.

Region wise, North America was the highest revenue generator in 2023.

### Competition Analysis

The major players in the global hemophilia gene therapy market include BioMarin Pharmaceutical Inc., Spark Therapeutics, Inc., uniQure N.V., Pfizer Inc., Freeline Therapeutics Holdings plc, Sangamo Therapeutics, Inc., Bioverativ, CSL Behring, Ultragenyx, and Shire Plc. These major players have adopted various key development strategies such as business expansion, new product launches, and partnerships to strengthen their foothold in the competitive market.

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SWOT Analysis

## Key Market Segments

### By Type

Hemophilia A

Hemophilia B

### By End User

Hospitals

Specialty Clinics

Others

### By Region

North America

U.S.

Canada

Mexico

Europe

France

Germany

Italy

Spain

UK

Rest of Europe

Asia-Pacific

China

Japan

India

South Korea

Australia

Rest of Asia-Pacific

LAMEA

Brazil

South Africa

Saudi Arabia

Rest of LAMEA

Key Market Players

BioMarin Pharmaceutical Inc.

Spark Therapeutics, Inc.

uniQure N.V.

Pfizer Inc.

Freeline Therapeutics Holdings plc.

Sangamo Therapeutics, Inc

Bioverativ

CSL Behring

Ultragenyx

Shire Plc

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