

# 2018-2023 Global Hemophilia Gene Therapy Market Report (Status and Outlook)

https://marketpublishers.com/r/272806B8F08EN.html

Date: September 2018

Pages: 118

Price: US\$ 4,660.00 (Single User License)

ID: 272806B8F08EN

## **Abstracts**

The report requires updating with new data and is sent in 48 hours after order is placed.

In this report, LP Information studies the present scenario (with the base year being 2017) and the growth prospects of global Hemophilia Gene Therapy market for 2018-2023.

Hemophilia is a rare bleeding disorder in which the blood does not clot normally. Hemophilia is a monogenic disease (a disease that is caused by a genetic defect in a single gene). There are two types of hemophilia caused by mutations in genes that encode protein factors which help the blood clot and stop bleeding when blood vessels are injured. Individuals with hemophilia experience bleeding episodes after injuries and spontaneous bleeding episodes that often lead to joint disease such as arthritis. The most frequent forms of hemophilia affect males.

About 80% of them have hemophilia A, which affects the clotting factor VIII. The second most common form, hemophilia B, is due to a deficiency of the clotting factor IX. Several biotechs are racing to launch the first gene therapy for hemophilia. Currently, uniQure in the Netherlands and Spark Therapeutics in the US have the most advanced programs. Spark scored a victory in December when it presented Phase I/II for its candidate SPK-9001. The gene therapy was able to reduce annual bleeding episodes by 97%, as compared to its competitor uniQure's candidate, AMT-060. However, uniQure has fought back. The company added a modification in its gene therapy that is known to increase clotting activity by 8- to 9- fold. This improved version has already been cleared to start a Phase III clinical trial in both Europe and the US.

Hemophilia B has traditionally been the main focus of biotechs, since most big pharma efforts were focused on the bigger hemophilia A market. In addition, applying gene therapy to hemophilia A is more challenging; The gene coding for the factor IX protein



missing in hemophilia B is simply smaller than that for factor VIII missing in hemophilia A, and therefore easier to fit in the viral vectors used for gene delivery. US-based BioMarin is leading the development of a gene therapy. To overcome the size limit, the company has deleted a region from the factor VIII protein that is not necessary for clotting.

Hemophilia treatment is currently in the pre-clinical stage. And the multiple treatments that are underway might significantly improve the quality of life of patients with hemophilia, by getting rid of frequent infusions and hospital visits, and transitioning patients from severe to mild hemophilia.

Over the next five years, LPI(LP Information) projects that Hemophilia Gene Therapy will register a xx% CAGR in terms of revenue, reach US\$ xx million by 2023, from US\$ xx million in 2017.

This report presents a comprehensive overview, market shares and growth opportunities of Hemophilia Gene Therapy market by product type, application, key companies and key regions.

To calculate the market size, LP Information considers value generated from the sales of the following segments:

Segmentation by product type:

Hemophilia A

Hemophilia B

Segmentation by application:

Hemophilia A Gene Therapy

Hemophilia B Gene Therapy

We can also provide the customized separate regional or country-level reports, for the following regions:

**Americas** 



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Canada
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Brazil
APAC
China
Japan
Korea
Southeast Asia
India
Australia
Europe
Germany
France
UK
Italy
Russia
Spain
Middle East & Africa
Egypt



South Africa

	Israel		
	Turkey		
	GCC Countries		
The report also presents the market competition landscape and a corresponding detailed analysis of the major players in the market. The key players covered in this report:			
	Spark Therapeutics		
Shire P	Ultragenyx		
	Shire PLC		
	Sangamo Therapeutics		
	ioverativ		
	BioMarin		
	uniQure		
	Freeline Therapeutics		
	ion, this report discusses the key drivers influencing market growth, inities, the challenges and the risks faced by key players and the market as a		

Research objectives

development.

To study and analyze the global Hemophilia Gene Therapy market size by key

whole. It also analyzes key emerging trends and their impact on present and future



regions/countries, product type and application, history data from 2013 to 2017, and forecast to 2023.

To understand the structure of Hemophilia Gene Therapy market by identifying its various subsegments.

Focuses on the key global Hemophilia Gene Therapy players, to define, describe and analyze the value, market share, market competition landscape, SWOT analysis and development plans in next few years.

To analyze the Hemophilia Gene Therapy with respect to individual growth trends, future prospects, and their contribution to the total market.

To share detailed information about the key factors influencing the growth of the market (growth potential, opportunities, drivers, industry-specific challenges and risks).

To project the size of Hemophilia Gene Therapy submarkets, with respect to key regions (along with their respective key countries).

To analyze competitive developments such as expansions, agreements, new product launches and acquisitions in the market.

To strategically profile the key players and comprehensively analyze their growth strategies.



## **Contents**

#### 1 SCOPE OF THE REPORT

- 1.1 Market Introduction
- 1.2 Research Objectives
- 1.3 Years Considered
- 1.4 Market Research Methodology
- 1.5 Economic Indicators
- 1.6 Currency Considered

#### 2 EXECUTIVE SUMMARY

- 2.1 World Market Overview
  - 2.1.1 Global Hemophilia Gene Therapy Market Size 2013-2023
  - 2.1.2 Hemophilia Gene Therapy Market Size CAGR by Region
- 2.2 Hemophilia Gene Therapy Segment by Type
  - 2.2.1 Hemophilia A
  - 2.2.2 Hemophilia B
- 2.3 Hemophilia Gene Therapy Market Size by Type
- 2.3.1 Global Hemophilia Gene Therapy Market Size Market Share by Type (2013-2018)
- 2.3.2 Global Hemophilia Gene Therapy Market Size Growth Rate by Type (2013-2018)
- 2.4 Hemophilia Gene Therapy Segment by Application
  - 2.4.1 Hemophilia A Gene Therapy
  - 2.4.2 Hemophilia B Gene Therapy
- 2.5 Hemophilia Gene Therapy Market Size by Application
- 2.5.1 Global Hemophilia Gene Therapy Market Size Market Share by Application (2013-2018)
- 2.5.2 Global Hemophilia Gene Therapy Market Size Growth Rate by Application (2013-2018)

#### 3 GLOBAL HEMOPHILIA GENE THERAPY BY PLAYERS

- 3.1 Global Hemophilia Gene Therapy Market Size Market Share by Players
  - 3.1.1 Global Hemophilia Gene Therapy Market Size by Players (2016-2018)
- 3.1.2 Global Hemophilia Gene Therapy Market Size Market Share by Players (2016-2018)
- 3.2 Global Hemophilia Gene Therapy Key Players Head office and Products Offered



- 3.3 Market Concentration Rate Analysis
  - 3.3.1 Competition Landscape Analysis
  - 3.3.2 Concentration Ratio (CR3, CR5 and CR10) (2016-2018)
- 3.4 New Products and Potential Entrants
- 3.5 Mergers & Acquisitions, Expansion

#### **4 HEMOPHILIA GENE THERAPY BY REGIONS**

- 4.1 Hemophilia Gene Therapy Market Size by Regions
- 4.2 Americas Hemophilia Gene Therapy Market Size Growth
- 4.3 APAC Hemophilia Gene Therapy Market Size Growth
- 4.4 Europe Hemophilia Gene Therapy Market Size Growth
- 4.5 Middle East & Africa Hemophilia Gene Therapy Market Size Growth

#### **5 AMERICAS**

- 5.1 Americas Hemophilia Gene Therapy Market Size by Countries
- 5.2 Americas Hemophilia Gene Therapy Market Size by Type
- 5.3 Americas Hemophilia Gene Therapy Market Size by Application
- 5.4 United States
- 5.5 Canada
- 5.6 Mexico
- 5.7 Key Economic Indicators of Few Americas Countries

#### 6 APAC

- 6.1 APAC Hemophilia Gene Therapy Market Size by Countries
- 6.2 APAC Hemophilia Gene Therapy Market Size by Type
- 6.3 APAC Hemophilia Gene Therapy Market Size by Application
- 6.4 China
- 6.5 Japan
- 6.6 Korea
- 6.7 Southeast Asia
- 6.8 India
- 6.9 Australia
- 6.10 Key Economic Indicators of Few APAC Countries

#### **7 EUROPE**



- 7.1 Europe Hemophilia Gene Therapy by Countries
- 7.2 Europe Hemophilia Gene Therapy Market Size by Type
- 7.3 Europe Hemophilia Gene Therapy Market Size by Application
- 7.4 Germany
- 7.5 France
- 7.6 UK
- 7.7 Italy
- 7.8 Russia
- 7.9 Spain
- 7.10 Key Economic Indicators of Few Europe Countries

#### **8 MIDDLE EAST & AFRICA**

- 8.1 Middle East & Africa Hemophilia Gene Therapy by Countries
- 8.2 Middle East & Africa Hemophilia Gene Therapy Market Size by Type
- 8.3 Middle East & Africa Hemophilia Gene Therapy Market Size by Application
- 8.4 Egypt
- 8.5 South Africa
- 8.6 Israel
- 8.7 Turkey
- 8.8 GCC Countries

#### 9 MARKET DRIVERS, CHALLENGES AND TRENDS

- 9.1 Market Drivers and Impact
  - 9.1.1 Growing Demand from Key Regions
  - 9.1.2 Growing Demand from Key Applications and Potential Industries
- 9.2 Market Challenges and Impact
- 9.3 Market Trends

## 10 GLOBAL HEMOPHILIA GENE THERAPY MARKET FORECAST

- 10.1 Global Hemophilia Gene Therapy Market Size Forecast (2018-2023)
- 10.2 Global Hemophilia Gene Therapy Forecast by Regions
  - 10.2.1 Global Hemophilia Gene Therapy Forecast by Regions (2018-2023)
  - 10.2.2 Americas Market Forecast
  - 10.2.3 APAC Market Forecast
  - 10.2.4 Europe Market Forecast
- 10.2.5 Middle East & Africa Market Forecast



- 10.3 Americas Forecast by Countries
  - 10.3.1 United States Market Forecast
  - 10.3.2 Canada Market Forecast
  - 10.3.3 Mexico Market Forecast
  - 10.3.4 Brazil Market Forecast
- 10.4 APAC Forecast by Countries
  - 10.4.1 China Market Forecast
  - 10.4.2 Japan Market Forecast
  - 10.4.3 Korea Market Forecast
  - 10.4.4 Southeast Asia Market Forecast
  - 10.4.5 India Market Forecast
- 10.4.6 Australia Market Forecast
- 10.5 Europe Forecast by Countries
- 10.5.1 Germany Market Forecast
- 10.5.2 France Market Forecast
- 10.5.3 UK Market Forecast
- 10.5.4 Italy Market Forecast
- 10.5.5 Russia Market Forecast
- 10.5.6 Spain Market Forecast
- 10.6 Middle East & Africa Forecast by Countries
  - 10.6.1 Egypt Market Forecast
  - 10.6.2 South Africa Market Forecast
  - 10.6.3 Israel Market Forecast
  - 10.6.4 Turkey Market Forecast
- 10.6.5 GCC Countries Market Forecast
- 10.7 Global Hemophilia Gene Therapy Forecast by Type
- 10.8 Global Hemophilia Gene Therapy Forecast by Application

### 11 KEY PLAYERS ANALYSIS

- 11.1 Spark Therapeutics
  - 11.1.1 Company Details
  - 11.1.2 Hemophilia Gene Therapy Product Offered
  - 11.1.3 Spark Therapeutics Hemophilia Gene Therapy Revenue, Gross Margin and
- Market Share (2016-2018)
  - 11.1.4 Main Business Overview
  - 11.1.5 Spark Therapeutics News
- 11.2 Ultragenyx
- 11.2.1 Company Details



- 11.2.2 Hemophilia Gene Therapy Product Offered
- 11.2.3 Ultragenyx Hemophilia Gene Therapy Revenue, Gross Margin and Market Share (2016-2018)
  - 11.2.4 Main Business Overview
  - 11.2.5 Ultragenyx News
- 11.3 Shire PLC
  - 11.3.1 Company Details
  - 11.3.2 Hemophilia Gene Therapy Product Offered
- 11.3.3 Shire PLC Hemophilia Gene Therapy Revenue, Gross Margin and Market Share (2016-2018)
  - 11.3.4 Main Business Overview
  - 11.3.5 Shire PLC News
- 11.4 Sangamo Therapeutics
  - 11.4.1 Company Details
  - 11.4.2 Hemophilia Gene Therapy Product Offered
- 11.4.3 Sangamo Therapeutics Hemophilia Gene Therapy Revenue, Gross Margin and Market Share (2016-2018)
  - 11.4.4 Main Business Overview
  - 11.4.5 Sangamo Therapeutics News
- 11.5 Bioverativ
  - 11.5.1 Company Details
  - 11.5.2 Hemophilia Gene Therapy Product Offered
- 11.5.3 Bioverativ Hemophilia Gene Therapy Revenue, Gross Margin and Market Share (2016-2018)
- 11.5.4 Main Business Overview
- 11.5.5 Bioverativ News
- 11.6 BioMarin
- 11.6.1 Company Details
- 11.6.2 Hemophilia Gene Therapy Product Offered
- 11.6.3 BioMarin Hemophilia Gene Therapy Revenue, Gross Margin and Market Share (2016-2018)
  - 11.6.4 Main Business Overview
  - 11.6.5 BioMarin News
- 11.7 uniQure
- 11.7.1 Company Details
- 11.7.2 Hemophilia Gene Therapy Product Offered
- 11.7.3 uniQure Hemophilia Gene Therapy Revenue, Gross Margin and Market Share (2016-2018)
- 11.7.4 Main Business Overview



- 11.7.5 uniQure News
- 11.8 Freeline Therapeutics
  - 11.8.1 Company Details
  - 11.8.2 Hemophilia Gene Therapy Product Offered
- 11.8.3 Freeline Therapeutics Hemophilia Gene Therapy Revenue, Gross Margin and Market Share (2016-2018)
  - 11.8.4 Main Business Overview
  - 11.8.5 Freeline Therapeutics News

## 12 RESEARCH FINDINGS AND CONCLUSION



## **List Of Tables**

#### LIST OF TABLES AND FIGURES

Table Product Specifications of Hemophilia Gene Therapy

Figure Hemophilia Gene Therapy Report Years Considered

Figure Market Research Methodology

Figure Global Hemophilia Gene Therapy Market Size Growth Rate 2013-2023 (\$ Millions)

Table Hemophilia Gene Therapy Market Size CAGR by Region 2013-2023 (\$ Millions)

Table Major Players of Hemophilia A

Table Major Players of Hemophilia B

Table Market Size by Type (2013-2018) (\$ Millions)

Table Global Hemophilia Gene Therapy Market Size Market Share by Type (2013-2018)

Figure Global Hemophilia Gene Therapy Market Size Market Share by Type (2013-2018)

Figure Global Hemophilia A Market Size Growth Rate

Figure Global Hemophilia B Market Size Growth Rate

Figure Hemophilia Gene Therapy Consumed in Hemophilia A Gene Therapy

Figure Global Hemophilia Gene Therapy Market: Hemophilia A Gene Therapy (2013-2018) (\$ Millions)

Figure Global Hemophilia A Gene Therapy YoY Growth (\$ Millions)

Figure Hemophilia Gene Therapy Consumed in Hemophilia B Gene Therapy

Figure Global Hemophilia Gene Therapy Market: Hemophilia B Gene Therapy (2013-2018) (\$ Millions)

Figure Global Hemophilia B Gene Therapy YoY Growth (\$ Millions)

Table Global Hemophilia Gene Therapy Market Size by Application (2013-2018) (\$ Millions)

Table Global Hemophilia Gene Therapy Market Size Market Share by Application (2013-2018)

Figure Global Hemophilia Gene Therapy Market Size Market Share by Application (2013-2018)

Figure Global Hemophilia Gene Therapy Market Size in Hemophilia A Gene Therapy Growth Rate

Figure Global Hemophilia Gene Therapy Market Size in Hemophilia B Gene Therapy Growth Rate

Table Global Hemophilia Gene Therapy Revenue by Players (2016-2018) (\$ Millions) Table Global Hemophilia Gene Therapy Revenue Market Share by Players (2016-2018)



Figure Global Hemophilia Gene Therapy Revenue Market Share by Players in 2017 Table Global Hemophilia Gene Therapy Key Players Head office and Products Offered Table Hemophilia Gene Therapy Concentration Ratio (CR3, CR5 and CR10) (2016-2018)

Table Global Hemophilia Gene Therapy Market Size by Regions 2013-2018 (\$ Millions) Table Global Hemophilia Gene Therapy Market Size Market Share by Regions 2013-2018

Figure Global Hemophilia Gene Therapy Market Size Market Share by Regions 2013-2018

Figure Americas Hemophilia Gene Therapy Market Size 2013-2018 (\$ Millions)

Figure APAC Hemophilia Gene Therapy Market Size 2013-2018 (\$ Millions)

Figure Europe Hemophilia Gene Therapy Market Size 2013-2018 (\$ Millions)

Figure Middle East & Africa Hemophilia Gene Therapy Market Size 2013-2018 (\$ Millions)

Table Americas Hemophilia Gene Therapy Market Size by Countries (2013-2018) (\$ Millions)

Table Americas Hemophilia Gene Therapy Market Size Market Share by Countries (2013-2018)

Figure Americas Hemophilia Gene Therapy Market Size Market Share by Countries in 2017

Table Americas Hemophilia Gene Therapy Market Size by Type (2013-2018) (\$ Millions)

Table Americas Hemophilia Gene Therapy Market Size Market Share by Type (2013-2018)

Figure Americas Hemophilia Gene Therapy Market Size Market Share by Type in 2017 Table Americas Hemophilia Gene Therapy Market Size by Application (2013-2018) (\$ Millions)

Table Americas Hemophilia Gene Therapy Market Size Market Share by Application (2013-2018)

Figure Americas Hemophilia Gene Therapy Market Size Market Share by Application in 2017

Figure United States Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions)

Figure Canada Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure Mexico Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Table APAC Hemophilia Gene Therapy Market Size by Countries (2013-2018) (\$ Millions)

Table APAC Hemophilia Gene Therapy Market Size Market Share by Countries (2013-2018)



Figure APAC Hemophilia Gene Therapy Market Size Market Share by Countries in 2017

Table APAC Hemophilia Gene Therapy Market Size by Type (2013-2018) (\$ Millions) Table APAC Hemophilia Gene Therapy Market Size Market Share by Type (2013-2018) Figure APAC Hemophilia Gene Therapy Market Size Market Share by Type in 2017 Table APAC Hemophilia Gene Therapy Market Size by Application (2013-2018) (\$ Millions)

Table APAC Hemophilia Gene Therapy Market Size Market Share by Application (2013-2018)

Figure APAC Hemophilia Gene Therapy Market Size Market Share by Application in 2017

Figure China Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure Japan Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure Korea Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure Southeast Asia Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions)

Figure India Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure Australia Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Table Europe Hemophilia Gene Therapy Market Size by Countries (2013-2018) (\$ Millions)

Table Europe Hemophilia Gene Therapy Market Size Market Share by Countries (2013-2018)

Figure Europe Hemophilia Gene Therapy Market Size Market Share by Countries in 2017

Table Europe Hemophilia Gene Therapy Market Size by Type (2013-2018) (\$ Millions) Table Europe Hemophilia Gene Therapy Market Size Market Share by Type (2013-2018)

Figure Europe Hemophilia Gene Therapy Market Size Market Share by Type in 2017 Table Europe Hemophilia Gene Therapy Market Size by Application (2013-2018) (\$ Millions)

Table Europe Hemophilia Gene Therapy Market Size Market Share by Application (2013-2018)

Figure Europe Hemophilia Gene Therapy Market Size Market Share by Application in 2017

Figure Germany Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure France Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure UK Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure Italy Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure Russia Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions)



Figure Spain Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Table Middle East & Africa Hemophilia Gene Therapy Market Size by Countries (2013-2018) (\$ Millions)

Table Middle East & Africa Hemophilia Gene Therapy Market Size Market Share by Countries (2013-2018)

Figure Middle East & Africa Hemophilia Gene Therapy Market Size Market Share by Countries in 2017

Table Middle East & Africa Hemophilia Gene Therapy Market Size by Type (2013-2018) (\$ Millions)

Table Middle East & Africa Hemophilia Gene Therapy Market Size Market Share by Type (2013-2018)

Figure Middle East & Africa Hemophilia Gene Therapy Market Size Market Share by Type in 2017

Table Middle East & Africa Hemophilia Gene Therapy Market Size by Application (2013-2018) (\$ Millions)

Table Middle East & Africa Hemophilia Gene Therapy Market Size Market Share by Application (2013-2018)

Figure Middle East & Africa Hemophilia Gene Therapy Market Size Market Share by Application in 2017

Figure Egypt Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure South Africa Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions)

Figure Israel Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure Turkey Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions) Figure GCC Countries Hemophilia Gene Therapy Market Size Growth 2013-2018 (\$ Millions)

Figure Global Hemophilia Gene Therapy arket Size Forecast (2018-2023) (\$ Millions) Table Global Hemophilia Gene Therapy Market Size Forecast by Regions (2018-2023) (\$ Millions)

Table Global Hemophilia Gene Therapy Market Size Market Share Forecast by Regions Figure Americas Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure APAC Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Europe Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Middle East & Africa Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure United States Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Canada Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Mexico Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Brazil Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)



Figure China Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Japan Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Korea Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Southeast Asia Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure India Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Australia Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Germany Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure France Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure UK Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Italy Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Russia Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Spain Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Egypt Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure South Africa Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Israel Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure Turkey Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Figure GCC Countries Hemophilia Gene Therapy Market Size 2018-2023 (\$ Millions)

Table Global Hemophilia Gene Therapy Market Size Forecast by Type (2018-2023) (\$ Millions)

Table Global Hemophilia Gene Therapy Market Size Market Share Forecast by Type (2018-2023)

Table Global Hemophilia Gene Therapy Market Size Forecast by Application (2018-2023) (\$ Millions)

Table Global Hemophilia Gene Therapy Market Size Market Share Forecast by Application (2018-2023)

Table Spark Therapeutics Basic Information, Head Office, Major Market Areas and Its Competitors

Table Spark Therapeutics Hemophilia Gene Therapy Revenue and Gross Margin (2016-2018)

Figure Spark Therapeutics Hemophilia Gene Therapy Market Share (2016-2018) Table Ultragenyx Basic Information, Head Office, Major Market Areas and Its Competitors

Table Ultragenyx Hemophilia Gene Therapy Revenue and Gross Margin (2016-2018) Figure Ultragenyx Hemophilia Gene Therapy Market Share (2016-2018)

Table Shire PLC Basic Information, Head Office, Major Market Areas and Its Competitors

Table Shire PLC Hemophilia Gene Therapy Revenue and Gross Margin (2016-2018)

Figure Shire PLC Hemophilia Gene Therapy Market Share (2016-2018)

Table Sangamo Therapeutics Basic Information, Head Office, Major Market Areas and



Its Competitors

Table Sangamo Therapeutics Hemophilia Gene Therapy Revenue and Gross Margin (2016-2018)

Figure Sangamo Therapeutics Hemophilia Gene Therapy Market Share (2016-2018) Table Bioverativ Basic Information, Head Office, Major Market Areas and Its Competitors

Table Bioverativ Hemophilia Gene Therapy Revenue and Gross Margin (2016-2018) Figure Bioverativ Hemophilia Gene Therapy Market Share (2016-2018)

Table BioMarin Basic Information, Head Office, Major Market Areas and Its Competitors Table BioMarin Hemophilia Gene Therapy Revenue and Gross Margin (2016-2018) Figure BioMarin Hemophilia Gene Therapy Market Share (2016-2018)

Table uniQure Basic Information, Head Office, Major Market Areas and Its Competitors Table uniQure Hemophilia Gene Therapy Revenue and Gross Margin (2016-2018) Figure uniQure Hemophilia Gene Therapy Market Share (2016-2018)

Table Freeline Therapeutics Basic Information, Head Office, Major Market Areas and Its Competitors

Table Freeline Therapeutics Hemophilia Gene Therapy Revenue and Gross Margin (2016-2018)

Figure Freeline Therapeutics Hemophilia Gene Therapy Market Share (2016-2018)



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