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

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Abstracts

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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

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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

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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

Ultragenyx

Shire PLC

Sangamo Therapeutics

Bioverativ

BioMarin

uniQure

Freeline Therapeutics

In addition, this report discusses the key drivers influencing market growth, opportunities, the challenges and the risks faced by key players and the market as a whole. It also analyzes key emerging trends and their impact on present and future development.

Research objectives

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

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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.

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