

Global Hemophilia Gene Therapy Market 2018 by Manufacturers, Countries, Type and Application, Forecast to 2023

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Abstracts

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.

SCOPE OF THE REPORT:

This report studies the Hemophilia Gene Therapy market status and outlook of Global and major regions, from angles of players, countries, product types and end industries; this report analyzes the top players in global market, and splits the Hemophilia Gene Therapy market by product type and applications/end industries.

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.

The global Hemophilia Gene Therapy market is valued at xx million USD in 2017 and is expected to reach xx million USD by the end of 2023, growing at a CAGR of xx% between 2017 and 2023.

The Asia-Pacific will occupy for more market share in following years, especially in China, also fast growing India and Southeast Asia regions.

North America, especially The United States, will still play an important role which cannot be ignored. Any changes from United States might affect the development trend of Hemophilia Gene Therapy.

Europe also play important roles in global market, with market size of xx million USD in 2017 and will be xx million USD in 2023, with a CAGR of xx%.

Market Segment by Companies, this report covers

Spark Therapeutics

Ultragenyx

Shire PLC

Sangamo Therapeutics

Bioverativ

BioMarin

uniQure

Freeline Therapeutics

Market Segment by Regions, regional analysis covers

North America (United States, Canada and Mexico)

Europe (Germany, France, UK, Russia and Italy)

Asia-Pacific (China, Japan, Korea, India and Southeast Asia)

South America (Brazil, Argentina, Colombia)

Middle East and Africa (Saudi Arabia, UAE, Egypt, Nigeria and South Africa)

Market Segment by Type, covers

Hemophilia A

Hemophilia B

Market Segment by Applications, can be divided into

Hemophilia A Gene Therapy

Hemophilia B Gene Therapy

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