

# Hemophilia A - Pipeline Insight, 2021

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## Abstracts

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DelveInsight's, "Hemophilia A - Pipeline Insight, 2021," report provides comprehensive insights about 30+ companies and 40+ pipeline drugs in Hemophilia A pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

### Geography Covered

Global coverage

### Hemophilia A Understanding

#### Hemophilia A: Overview

Hemophilia A, also known as classical hemophilia, is a genetic bleeding disorder caused by insufficient levels of a blood protein called factor VIII. Factor VIII is a clotting factor. Hemophilia A is caused by disruptions or changes (mutations) to the F8 gene located on the X chromosome. This mutation may be inherited or occur randomly with no previous family history of the disorder (spontaneously). Hemophilia A is mostly expressed in males but some females who carry the gene may have mild or, rarely, severe symptoms of bleeding.

A diagnosis of hemophilia A is based upon identification of characteristic symptoms, a detailed patient history, a thorough clinical evaluation, and a variety of specialized

laboratory tests. The main medication to treat hemophilia A is concentrated FVIII product, called clotting factor or simply factor. There are two types of clotting factor: plasma-derived and recombinant. The U.S. Food and Drug Administration (FDA) has approved NovoSeven RT, a genetically engineered (recombinant) version of activated coagulation factor VII (factor VIIa), for the treatment of inhibitors in hemophilia A. In 2020, FDA approved Sevenfact (recombinant human coagulation factor VIIa expressed in the mammary gland of genetically engineered rabbits and secreted into the rabbits' milk) for treatment and control of bleeding in adults and adolescents age 12 and older with hemophilia A or B with inhibitors (neutralizing antibodies).

## Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Hemophilia A R&D. The therapies under development are focused on novel approaches for Hemophilia A.

## Hemophilia A Emerging Drugs Chapters

This segment of the Hemophilia A report encloses its detailed analysis of various drugs in different stages of clinical development, including phase III, II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

## Hemophilia A Emerging Drugs

### SPK-8011: Spark Therapeutics

Spark Therapeutics is developing SPK-8011, an investigational gene therapy for hemophilia A, or factor VIII deficiency. Spark retains global commercialization rights to its SPK-FVIII program includes both SPK-8011 and SPK-8016 for hemophilia A. It is currently in Phase III stage of development and is being developed by Spark Therapeutics.

### BIVV001: Sanofi Genzyme

BIVV001 (efanesoctocog alfa) is a recombinant coagulation factor VIII Fc – von

Willebrand Factor – XTEN Fusion protein which is being developed by Sanofi Genzyme for Haemophilia A. It is currently in Phase III stage of development.

Further product details are provided in the report.....

## Hemophilia A: Therapeutic Assessment

This segment of the report provides insights about the different Hemophilia A drugs segregated based on following parameters that define the scope of the report, such as:

### Major Players working on Hemophilia A

There are approx. 30+ key companies which are developing the Hemophilia A. The companies which have their Hemophilia A drug candidates in the most advanced stage, i.e. Phase III include, Spark Therapeutics.

### Phases

DelveInsight's report covers around 40+ products under different phases of clinical development like

Late-stage products (Phase III)

Mid-stage products (Phase II)

Early-stage products (Phase I/II and Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Hemophilia A pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Infusion

Intradermal

Intramuscular

Intranasal

Intravaginal

Oral

Parenteral

Subcutaneous

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Vaccines

Monoclonal Antibody

Peptides

Polymer

Small molecule

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

## Hemophilia A: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, I, preclinical and discovery stage. It also analyses Hemophilia A therapeutic drugs key players involved in developing key drugs.

### Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Hemophilia A drugs.

### Hemophilia A Report Insights

- Hemophilia A Pipeline Analysis

- Therapeutic Assessment

- Unmet Needs

- Impact of Drugs

### Hemophilia A Report Assessment

- Pipeline Product Profiles

- Therapeutic Assessment

- Pipeline Assessment

- Inactive drugs assessment

- Unmet Needs

### Key Questions

Current Scenario and Emerging Therapies:

How many companies are developing Hemophilia A drugs?

How many Hemophilia A drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for Hemophilia A?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Hemophilia A therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Hemophilia A and their status?

What are the key designations that have been granted to the emerging drugs?

## Key Players

Spark Therapeutics

Sigilon Therapeutics

ASC Therapeutics

Pfizer

Sanofi Genzyme

Novo Nordisk

## Key Products

SPK-8011

SPK-8016

SIG-001

ASC618

ASC518

PF-07055480

Fitusiran

BIVV001

Mim 8

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- Preclinical Stage Products
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ASC518: ASC Therapeutics

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Inactive Products

Comparative Analysis

Hemophilia A Key Companies

Hemophilia A Key Products

Hemophilia A- Unmet Needs

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Hemophilia A- Future Perspectives and Conclusion

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