

Hemophilia B - Pipeline Review, H1 2018

<https://marketpublishers.com/r/H3BC88AA32AEN.html>

Date: March 2018

Pages: 126

Price: US\$ 2,000.00 (Single User License)

ID: H3BC88AA32AEN

Abstracts

Hemophilia B - Pipeline Review, H1 2018

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Hemophilia B - Pipeline Review, H1 2018, provides an overview of the Hemophilia B (Hematological Disorders) pipeline landscape.

Hemophilia B is a hereditary bleeding disorder caused by a lack of blood clotting factor IX. Symptoms include bleeding into joints and associated pain and swelling, bruising, excessive bleeding following circumcision, gastrointestinal tract and urinary tract hemorrhage and nosebleeds. Treatment includes replacing the defective clotting factor.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Hemophilia B - Pipeline Review, H1 2018, provides comprehensive information on the therapeutics under development for Hemophilia B (Hematological Disorders), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development history and latest news and press releases.

The Hemophilia B (Hematological Disorders) pipeline guide also reviews of key players involved in therapeutic development for Hemophilia B and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Pre-Registration, Phase III, Phase II, Phase I, Preclinical, Discovery and Unknown stages

are 1, 3, 10, 2, 11, 1 and 1 respectively. Similarly, the Universities portfolio in Phase II, Preclinical and Discovery stages comprises 1, 4 and 1 molecules, respectively.

Hemophilia B (Hematological Disorders) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor presentations and featured press releases from company/university sites and industry-specific third party sources. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

Note: Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Hemophilia B (Hematological Disorders).

The pipeline guide reviews pipeline therapeutics for Hemophilia B (Hematological Disorders) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Hemophilia B (Hematological Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Hemophilia B (Hematological Disorders) therapeutics based on mechanism of action (MoA), drug target, route of

administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Hemophilia B (Hematological Disorders)

REASONS TO BUY

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Hemophilia B (Hematological Disorders).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it's most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Hemophilia B (Hematological Disorders) pipeline depth and focus of Indication therapeutics.

Develop and design in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and @Scope.

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

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- GC Pharma
- Genethon SA
- LFB SA
- Logicbio Therapeutics Inc
- MolMed SpA
- Novo Nordisk AS
- OPKO Biologics Ltd
- Pfizer Inc
- Pharming Group NV
- Promethera Biosciences SA
- RegenxBio Inc
- Sangamo Therapeutics Inc
- Shire Plc
- Spark Therapeutics Inc

UniQure NV

Hemophilia B - Drug Profiles

AMT-060 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

AMT-061 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

BAX-335 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

BAY-1093884 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

BIVV-002 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

CB-2679d - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Cell Therapy to Activate F9 for Hemophilia B - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

coagulation factor IX (human) - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

concizumab - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

CSL-689 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

fidanacogene elaparvovec - Drug Profile

Product Description

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R&D Progress

fitusiran - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy for Hematological Disorders - Drug Profile

Product Description

Mechanism Of Action

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R&D Progress

Gene Therapy to Activate Coagulation Factor IX for Hemophilia B - Drug Profile

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Gene Therapy to Activate Factor IX for Hemophilia B - Drug Profile

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HepaStem - Drug Profile

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R&D Progress

LR-769 - Drug Profile

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R&D Progress

marstacimab - Drug Profile

Product Description

Mechanism Of Action

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marzeptacog alfa - Drug Profile

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MG-1113A - Drug Profile

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OPK-88005 - Drug Profile

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Mechanism Of Action

R&D Progress

SB-FIX - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SHP-648 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Stem Cell Therapy to Activate Coagulation Factor IX for Hemophilia B - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SVF-VIIa - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Hemophilia B - Dormant Projects

Hemophilia B - Discontinued Products

Hemophilia B - Product Development Milestones

Featured News & Press Releases

Feb 28, 2018: Sangamo Announces U.K. Authorization Of Clinical Trial Evaluating Zinc Finger Nuclease In Vivo Genome Editing Treatment For Hemophilia B

Feb 05, 2018: Spark Therapeutics to Present Data on its Hemophilia B Drug Candidate SPK-8011 in EAHAD Conference, Spain

Dec 15, 2017: FDA lifts clinical hold on fitusiran

Dec 11, 2017: New Data Show Extended Prophylactic Dosing with ALPROLIX Provides Safe and Effective Protection in People with Severe Hemophilia B

Dec 11, 2017: Spark Therapeutics and Pfizer Announce Longer-term Preliminary Data Showing Consistent and Sustained Factor IX Levels in Hemophilia B at 59th American

Society of Hematology (ASH) Annual Meeting and Exposition

Dec 09, 2017: ALPROLIX Demonstrates Higher Tissue Distribution and Retention in Joints Compared to Other Factor IX Molecules in a Preclinical Imaging Study

Dec 06, 2017: One-dose gene therapy produces clotting factor, safely stops bleeding in hemophilia B patients

Dec 06, 2017: Spark Therapeutics and Pfizer Announce Publication in The New England Journal of Medicine of Interim Data from Phase 1/2 Clinical Trial of Investigational Gene Therapy for Hemophilia B

Dec 05, 2017: Spark Therapeutics Announces Presentation on Updated Hemophilia B Clinical Trial at 59th American Society of Hematology Annual Meeting and Exposition

Nov 27, 2017: U.S. Label Update for Bioverativs ALPROLIX Adds Data Reinforcing Efficacy and Safety of Long-Term Prophylactic Treatment in Adults, Adolescents and Children With Hemophilia B

Nov 09, 2017: Alnylam Announces Successful Outcome Following FDA Type A Meeting to Discuss Fitusiran Program in Hemophilia

Nov 01, 2017: Bioverativ to Present New Data on ALPROLIX at ASH 2017 Annual Meeting

Oct 25, 2017: Alprolix approved in the Kingdom of Saudi Arabia for the treatment of hemophilia B

Sep 07, 2017: Alnylam Provides Pipeline Update on Fitusiran Investigational RNAi Therapeutic Programs

Sep 07, 2017: Sanofi Alliance Partner Alnylam Provides Update on Fitusiran Development Program

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COMPANIES MENTIONED

Alnylam Pharmaceuticals Inc
Amarna Therapeutics BV
Bayer AG
Bioverativ Inc
Catalyst Biosciences Inc
China Biologic Products Inc
CSL Ltd
Expression Therapeutics LLC
GC Pharma
Genethon SA
LFB SA
Logicbio Therapeutics Inc
MolMed SpA
Novo Nordisk AS
OPKO Biologics Ltd
Pfizer Inc
Pharming Group NV
Promethera Biosciences SA
RegenxBio Inc
Sangamo Therapeutics Inc
Shire Plc

Spark Therapeutics Inc
UniQure NV

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