

# Homozygous Familial Hypercholesterolemia (HoFH) -Pipeline Review, H1 2018

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### **Abstracts**

Homozygous Familial Hypercholesterolemia (HoFH) - Pipeline Review, H1 2018

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Homozygous Familial Hypercholesterolemia (HoFH) - Pipeline Review, H1 2018, provides an overview of the Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic Disorders) pipeline landscape.

Homozygous familial hypercholesterolemia is a genetic disorder which is caused due to mutation from both parents. HoFH causes LDL cholesterol level to be very high. Symptoms include xanthelasmas, chest pain and sores on the toes that do not heal. Treatment includes diet changes and statin drugs.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Homozygous Familial Hypercholesterolemia (HoFH) - Pipeline Review, H1 2018, provides comprehensive information on the therapeutics under development for Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic 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 Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic Disorders) pipeline



guide also reviews of key players involved in therapeutic development for Homozygous Familial Hypercholesterolemia (HoFH) and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Phase III, Phase II, Phase I, Preclinical and Unknown stages are 3, 5, 1, 2 and 1 respectively.

Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic 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 Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic Disorders).

The pipeline guide reviews pipeline therapeutics for Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic 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 Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic Disorders) therapeutics and enlists all their major and minor projects.



The pipeline guide evaluates Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic 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 Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic 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 Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic 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 Homozygous Familial Hypercholesterolemia (HoFH) (Metabolic 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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Apr 30, 2018: The Medicines Company Presents New Data at the National Lipid Association Scientific Sessions

Mar 08, 2018: The Medicines Company Reaches Enrollment Target for ORION-10 Ahead of Schedule

Jan 25, 2018: Inclisiran's First Pivotal Trial Achieves Target Enrollment Ahead of Schedule

Nov 13, 2017: Akcea Initiates AKCEA-ANGPTL3-LRx Phase 2 Program in Patients with Rare Hyperlipidemias

Sep 25, 2017: Gemphire to Present New COBALT-1 Clinical Data at the 2017 FH Global Summit

Jun 28, 2017: Gemphire Announces Top-Line Data from COBALT-1 Phase 2b Clinical Trial in HoFH Patients

May 24, 2017: Regeneron Announces ANGPTL3/Evinacumab Publication in New England Journal of Medicine and Positive Phase 2 Data in People with HoFH

Apr 06, 2017: Regeneron Announces Evinacumab has Received FDA Breakthrough Therapy Designation for Homozygous Familial Hypercholesterolemia

Mar 14, 2017: The Medicines Company to Webcast Presentation of ORION-1 Phase II Study of Inclisiran at ACC.17

Jan 30, 2017: Gemphire Announces Interim LDL-C Lowering Data from COBALT-1 Phase 2b Clinical Trial

Jan 05, 2017: Gemphire Therapeutics Provides Clinical Update

Dec 19, 2016: Capstone Therapeutics Announces Profound, Rapid LDL Cholesterol Reduction in AEM-28-14 Primate Study

Sep 26, 2016: Gemphire Therapeutics Enrolls First Patient in the COBALT-1 Trial Investigating Gemcabene in Homozygous Familial Hypercholesterolemia

May 31, 2016: Regeneron Presents Positive Interim Data from Phase 2 Proof-of-

Concept Study of Evinacumab in Patients with Homozygous Familial

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Mar 17, 2016: CymaBay Therapeutics Announces Positive Results from its Pilot Phase 2 Clinical Study of MBX-8025 in Patients with Homozygous Familial

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Akcea Therapeutics Inc CymaBay Therapeutics Inc Daewoong Co Ltd Gemphire Therapeutics Inc LipimetiX Development Inc Madrigal Pharmaceuticals Inc. Regeneron Pharmaceuticals Inc RegenxBio Inc The Medicines Company



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