

Mucopolysaccharidosis III - Pipeline Review, H1 2017

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Abstracts

Mucopolysaccharidosis III - Pipeline Review, H1 2017

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Mucopolysaccharidosis III - Pipeline Review, H1 2017, provides an overview of the Mucopolysaccharidosis III (Metabolic Disorders) pipeline landscape.

Mucopolysaccharidosis type III (MPS III), also known as Sanfilippo syndrome, is an inherited metabolic disease caused by an absence or malfunctioning of certain enzymes needed to breakdown molecules called glycosaminoglycans (GAG). Symptoms include seizures, hyperactivity, liver and spleen enlargement, severe diarrhea or constipation and enlargement of tonsils and adenoids. Treatment includes enzyme replacement therapy (ERT).

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Mucopolysaccharidosis III - Pipeline Review, H1 2017, provides comprehensive information on the therapeutics under development for Mucopolysaccharidosis III (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 Mucopolysaccharidosis III (Metabolic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) and features dormant and discontinued projects. The guide

covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Phase II, IND/CTA Filed, Preclinical and Discovery stages are 6, 1, 6 and 3 respectively. Similarly, the Universities portfolio in Preclinical stages comprises 1 molecules, respectively.

Mucopolysaccharidosis III (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 Mucopolysaccharidosis III (Metabolic Disorders).

The pipeline guide reviews pipeline therapeutics for Mucopolysaccharidosis III (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 Mucopolysaccharidosis III (Metabolic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Mucopolysaccharidosis III (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 Mucopolysaccharidosis III (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 Mucopolysaccharidosis III (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 Mucopolysaccharidosis III (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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ArmaGen Inc

Axcentua Pharmaceuticals AB

BioMarin Pharmaceutical Inc

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Lysogene SAS

Swedish Orphan Biovitrum AB

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Jan 19, 2017: Abeona Therapeutics Receives Orphan Drug Designation in The European Union for ABO-101 Gene Therapy in Sanfilippo Syndrome Type B

Oct 19, 2016: European Commission Grants SOBI003 Orphan Designation for the Treatment of MPS IIIA

May 24, 2016: Abeona Therapeutics Announces FDA Allowance of Investigational New Drug for Phase 1/2 Clinical Study With ABO-101 Gene Therapy for Patients With Sanfilippo Syndrome Type B (MPS IIIB)

Feb 25, 2016: ESTEVE Provides Update On EGT-201 For Sanfilippo B Syndrome

Jan 11, 2016: Abeona Therapeutics Announces Initial European Regulatory Approval for Phase 1/2 Gene Therapy Clinical Study for Patients With Sanfilippo Syndrome

May 20, 2015: PlasmaTech Biopharmaceuticals Announces Orphan Drug and Rare Pediatric Disease Designations For ABX-101 From FDA

Apr 02, 2014: Orphan designation granted for MPSIII

Jul 25, 2013: New stem cell gene therapy gives hope to prevent inherited neurological disease

Apr 11, 2013: Axcentua granted patent on ten solid forms of genistein

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Abeona Therapeutics Inc

Alexion Pharmaceuticals Inc

ArmaGen Inc

Axcentua Pharmaceuticals AB

BioMarin Pharmaceutical Inc

Laboratorios Del Dr Esteve SA

Lysogene SAS

Swedish Orphan Biovitrum AB

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