

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

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Abstracts

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) - Drugs In Development, 2022, provides an overview of the Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) (Genetic 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 (MPS III) (Sanfilippo Syndrome) - Drugs In Development, 2022, provides comprehensive information on the therapeutics under development for Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) (Genetic 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 (MPS III) (Sanfilippo Syndrome) (Genetic 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 III, Phase II, Phase I, Preclinical and Discovery stages are 3, 3, 1, 18 and 4 respectively. Similarly, the Universities portfolio in Preclinical stages comprises 1 molecules, respectively.

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) (Genetic 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 (MPS III) (Sanfilippo Syndrome) (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) (Genetic 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 (MPS III) (Sanfilippo Syndrome) (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) (Genetic 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 (MPS III) (Sanfilippo Syndrome) (Genetic 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 (MPS III) (Sanfilippo Syndrome) (Genetic 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 (MPS III) (Sanfilippo Syndrome) (Genetic 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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Amicus Therapeutics Inc

ArmaGen Inc

BioStrategies LC

Denali Therapeutics Inc

Esteve Pharmaceuticals SA

GC Biopharma Corp

JCR Pharmaceuticals Co Ltd

Jupiter Neurosciences Inc

Lacerta Therapeutics Inc

M6P Therapeutics

Neurogt Inc

Orchard Therapeutics Plc

Phoenix Nest Inc

Seelos Therapeutics, Inc.

Swedish Orphan Biovitrum AB

Tega Therapeutics Inc

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Milestones

Featured News & Press Releases

Feb 10, 2022: JCR pharmaceuticals to present posters on JR-441 at the 18th annual WORLDSymposium 2022

Feb 10, 2022: M6P Therapeutics presents promising preclinical data in lysosomal storage disorders at the 18th Annual WORLDSymposium 2022

Jan 21, 2022: EC grants orphan drug designation to JR-441 for the treatment of mucopolysaccharidosis type III A (MPS IIIA)

Jul 28, 2021: M6P Therapeutics presented data on M041, a recombinant enzyme therapy, for the treatment of Sanfilippo B syndrome at MPS 2021

Dec 21, 2020: Seelos Therapeutics announces issuance of a patent for Trehalose (SLS-005) in Israel

Nov 09, 2020: Seelos Therapeutics to participate in the 3rd International Conference on Sanfilippo Syndrome and related diseases

Aug 25, 2020: Seelos Therapeutics receives European Orphan Drug Designation for SLS-005 (Trehalose) in Sanfilippo syndrome

Jul 21, 2020: Seelos Therapeutics receives positive EMA opinion on orphan drug designation for SLS-005 (Trehalose) in Sanfilippo syndrome

May 15, 2020: Seelos Therapeutics receives rare pediatric disease designation for SLS-005 (Trehalose) in Sanfilippo Syndrome

Apr 30, 2020: Seelos Therapeutics receives Orphan Drug Designation for SLS-005 (Trehalose) in Sanfilippo Syndrome

Apr 21, 2020: JCR to initiate development of new drug candidate JR-446 for Sanfilippo

Syndrome Type B using J-Brain Cargo

Mar 11, 2020: Seelos Therapeutics announces European Medicines Agency guidance to design an open-label pivotal study for SLS-005 in Sanfilippo Syndrome

Jan 22, 2020: Seelos Therapeutics announces updates to SLS-005 (trehalose) programs for Europe and U.S.

Dec 13, 2019: Seelos Therapeutics to participate in 33rd annual National MPS Society Family Conference

Dec 04, 2019: Seelos Therapeutics receives notice of allowance for an additional US patent for Trehalose (SLS-005)

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