

# Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline Review, H1 2020

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## Abstracts

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline Review, H1 2020

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Mucopolysaccharidosis II (MPS II) (Hunter Syndrome ) - Pipeline Review, H1 2020, provides an overview of the Mucopolysaccharidosis II (MPS II) (Hunter 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 II (MPS II) (Hunter Syndrome ) - Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Mucopolysaccharidosis II (MPS II) (Hunter 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 II (MPS II) (Hunter 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, Discovery and Unknown stages are 2, 6, 1, 9, 4 and 1 respectively.

Mucopolysaccharidosis II (MPS II) (Hunter 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 II (MPS II) (Hunter Syndrome ) (Genetic Disorders).

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

The pipeline guide evaluates Mucopolysaccharidosis II (MPS II) (Hunter 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 II (MPS II) (Hunter 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 II (MPS II) (Hunter 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 II (MPS II) (Hunter 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.

## Contents

Introduction

Global Markets Direct Report Coverage

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Overview

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Therapeutics Development

Pipeline Overview

Pipeline by Companies

Products under Development by Companies

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Therapeutics Assessment

Assessment by Target

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Companies Involved in Therapeutics Development

Abeona Therapeutics Inc

Allievex Corp

Amicus Therapeutics Inc

ArmaGen Inc

Denali Therapeutics Inc

Esteve Pharmaceuticals SA

JCR Pharmaceuticals Co Ltd

Jupiter Orphan Therapeutics Inc

Lacerta Therapeutics Inc

Lysogene SAS

Orchard Therapeutics Plc

Phoenix Nest Inc

Seelos Therapeutics, Inc.

Swedish Orphan Biovitrum AB

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Drug Profiles

ABO-101 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

ABO-102 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

AGT-184 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

AGT-187 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

anakinra - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

EGT-101 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

EGT-201 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy for Mucopolysaccharidosis III (Sanfilippo Syndrome) - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate HGSNAT for Mucopolysaccharidosis Type IIIC - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate NAGLU for Mucopolysaccharidosis Type IIIB - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate NAGLU for Mucopolysaccharidosis Type IIIB - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate SGSH for Mucopolysaccharidosis Type IIIA - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

JOT-109 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

JR-441 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

JR-446 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

LYSSAF-302 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

OTL-201 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

OTL-202 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Proteins for Mucopolysaccharidosis Type IIIC - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant N-Acetylgalactosamine-6-Sulfatase Replacement for  
Mucopolysaccharidosis Type IIID - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant Sulphamidase Replacement for Mucopolysaccharidosis III (Sanfilippo  
Syndrome) - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

tralesinidase alfa - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

trehalose - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Dormant Projects

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Discontinued Products

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Product Development  
Milestones

Featured News & Press Releases

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)

Aug 22, 2019: Seelos Therapeutics announces FDA acceptance of IND application for SLS-005 for mucopolysaccharidosis type III (sanfilippo syndrome)

Jul 17, 2019: Seelos Therapeutics announces Investigational New Drug application submission for SLS-005

Jul 15, 2019: Seelos Therapeutics announces receipt of a grant for funding of SLS-005 Program

Jun 06, 2019: Seelos Therapeutics provides update on its pipeline product SLS-005

Feb 14, 2019: Bioblast Pharma announces collaboration with Team Sanfilippo to evaluate trehalose for the treatment of Sanfilippo syndrome

Feb 04, 2019: BioMarin announces presentation of data on drug candidate BMN 250 at 15th Annual WORLDSymposium 2019

Nov 07, 2018: Phoenix Nest Los Angeles Biomedical Research Institute at Harbor-



UCLA Medical Center (LA BioMed) and Washington University in St. Louis announce a New NIH Grant for Sanfilippo Syndrome (MPSIIID)

Sep 14, 2018: JCR to initiate development of a new drug candidate for sanfilippo syndrome Type A using J-Brain Cargo

[Appendix](#)

[Methodology](#)

[Coverage](#)

[Secondary Research](#)

[Primary Research](#)

[Expert Panel Validation](#)

[Contact Us](#)

[Disclaimer](#)

## List Of Tables

### LIST OF TABLES

Number of Products under Development for Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ), H1 2020

Number of Products under Development by Companies, H1 2020

Products under Development by Companies, H1 2020

Products under Development by Companies, H1 2020 (Contd..1), H1 2020

Number of Products by Stage and Target, H1 2020

Number of Products by Stage and Mechanism of Action, H1 2020

Number of Products by Stage and Route of Administration, H1 2020

Number of Products by Stage and Molecule Type, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Abeona Therapeutics Inc, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Allievex Corp, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Amicus Therapeutics Inc, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by ArmaGen Inc, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Denali Therapeutics Inc, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Esteve Pharmaceuticals SA, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by JCR Pharmaceuticals Co Ltd, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Jupiter Orphan Therapeutics Inc, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Lacerta Therapeutics Inc, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Lysogene SAS, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Orchard Therapeutics Plc, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Phoenix Nest Inc, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Seelos Therapeutics, Inc., H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Pipeline by Swedish Orphan Biovitrum AB, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Dormant Projects, H1 2020

Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ) - Discontinued Products, H1 2020

## List Of Figures

### LIST OF FIGURES

Number of Products under Development for Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ), H1 2020

Number of Products under Development by Companies, H1 2020

Number of Products by Top 10 Targets, H1 2020

Number of Products by Stage and Top 10 Targets, H1 2020

Number of Products by Top 10 Mechanism of Actions, H1 2020

Number of Products by Stage and Top 10 Mechanism of Actions, H1 2020

Number of Products by Top 10 Routes of Administration, H1 2020

Number of Products by Stage and Top 10 Routes of Administration, H1 2020

Number of Products by Top 10 Molecule Types, H1 2020

Number of Products by Stage and Top 10 Molecule Types, H1 2020

### COMPANIES MENTIONED

Abeona Therapeutics Inc

Allievex Corp

Amicus Therapeutics Inc

ArmaGen Inc

Denali Therapeutics Inc

Esteve Pharmaceuticals SA

JCR Pharmaceuticals Co Ltd

Jupiter Orphan Therapeutics Inc

Lacerta Therapeutics Inc

Lysogene SAS

Orchard Therapeutics Plc

Phoenix Nest Inc

Seelos Therapeutics, Inc.

Swedish Orphan Biovitrum AB

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