

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

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Abstracts

Mucopolysaccharidosis I (MPS I) (Hurler 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 I (MPS I) (Hurler Syndrome) - Drugs In Development, 2022, provides an overview of the Mucopolysaccharidosis I (MPS I) (Hurler Syndrome) (Genetic Disorders) pipeline landscape.

MPS I (Mucopolysaccharidosis I) is an inherited lysosomal storage disorder caused by a deficiency of alpha-L-iduronidase, a lysosomal enzyme normally required for the breakdown of certain complex carbohydrates known as glycosaminoglycans (GAGs). Symptoms include abnormal bones in the spine, claw hand, cloudy corneas, deafness and heart valve problems. Treatment includes bone marrow transplantation, enzyme therapy and gene therapy.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Mucopolysaccharidosis I (MPS I) (Hurler Syndrome) - Drugs In Development, 2022, provides comprehensive information on the therapeutics under development for Mucopolysaccharidosis I (MPS I) (Hurler 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 I (MPS I) (Hurler Syndrome) (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Mucopolysaccharidosis I (MPS I) (Hurler 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, IND/CTA Filed, Preclinical, Discovery and Unknown stages are 1, 5, 2, 2, 6, 3 and 1 respectively. Similarly, the Universities portfolio in Discovery stages comprises 2 molecules, respectively.

Mucopolysaccharidosis I (MPS I) (Hurler 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 I (MPS I) (Hurler Syndrome) (Genetic Disorders).

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

The pipeline guide evaluates Mucopolysaccharidosis I (MPS I) (Hurler 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 I (MPS I) (Hurler 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 I (MPS I) (Hurler 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 I (MPS I) (Hurler 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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BioStrategies LC

Chengdu Genevector Biotechnology Co Ltd

Cinnagen Co

Denali Therapeutics Inc

EdiGene Inc

Eloxx Pharmaceuticals Inc

Gain Therapeutics Inc

Immusoft Corp

JCR Pharmaceuticals Co Ltd

Jupiter Neurosciences Inc

Mina Therapeutics Ltd

Orchard Therapeutics Plc

Ossianix Inc

Paradigm Biopharmaceuticals Ltd

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ReqMed Co Ltd

Sigilon Therapeutics Inc

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Featured News & Press Releases

Sep 26, 2022: Paradigm Reports Successful Safety Review in MPS VI Phase 2 Clinical
Trial and update on timing of PARA_008 top-line data readout.

Sep 01, 2022: Immusoft announces FDA clearance of IND application for ISP-001 for
MPS I, the first engineered B cell therapy to enter into clinical trials

Aug 08, 2022: MPS clinical program update and paradigm to present at the International

Conference on lysosomal diseases

Feb 11, 2022: International researchers highlight the need for PPS as a new adjunct therapy treatment for unmet needs of patients with MPS VI

Feb 10, 2022: JCR pharmaceuticals to present posters on JR-171 at the 18th annual WORLDSymposium 2022

Feb 07, 2022: Immusoft to present at WORLDSymposium 2022 Conference

Jan 31, 2022: Sigilon Therapeutics to present preclinical data at the 18th Annual WORLDSymposium on Lysosomal Diseases, MPS-1 and MPS-6

Nov 23, 2021: Positive interim data from phase 2 rare disease trial presented at international medical congress

Oct 05, 2021: JCR Pharmaceuticals: US FDA grants Fast Track Designation for JR-171 for the treatment of Mucopolysaccharidosis Type I (MPSI)

Sep 09, 2021: Sigilon Therapeutics announces acceptance of clinical trial application in the UK for SIG-005 for the treatment of mucopolysaccharidosis type I

Jul 23, 2021: Sigilon Therapeutics presents preclinical data at the 16th International Symposium on MPS and Related Diseases

Mar 30, 2021: EMA grants Orphan Drug Designation to JR-171 for the treatment of Mucopolysaccharidosis Type I (MPS I)

Feb 12, 2021: US FDA grants orphan drug designation to JR-171 for the treatment of Mucopolysaccharidosis Type I (MPS I)

Dec 17, 2020: Sigilon Therapeutics receives Orphan Drug Designation for SIG-005 for the treatment of mucopolysaccharidosis type I

Nov 12, 2020: First patient dosed in Phase 2 clinical trial evaluating PPS in Mucopolysaccharidoses Type-1 (mps-i)

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