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Mucopolysaccharidosis I - Pipeline Review, H1 2017

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Abstracts

Mucopolysaccharidosis I - Pipeline Review, H1 2017

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Mucopolysaccharidosis I - Pipeline Review, H1 2017, provides an overview of the Mucopolysaccharidosis I (Metabolic 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 - Pipeline Review, H1 2017, provides comprehensive information on the therapeutics under development for Mucopolysaccharidosis I (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 I (Metabolic 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

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therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Phase II, Phase I, Preclinical and Discovery stages are 5, 1, 6 and 3 respectively. Similarly, the Universities portfolio in Preclinical and Discovery stages comprises 2 and 1 molecules, respectively.

Mucopolysaccharidosis I (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 I (Metabolic Disorders).

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

The pipeline guide evaluates Mucopolysaccharidosis I (Metabolic Disorders) therapeutics based on mechanism of action (MoA), drug target, route of

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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 (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 I (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 I (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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Feb 07, 2017: ArmaGen Announces Oral Presentation of Preliminary Results from its Phase 2 Clinical Trial of AGT-181 in Patients with MPS 1 to be Presented at WORLDSymposium 2017
Nov 07, 2016: Eloxx Pharmaceuticals Announces Orphan Drug Designation in the U.S. and Europe for ELX-02 in Mucopolysacchardisis Type 1 (MPS 1)
Sep 13, 2016: REGENXBIO Publishes Data from Ongoing Preclinical Studies of NAV Gene Therapy RGX-111

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Jul 05, 2016: REGENXBIO Provides Update On Gene Therapy Development Program RGX-111
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Bioasis Technologies Inc
CRISPR Therapeutics
Eloxx Pharmaceuticals Ltd
Immusoft Corp
Inventiva
OPKO Health Inc
PTC Therapeutics Inc
RegenxBio Inc
Sangamo Therapeutics Inc

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