

Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) - Pipeline Review, H1 2020

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Abstracts

Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) - Pipeline Review, H1 2020

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Mucopolysaccharidosis II - Pipeline Review, H1 2020, provides an overview of the Mucopolysaccharidosis II (Metabolic Disorders) pipeline landscape. Mucopolysaccharidosis type II (MPS II), also known as Hunter syndrome, is a condition that affects many different parts of the body and occurs almost exclusively in males. Signs and symptoms include claw-like hands, protruding tongue, changing facial features, including thickening of the lips, tongue and nostrils and delayed development. Treatment includes bone marrow transplantation, enzyme therapy and gene therapy.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Mucopolysaccharidosis II - Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Mucopolysaccharidosis II (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 II (Metabolic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules



developed by Companies in Pre-Registration, Phase III, Phase II, IND/CTA Filed, Preclinical and Discovery stages are 1, 2, 3, 1, 2 and 1 respectively. Similarly, the Universities portfolio in Phase II and Phase I stages comprises 1 and 1 molecules, respectively.

Mucopolysaccharidosis II (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 II (Metabolic Disorders).

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

The pipeline guide evaluates Mucopolysaccharidosis II (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 II (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 II (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 II (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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Bioasis Technologies Inc

Denali Therapeutics Inc

Esteve Pharmaceuticals SA

GC Pharma

Immusoft Corp

JCR Pharmaceuticals Co Ltd

RegenxBio Inc

ReqMed Co Ltd

Sangamo Therapeutics Inc

Takeda Pharmaceutical Co Ltd

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Mechanism Of Action

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Featured News & Press Releases

May 27, 2020: Denali Therapeutics announces publication of two new papers describing its blood-brain barrier delivery technology in science translational medicine

Apr 28, 2020: REGENXBIO announces presentations on RGX-121 at the American Society of Gene and Cell Therapy's 23rd Annual Meeting

Apr 01, 2020: Clinigen and GC Pharma submit new drug application for Hunterase (Idursulfase-beta) ICV in Japan

Jan 29, 2020: REGENXBIO announces presentations at the 16th Annual WORLDSymposium 2020

Dec 18, 2019: REGENXBIO announces interim data from phase I/II trial of RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II)

Sep 09, 2019: CANbridge Pharmaceuticals' Hunterase Granted Priority Review by the Chinese National Medical Products Administration

Jul 29, 2019: CANbridge Pharmaceuticals submits New Drug Application for Hunterase for the treatment of Hunter Syndrome in China

Jun 11, 2019: Denali Therapeutics receives orphan drug and rare pediatric disease designation for DNL310, and expands its portfolio of brain penetrant enzyme replacement programs

Feb 28, 2019: JCR completes patient enrollment in phase 2 clinical trial of JR-141 for Hunter Syndrome in Brazil

Feb 28, 2019: JCR receives EMA Orphan Designation for JR-141 in Hunter Syndrome Dec 28, 2018: JCR completed enrollment in phase 3 clinical trial of JR-141 in Japan for Hunter syndrome

Dec 21, 2018: Notice on the publication of the phase 1/2 clinical trial results for hunter syndrome in molecular therapy

Oct 19, 2018: JCR Pharmaceuticals: Notice of Orphan Drug Designation by the US Food Drug Administration for JR-141 for Hunter Syndrome

Aug 09, 2018: JCR initiates phase 3 clinical trial of JR-141 for Hunter Syndrome Jun 29, 2018: JCR Initiates Phase 2 Clinical Trial of JR-141 for Hunter Syndrome in Brazil

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COMPANIES MENTIONED

Bioasis Technologies Inc
Denali Therapeutics Inc
Esteve Pharmaceuticals SA
GC Pharma
Immusoft Corp
JCR Pharmaceuticals Co Ltd
RegenxBio Inc
ReqMed Co Ltd
Sangamo Therapeutics Inc



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