

Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) - Drugs in Development, 2021

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Abstracts

Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) - Drugs in Development, 2021

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Mucopolysaccharidosis II - Drugs In Development, 2021, 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 - Drugs In Development, 2021, 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, Phase I, Preclinical and Discovery stages are 1, 1, 4, 2, 5 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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AngioChem Inc

AVROBIO Inc

Bioasis Technologies Inc

Denali Therapeutics Inc

Esteve Pharmaceuticals SA

GC Pharma

Generium

Homology Medicines Inc

Immusoft Corp

JCR Pharmaceuticals Co Ltd

RegenxBio Inc

ReqMed Co Ltd

Sigilon Therapeutics Inc

Takeda Pharmaceutical Co Ltd

Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) - Drug Profiles

AVRRD-05 - Drug Profile

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Mechanism Of Action

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Product Description

Mechanism Of Action

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DUOC-01 - Drug Profile

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Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) - Product Development

Milestones

Featured News & Press Releases

Nov 03, 2021: AVROBIO receives Rare Pediatric Disease Designation from the U.S. FDA for AVR-RD-05, a Gene Therapy for Mucopolysaccharidosis Type II (MPSII) or Hunter Syndrome

Nov 02, 2021: GC Green Cross designated as a European orphan drug for the treatment of severe Hunter syndrome

Oct 20, 2021: Homology Medicines announces presentation of data supporting clinical programs in MPS II and PKU, including nonclinical and patient-focused research, at American Society of Human Genetics Meeting

Oct 18, 2021: Homology medicines initiates clinical trial for HMI-203, a one-time investigational gene therapy candidate for adults with MPS II (Hunter Syndrome)

Oct 18, 2021: JCR Pharmaceuticals Co : EMA grants PRIME designation for JR-141 for the treatment of Mucopolysaccharidosis type II (Hunter Syndrome)

Oct 15, 2021: JR-141 (Pabinafusp Alfa) for Hunter syndrome notice on the publication

of a nonclinical and clinical evidence in International Journal of Molecular Sciences

Jul 29, 2021: JCR Pharmaceuticals announces presentation at 16th International Symposium on MPS and Related Diseases.

Jul 29, 2021: JR-141 (Pabinafusp Alfa) for Hunter Syndrome notice on the publication of a case report in Japan in JIMD reports

May 19, 2021: JCR Pharmaceuticals: Launch of IZCARGO I.V. infusion 10mg (JR-141: Pabinafusp Alfa) for treatment of MPS II (Hunter Syndrome) in Japan

Apr 19, 2021: JR-141 (Pabinafusp Alfa) for Hunter Syndrome notice on the publication of the results of non-clinical trials in molecular genetics and metabolism report

Mar 23, 2021: JCR Pharmaceuticals announces approval of IZCARGO (pabinafusp alfa) for treatment of MPS II (Hunter syndrome) in Japan

Mar 03, 2021: GC Green Cross to ship 'Hunterase ICV' in Japan

Feb 10, 2021: JCR Pharmaceuticals receives FDA IND clearance to initiate global phase 3 clinical trial of JR-141 for Mucopolysaccharidosis Type II (Hunter Syndrome)

Feb 10, 2021: FDA grants fast track designation for JR-141 for the treatment of mucopolysaccharidosis type II (Hunter syndrome)

Feb 08, 2021: Homology Medicines announces first presentation of data with HMI-203 in vivo gene therapy development candidate for Hunter syndrome

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