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Mucopolysaccharidosis Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034

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Abstracts

The mucopolysaccharidosis market has been comprehensively analyzed in IMARC's new report titled "Mucopolysaccharidosis Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034". Mucopolysaccharidosis (MPS) refers to a group of rare genetic disorders characterized by the accumulation of glycosaminoglycans (GAGs) within cells, leading to progressive damage in various tissues and organs. These disorders are caused by deficiencies in enzymes responsible for breaking down GAGs, resulting in their abnormal buildup. Symptoms of MPS can vary widely depending on the specific subtype but often include skeletal abnormalities, joint stiffness, organ enlargement, and impaired connective tissue function. Additionally, individuals suffering from MPS may experience developmental delays, cognitive impairment, and distinctive facial features. Diagnosing MPS involves a combination of clinical evaluations, biochemical testing, and genetic analysis. Clinical assessments may include physical examinations, imaging studies, and tests to measure enzyme activity and GAG levels. In addition to this, the healthcare providers may perform genetic testing to confirm the specific subtype of MPS and identify the responsible genetic mutation.

The increasing prevalence of genetic variations, which cause deficiencies in enzymes responsible for breaking down and recycling GAGs, is primarily driving the mucopolysaccharidosis market. In addition to this, the inflating utilization of effective therapeutic interventions, including substrate reduction therapies and hematopoietic stem cell transplantation to manage the ailment is also creating a positive outlook for the market. Moreover, the widespread adoption of supportive care measures, such as physical and occupational therapies, is further bolstering the market growth. These therapies aid in addressing musculoskeletal issues, enhancing mobility, and improving

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overall functional abilities. Apart from this, the rising usage of novel treatment modalities like chaperone therapy, that is designed to stabilize and enhance the activity of mutated enzymes, is acting as another significant growth-inducing factor. Additionally, the escalating application of enzyme replacement therapies, which involve intravenous infusions of the missing or deficient enzyme to alleviate symptoms of the condition and improve the quality of life for individuals suffering from MPS, is also augmenting the market growth. Furthermore, the emerging popularity of gene therapy, involving the introduction of functional genetic material to rectify or replace mutated genes responsible for the disorder, is expected to drive the mucopolysaccharidosis market during the forecast period.

IMARC Group's new report provides an exhaustive analysis of the mucopolysaccharidosis market in the United States, EU4 (Germany, Spain, Italy, and France), United Kingdom, and Japan. This includes treatment practices, in-market, and pipeline drugs, share of individual therapies, market performance across the seven major markets, market performance of key companies and their drugs, etc. The report also provides the current and future patient pool across the seven major markets. According to the report, the United States has the largest patient pool for mucopolysaccharidosis and also represents the largest market for its treatment. Furthermore, the current treatment practice/algorithm, market drivers, challenges, opportunities, reimbursement scenario, unmet medical needs, etc., have also been provided in the report. This report is a must-read for manufacturers, investors, business strategists, researchers, consultants, and all those who have any kind of stake or are planning to foray into the mucopolysaccharidosis market in any manner.

Recent Developments:
In February 2024, Denali Therapeutics Inc. released new data presentations that illustrate the broad potential of its BBB-crossing enzyme replacement medicines in development for the management of mucopolysaccharidosis (MPS). New research showed that tividenofusp alfa (DNL310) treatment for 104 weeks improved and stabilized clinical outcomes in MPS II. The single-arm, open-label Phase 1/2 study also showed a robust and sustained reduction in neurofilament light chain (NfL) and sustained normalization of CSF heparan sulfate.
In February 2024, REGENXBIO Inc. reported topline results from the Phase I/II/III CAMPSIITE trial of RGX-121 for the treatment of patients up to 5 years old with MPS II, also known as Hunter syndrome, indicating that the pivotal phase of the trial met its primary endpoint with statistical significance.
In October 2023, JCR Pharmaceuticals Co., Ltd. announced that it had dosed the first patient in Phase I/II clinical trial for mucopolysaccharidosis type IIIA (Sanfilippo

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syndrome type A) with JR-441, a blood-brain barrier (BBB) penetrating form of heparan N-sulfatase developed using JCR's proprietary J-Brain Cargo BBB-penetrating technology.
In September 2023, JCR Pharmaceuticals Co., Ltd. reported major findings from the 52-week interim data of its global phase I/II research with JR-171 (lepunafusp alfa) in patients with MPS I. JR-171 is a BBB-penetrating recombinant ?-L-iduronidase produced with JCR's patented J-Brain Cargo technology.

Key Highlights:
The prevalence of all kinds of mucopolysaccharidosis is estimated to be one per 25,000 births.
The incidence of MPS in the United States was determined to be 0.98 per 100,000 live births.
The highest birth prevalence was observed for MPS II, which accounts for 55% of all MPS.
In the United States, the birth rates for MPS IV, VI, and VII were 0.14, 0.04, and 0.027 per 100,000 live births, respectively.

Drugs:
ELAPRASE (idursulfase) is a medication that is prescribed to patients with Hunter syndrome (mucopolysaccharidosis II, MPS II). It is a pure form of the I2S enzyme developed through recombinant DNA technology in a human cell line. The medicine has been found to improve walking capacity in individuals aged five and above.

DNL310 is an experimental iduronate-2-sulfatase (IDS) fusion protein designed to cure both the cognitive and physical symptoms of MPS II with a single weekly IV infusion. The medicine is designed to penetrate the BBB via receptor-mediated transcytosis into the brain, allowing for widespread transport of IDS into cells and tissues throughout the body, to treat disease symptoms.

RGX-121 is an investigational, one-time gene treatment that uses the NAV AAV9 vector to deliver IDS, which encodes the iduronate-2-sulfatase enzyme (I2S). The RGX-121-produced protein is structurally identical to normal I2S. RGX-121 is delivered directly to the central nervous system (CNS) via intracisternal or intracerebroventricular routes.

Time Period of the Study

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Base Year: 2023
Historical Period: 2018-2023
Market Forecast: 2024-2034

Countries Covered

United States
Germany
France
United Kingdom
Italy
Spain
Japan

Analysis Covered Across Each Country

Historical, current, and future epidemiology scenario
Historical, current, and future performance of the mucopolysaccharidosis market
Historical, current, and future performance of various therapeutic categories in the market
Sales of various drugs across the mucopolysaccharidosis market
Reimbursement scenario in the market
In-market and pipeline drugs

Competitive Landscape:
This report also provides a detailed analysis of the current mucopolysaccharidosis marketed drugs and late-stage pipeline drugs.

In-Market Drugs

Drug Overview
Mechanism of Action
Regulatory Status
Clinical Trial Results
Drug Uptake and Market Performance

Late-Stage Pipeline Drugs

Drug Overview

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Mechanism of Action
Regulatory Status
Clinical Trial Results
Drug Uptake and Market Performance

*Kindly note that the drugs in the above table only represent a partial list of marketed/pipeline drugs, and the complete list has been provided in the report

Key Questions Answered in this Report:
Market Insights

How has the mucopolysaccharidosis market performed so far and how will it perform in the coming years?
What are the markets shares of various therapeutic segments in 2023 and how are they expected to perform till 2034?
What was the country-wise size of the mucopolysaccharidosis market across the seven major markets in 2023 and what will it look like in 2034?
What is the growth rate of the mucopolysaccharidosis market across the seven major markets and what will be the expected growth over the next ten years?
What are the key unmet needs in the market?

Epidemiology Insights

What is the number of prevalent cases (2018-2034) of mucopolysaccharidosis across the seven major markets?
What is the number of prevalent cases (2018-2034) of mucopolysaccharidosis by age across the seven major markets?
What is the number of prevalent cases (2018-2034) of mucopolysaccharidosis by gender across the seven major markets?
What is the number of prevalent cases (2018-2034) of mucopolysaccharidosis by type across the seven major markets?
How many patients are diagnosed (2018-2034) with mucopolysaccharidosis across the seven major markets?
What is the size of the mucopolysaccharidosis patient pool (2018-2023) across the seven major markets?
What would be the forecasted patient pool (2024-2034) across the seven major markets?
What are the key factors driving the epidemiological trend of mucopolysaccharidosis?

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What will be the growth rate of patients across the seven major markets?

Mucopolysaccharidosis: Current Treatment Scenario, Marketed Drugs and Emerging Therapies

What are the current marketed drugs and what are their market performance?
What are the key pipeline drugs and how are they expected to perform in the coming years?
How safe are the current marketed drugs and what are their efficacies?
How safe are the late-stage pipeline drugs and what are their efficacies?
What are the current treatment guidelines for mucopolysaccharidosis drugs across the seven major markets?
Who are the key companies in the market and what are their market shares?
What are the key mergers and acquisitions, licensing activities, collaborations, etc. related to the mucopolysaccharidosis market?
What are the key regulatory events related to the mucopolysaccharidosis market?
What is the structure of clinical trial landscape by status related to the mucopolysaccharidosis market?
What is the structure of clinical trial landscape by phase related to the mucopolysaccharidosis market?
What is the structure of clinical trial landscape by route of administration related to the mucopolysaccharidosis market?

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Contents

1 PREFACE

2 SCOPE AND METHODOLOGY

2.1 Objectives of the Study
2.2 Stakeholders
2.3 Data Sources
  2.3.1 Primary Sources
  2.3.2 Secondary Sources
2.4 Market Estimation
  2.4.1 Bottom-Up Approach
  2.4.2 Top-Down Approach
2.5 Forecasting Methodology

3 EXECUTIVE SUMMARY

4 MUCOPOLYSACCHARIDOSIS - INTRODUCTION

4.1 Overview
4.2 Regulatory Process
4.3 Epidemiology (2018-2023) and Forecast (2024-2034)
4.4 Market Overview (2018-2023) and Forecast (2024-2034)
4.5 Competitive Intelligence

5 MUCOPOLYSACCHARIDOSIS - DISEASE OVERVIEW

5.1 Introduction
5.2 Symptoms and Diagnosis
5.3 Pathophysiology
5.4 Causes and Risk Factors
5.5 Treatment

6 PATIENT JOURNEY

7 MUCOPOLYSACCHARIDOSIS - EPIDEMIOLOGY AND PATIENT POPULATION

7.1 Epidemiology - Key Insights

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7.2 Epidemiology Scenario - Top 7 Markets
7.2.1 Epidemiology Scenario (2018-2023)
7.2.2 Epidemiology Forecast (2024-2034)
7.2.3 Epidemiology by Age (2018-2034)
7.2.4 Epidemiology by Gender (2018-2034)
7.2.5 Epidemiology by Type (2018-2034)
7.2.6 Diagnosed Cases (2018-2034)
7.2.7 Patient Pool/Treated Cases (2018-2034)
7.3 Epidemiology Scenario - United States
7.3.1 Epidemiology Scenario (2018-2023)
7.3.2 Epidemiology Forecast (2024-2034)
7.3.3 Epidemiology by Age (2018-2034)
7.3.4 Epidemiology by Gender (2018-2034)
7.3.5 Epidemiology by Type (2018-2034)
7.3.6 Diagnosed Cases (2018-2034)
7.3.7 Patient Pool/Treated Cases (2018-2034)
7.4 Epidemiology Scenario - Germany
7.4.1 Epidemiology Scenario (2018-2023)
7.4.2 Epidemiology Forecast (2024-2034)
7.4.3 Epidemiology by Age (2018-2034)
7.4.4 Epidemiology by Gender (2018-2034)
7.4.5 Epidemiology by Type (2018-2034)
7.4.6 Diagnosed Cases (2018-2034)
7.4.7 Patient Pool/Treated Cases (2018-2034)
7.5 Epidemiology Scenario - France
7.5.1 Epidemiology Scenario (2018-2023)
7.5.2 Epidemiology Forecast (2024-2034)
7.5.3 Epidemiology by Age (2018-2034)
7.5.4 Epidemiology by Gender (2018-2034)
7.5.5 Epidemiology by Type (2018-2034)
7.5.6 Diagnosed Cases (2018-2034)
7.5.7 Patient Pool/Treated Cases (2018-2034)
7.6 Epidemiology Scenario - United Kingdom
7.6.1 Epidemiology Scenario (2018-2023)
7.6.2 Epidemiology Forecast (2024-2034)
7.6.3 Epidemiology by Age (2018-2034)
7.6.4 Epidemiology by Gender (2018-2034)
7.6.5 Epidemiology by Type (2018-2034)
7.6.6 Diagnosed Cases (2018-2034)

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7.6.7 Patient Pool/Treated Cases (2018-2034)
7.7 Epidemiology Scenario - Italy
7.7.1 Epidemiology Scenario (2018-2023)
7.7.2 Epidemiology Forecast (2024-2034)
7.7.3 Epidemiology by Age (2018-2034)
7.7.4 Epidemiology by Gender (2018-2034)
7.7.5 Epidemiology by Type (2018-2034)
7.7.6 Diagnosed Cases (2018-2034)
7.7.7 Patient Pool/Treated Cases (2018-2034)
7.8 Epidemiology Scenario - Spain
7.8.1 Epidemiology Scenario (2018-2023)
7.8.2 Epidemiology Forecast (2024-2034)
7.8.3 Epidemiology by Age (2018-2034)
7.8.4 Epidemiology by Gender (2018-2034)
7.8.5 Epidemiology by Type (2018-2034)
7.8.6 Diagnosed Cases (2018-2034)
7.8.7 Patient Pool/Treated Cases (2018-2034)
7.9 Epidemiology Scenario - Japan
7.9.1 Epidemiology Scenario (2018-2023)
7.9.2 Epidemiology Forecast (2024-2034)
7.9.3 Epidemiology by Age (2018-2034)
7.9.4 Epidemiology by Gender (2018-2034)
7.9.5 Epidemiology by Type (2018-2034)
7.9.6 Diagnosed Cases (2018-2034)
7.9.7 Patient Pool/Treated Cases (2018-2034)

8 MUCOPOLYSACCHARIDOSIS - TREATMENT ALGORITHM, GUIDELINES, AND MEDICAL PRACTICES

8.1 Guidelines, Management and Treatment
8.2 Treatment Algorithm

9 MUCOPOLYSACCHARIDOSIS - UNMET NEEDS

10 MUCOPOLYSACCHARIDOSIS - KEY ENDPOINTS OF TREATMENT

11 MUCOPOLYSACCHARIDOSIS - MARKETED PRODUCTS

11.1 List of Mucopolysaccharidosis Marketed Drugs Across the Top 7 Markets

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11.1.1 Elaprase (Idursulfase) - Takeda
11.1.1.1 Drug Overview
11.1.1.2 Mechanism of Action
11.1.1.3 Regulatory Status
11.1.1.4 Clinical Trial Results
11.1.1.5 Sales Across Major Markets
11.1.2 Vimizim (Elosulfase alfa) - BioMarin Pharmaceuticals
11.1.2.1 Drug Overview
11.1.2.2 Mechanism of Action
11.1.2.3 Regulatory Status
11.1.2.4 Clinical Trial Results
11.1.2.5 Sales Across Major Markets
11.1.3 Mepsevii (Vestronidase alfa) - Ultragenyx Pharmaceutical
11.1.3.1 Drug Overview
11.1.3.2 Mechanism of Action
11.1.3.3 Regulatory Status
11.1.3.4 Clinical Trial Results
11.1.3.5 Sales Across Major Markets
11.1.4 Aldurazyme (Laronidase) - BioMarin/Sanofi
11.1.4.1 Drug Overview
11.1.4.2 Mechanism of Action
11.1.4.3 Regulatory Status
11.1.4.4 Clinical Trial Results
11.1.4.5 Sales Across Major Markets
11.1.5 Naglazyme (Galsulfase) - BioMarin Pharmaceutical
11.1.5.1 Drug Overview
11.1.5.2 Mechanism of Action
11.1.5.3 Regulatory Status
11.1.5.4 Clinical Trial Results
11.1.5.5 Sales Across Major Markets

Kindly note that the above only represents a partial list of marketed drugs, and the complete list has been provided in the report

12 MUCOPOLYSACCHARIDOSIS - PIPELINE DRUGS

12.1 List of Mucopolysaccharidosis Pipeline Drugs Across the Top 7 Markets
12.1.1 DNL310 - Denali Therapeutics
12.1.1.1 Drug Overview
12.1.1.2 Mechanism of Action

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12.1.1.3 Clinical Trial Results
12.1.1.4 Safety and Efficacy
12.1.1.5 Regulatory Status
12.1.2 RGX121 - Regenxbio
12.1.2.1 Drug Overview
12.1.2.2 Mechanism of Action
12.1.2.3 Clinical Trial Results
12.1.2.4 Safety and Efficacy
12.1.2.5 Regulatory Status
12.1.3 JR 441 - JCR Pharmaceuticals
12.1.3.1 Drug Overview
12.1.3.2 Mechanism of Action
12.1.3.3 Clinical Trial Results
12.1.3.4 Safety and Efficacy
12.1.3.5 Regulatory Status
12.1.4 AGT-181 - ArmaGen
12.1.4.1 Drug Overview
12.1.4.2 Mechanism of Action
12.1.4.3 Clinical Trial Results
12.1.4.4 Safety and Efficacy
12.1.4.5 Regulatory Status
12.1.5 JR-171 - JCR Pharmaceuticals
12.1.5.1 Drug Overview
12.1.5.2 Mechanism of Action
12.1.5.3 Clinical Trial Results
12.1.5.4 Safety and Efficacy
12.1.5.5 Regulatory Status

Kindly note that the above only represents a partial list of pipeline drugs, and the complete list has been provided in the report.

13 MUCOPOLYSACCHARIDOSIS - ATTRIBUTE ANALYSIS OF KEY MARKETED AND PIPELINE DRUGS

14 MUCOPOLYSACCHARIDOSIS – CLINICAL TRIAL LANDSCAPE

14.1 Drugs by Status
14.2 Drugs by Phase
14.3 Drugs by Route of Administration
14.4 Key Regulatory Events

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15 MUCOPOLYSACCHARIDOSIS - MARKET SCENARIO

15.1 Market Scenario - Key Insights
15.2 Market Scenario - Top 7 Markets
15.2.1 Mucopolysaccharidosis - Market Size
15.2.1.1 Market Size (2018-2023)
15.2.1.2 Market Forecast (2024-2034)
15.2.2 Mucopolysaccharidosis - Market Size by Therapies
15.2.2.1 Market Size by Therapies (2018-2023)
15.2.2.2 Market Forecast by Therapies (2024-2034)
15.3 Market Scenario - United States
15.3.1 Mucopolysaccharidosis - Market Size
15.3.1.1 Market Size (2018-2023)
15.3.1.2 Market Forecast (2024-2034)
15.3.2 Mucopolysaccharidosis - Market Size by Therapies
15.3.2.1 Market Size by Therapies (2018-2023)
15.3.2.2 Market Forecast by Therapies (2024-2034)
15.3.3 Mucopolysaccharidosis - Access and Reimbursement Overview
15.4 Market Scenario - Germany
15.4.1 Mucopolysaccharidosis - Market Size
15.4.1.1 Market Size (2018-2023)
15.4.1.2 Market Forecast (2024-2034)
15.4.2 Mucopolysaccharidosis - Market Size by Therapies
15.4.2.1 Market Size by Therapies (2018-2023)
15.4.2.2 Market Forecast by Therapies (2024-2034)
15.4.3 Mucopolysaccharidosis - Access and Reimbursement Overview
15.5 Market Scenario - France
15.5.1 Mucopolysaccharidosis - Market Size
15.5.1.1 Market Size (2018-2023)
15.5.1.2 Market Forecast (2024-2034)
15.5.2 Mucopolysaccharidosis - Market Size by Therapies
15.5.2.1 Market Size by Therapies (2018-2023)
15.5.2.2 Market Forecast by Therapies (2024-2034)
15.5.3 Mucopolysaccharidosis - Access and Reimbursement Overview
15.6 Market Scenario - United Kingdom
15.6.1 Mucopolysaccharidosis - Market Size
15.6.1.1 Market Size (2018-2023)
15.6.1.2 Market Forecast (2024-2034)

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15.6.2 Mucopolysaccharidosis - Market Size by Therapies
15.6.2.1 Market Size by Therapies (2018-2023)
15.6.2.2 Market Forecast by Therapies (2024-2034)
15.6.3 Mucopolysaccharidosis - Access and Reimbursement Overview
15.7 Market Scenario - Italy
15.7.1 Mucopolysaccharidosis - Market Size
15.7.1.1 Market Size (2018-2023)
15.7.1.2 Market Forecast (2024-2034)
15.7.2 Mucopolysaccharidosis - Market Size by Therapies
15.7.2.1 Market Size by Therapies (2018-2023)
15.7.2.2 Market Forecast by Therapies (2024-2034)
15.7.3 Mucopolysaccharidosis - Access and Reimbursement Overview
15.8 Market Scenario - Spain
15.8.1 Mucopolysaccharidosis - Market Size
15.8.1.1 Market Size (2018-2023)
15.8.1.2 Market Forecast (2024-2034)
15.8.2 Mucopolysaccharidosis - Market Size by Therapies
15.8.2.1 Market Size by Therapies (2018-2023)
15.8.2.2 Market Forecast by Therapies (2024-2034)
15.8.3 Mucopolysaccharidosis - Access and Reimbursement Overview
15.9 Market Scenario - Japan
15.9.1 Mucopolysaccharidosis - Market Size
15.9.1.1 Market Size (2018-2023)
15.9.1.2 Market Forecast (2024-2034)
15.9.2 Mucopolysaccharidosis - Market Size by Therapies
15.9.2.1 Market Size by Therapies (2018-2023)
15.9.2.2 Market Forecast by Therapies (2024-2034)
15.9.3 Mucopolysaccharidosis - Access and Reimbursement Overview

16 MUCOPOLYSACCHARIDOSIS - RECENT EVENTS AND INPUTS FROM KEY OPINION LEADERS

17 MUCOPOLYSACCHARIDOSIS MARKET - SWOT ANALYSIS

17.1 Strengths
17.2 Weaknesses
17.3 Opportunities
17.4 Threats

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18 MUCOPOLYSACCHARIDOSIS MARKET – STRATEGIC RECOMMENDATIONS

19 APPENDIX

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