

Niemann-Pick Disease Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034

https://marketpublishers.com/r/NBC4FDF74A51EN.html

Date: May 2024 Pages: 137 Price: US\$ 6,499.00 (Single User License) ID: NBC4FDF74A51EN

Abstracts

The 7 major Niemann-Pick disease markets are expected to exhibit a CAGR of 4.22% during 2024-2034.

The Niemann-Pick disease market has been comprehensively analyzed in IMARC's new report titled "Niemann-Pick Disease Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034". Niemann-Pick disease refers to a rare genetic disorder that affects the body's ability to metabolize fat and cholesterol. The condition results in the accumulation of lipids in abnormal amounts due to a deficiency or absence of an enzyme called acid sphingomyelinase (ASM), which is responsible for breaking down fatty substances. There are several types of Niemann-Pick disease, with varying severity and age of onset. Types A and B are the most common forms of this illness, which primarily affect the liver and spleen and may cause enlargement of these organs, anemia, and low platelet counts. Type C is a more severe form of the disease, affecting the liver, spleen and brain and causing developmental delays, seizures, difficulty with movement, and cognitive impairment. The diagnosis of Niemann-Pick disease is typically made after a comprehensive review of the patient's indications, medical history, and physical examination. The healthcare provider may also perform a blood or skin sample biopsy to measure the amount of sphingomyelinase in white blood cells and confirm disease diagnosis.

The increasing prevalence of autosomal recessive inherited disorders, that result in genetic mutations, causing a buildup of cholesterol and other fats within the body cells, is primarily driving the Niemann-Pick disease market. Furthermore, the widespread utilization of enzyme inhibitors, involving miglustat, which work by inhibiting the body from generating fatty substances and their abnormal accumulation in the organs, is also



bolstering the market growth. Additionally, several government agencies and non-governmental organizations are funding health education programs to raise awareness about early diagnosis and available treatment options for Niemann-Pick disease. This, in turn, is creating a positive outlook for the market. Moreover, the inflating application of enzyme replacement therapy, since it aims to replace defective or deficient ASM enzymes, thereby minimizing fat accumulation, providing symptom relief, and enhancing overall well-being in patients, is further acting as another significant growth-inducing factor. Apart from this, the emerging popularity of gene therapy, that involves introducing a functional copy of the faulty gene into the body to treat this ailment, is expected to drive the Niemann-Pick disease market in the coming years.

IMARC Group's new report provides an exhaustive analysis of the Niemann-Pick disease market in the United States, EU5 (Germany, Spain, Italy, France, and 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 Niemann-Pick disease and also represents the largest market for its treatment. Furthermore, the current treatment practice/algorithm, market drivers, challenges, opportunities, reimbursement scenario and 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 Niemann-Pick disease market in any manner.

Time Period of the Study

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

Countries Covered

| United States |
|----------------|
| Germany |
| France |
| United Kingdom |
| Italy |
| Spain |

Niemann-Pick Disease Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 202...



Japan

Analysis Covered Across Each Country

Historical, current, and future epidemiology scenario Historical, current, and future performance of the Niemann-Pick disease market Historical, current, and future performance of various therapeutic categories in the market Sales of various drugs across the Niemann-Pick disease market Reimbursement scenario in the market In-market and pipeline drug Competitive Landscape: This report also provides a detailed analysis of the current Niemann-Pick disease 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 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 Niemann-Pick disease 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 Niemann-Pick disease market across the seven major markets in 2023 and what will it look like in 2034?

What is the growth rate of the Niemann-Pick disease 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 Niemann-Pick disease across the seven major markets?

What is the number of prevalent cases (2018-2034) of Niemann-Pick disease by age across the seven major markets?

What is the number of prevalent cases (2018-2034) of Niemann-Pick disease by gender across the seven major markets?

What is the number of prevalent cases (2018-2034) of Niemann-Pick disease by type across the seven major markets?

How many patients are diagnosed (2018-2034) with Niemann-Pick disease across the seven major markets?

What is the size of the Niemann-Pick disease 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 Niemann-Pick disease? What will be the growth rate of patients across the seven major markets?

Niemann-Pick Disease: 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 Niemann-Pick disease 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 Niemann-Pick disease market?

What are the key regulatory events related to the Niemann-Pick disease market?

What is the structure of clinical trial landscape by status related to the Niemann-Pick disease market?

What is the structure of clinical trial landscape by phase related to the Niemann-Pick disease market?

What is the structure of clinical trial landscape by route of administration related to the Niemann-Pick disease market?



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 NIEMANN-PICK DISEASE - 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 NIEMANN-PICK DISEASE - 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 NIEMANN-PICK DISEASE - EPIDEMIOLOGY AND PATIENT POPULATION

7.1 Epidemiology - Key Insights

Niemann-Pick Disease Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 202..



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)

Niemann-Pick Disease Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 202..



- 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 NIEMANN-PICK DISEASE - TREATMENT ALGORITHM, GUIDELINES, AND MEDICAL PRACTICES

- 8.1 Guidelines, Management and Treatment
- 8.2 Treatment Algorithm

9 NIEMANN-PICK DISEASE - UNMET NEEDS

10 NIEMANN-PICK DISEASE - KEY ENDPOINTS OF TREATMENT

11 NIEMANN-PICK DISEASE - MARKETED PRODUCTS

11.1 List of Niemann-Pick Disease Marketed Drugs Across the Top 7 Markets



- 11.1.1 Xenpozyme (Olipudase alfa) Sanofi
 - 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 Zavesca (Miglustat) Actelion 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

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

12 NIEMANN-PICK DISEASE - PIPELINE DRUGS

- 12.1 List of Niemann-Pick Disease Pipeline Drugs Across the Top 7 Markets
 - 12.1.1 Trappsol Cyclo Cyclo Therapeutics
 - 12.1.1.1 Drug Overview
 - 12.1.1.2 Mechanism of Action
 - 12.1.1.3 Clinical Trial Results
 - 12.1.1.4 Safety and Efficacy
 - 12.1.1.5 Regulatory Status
 - 12.1.2 VTS 270 Mandos
 - 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 AZ-3102 Azafaros
 - 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 Arimoclomol Zevra Therapeutics
 - 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

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

13. NIEMANN-PICK DISEASE - ATTRIBUTE ANALYSIS OF KEY MARKETED AND PIPELINE DRUGS

14. NIEMANN-PICK DISEASE – CLINICAL TRIAL LANDSCAPE

14.1 Drugs by Status14.2 Drugs by Phase14.3 Drugs by Route of Administration14.4 Key Regulatory Events

15 NIEMANN-PICK DISEASE - MARKET SCENARIO

15.1 Market Scenario - Key Insights 15.2 Market Scenario - Top 7 Markets 15.2.1 Niemann-Pick Disease - Market Size 15.2.1.1 Market Size (2018-2023) 15.2.1.2 Market Forecast (2024-2034) 15.2.2 Niemann-Pick Disease - 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 Niemann-Pick Disease - Market Size 15.3.1.1 Market Size (2018-2023) 15.3.1.2 Market Forecast (2024-2034) 15.3.2 Niemann-Pick Disease - 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 Niemann-Pick Disease - Access and Reimbursement Overview 15.4 Market Scenario - Germany 15.4.1 Niemann-Pick Disease - Market Size 15.4.1.1 Market Size (2018-2023) 15.4.1.2 Market Forecast (2024-2034) 15.4.2 Niemann-Pick Disease - 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 Niemann-Pick Disease - Access and Reimbursement Overview 15.5 Market Scenario - France 15.5.1 Niemann-Pick Disease - Market Size 15.5.1.1 Market Size (2018-2023) 15.5.1.2 Market Forecast (2024-2034) 15.5.2 Niemann-Pick Disease - 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 Niemann-Pick Disease - Access and Reimbursement Overview 15.6 Market Scenario - United Kingdom 15.6.1 Niemann-Pick Disease - Market Size 15.6.1.1 Market Size (2018-2023) 15.6.1.2 Market Forecast (2024-2034) 15.6.2 Niemann-Pick Disease - 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 Niemann-Pick Disease - Access and Reimbursement Overview 15.7 Market Scenario - Italy 15.7.1 Niemann-Pick Disease - Market Size 15.7.1.1 Market Size (2018-2023) 15.7.1.2 Market Forecast (2024-2034) 15.7.2 Niemann-Pick Disease - 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 Niemann-Pick Disease - Access and Reimbursement Overview 15.8 Market Scenario - Spain 15.8.1 Niemann-Pick Disease - Market Size 15.8.1.1 Market Size (2018-2023) 15.8.1.2 Market Forecast (2024-2034) 15.8.2 Niemann-Pick Disease - 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 Niemann-Pick Disease - Access and Reimbursement Overview 15.9 Market Scenario - Japan 15.9.1 Niemann-Pick Disease - Market Size 15.9.1.1 Market Size (2018-2023) 15.9.1.2 Market Forecast (2024-2034)



- 15.9.2 Niemann-Pick Disease 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 Niemann-Pick Disease Access and Reimbursement Overview

16 NIEMANN-PICK DISEASE - RECENT EVENTS AND INPUTS FROM KEY OPINION LEADERS

17 NIEMANN-PICK DISEASE MARKET - SWOT ANALYSIS

17.1 Strengths17.2 Weaknesses17.3 Opportunities17.4 Threats

18 NIEMANN-PICK DISEASE MARKET – STRATEGIC RECOMMENDATIONS

19 APPENDIX



I would like to order

Product name: Niemann-Pick Disease Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034 Product link: <u>https://marketpublishers.com/r/NBC4FDF74A51EN.html</u> Price: US\$ 6,499.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service: info@marketpublishers.com

Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <u>https://marketpublishers.com/r/NBC4FDF74A51EN.html</u>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name: Last name: Email: Company: Address: City: Zip code: Country: Tel: Fax: Your message:

**All fields are required

Custumer signature _____

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <u>https://marketpublishers.com/docs/terms.html</u>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970



Niemann-Pick Disease Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 202...