

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

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### **Abstracts**

The 7 major Niemann-Pick disease type A markets are expected to exhibit a CAGR of 4.31% during 2024-2034.

The Niemann-Pick disease type A market has been comprehensively analyzed in IMARC's new report titled "Niemann-Pick Disease Type A Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034". Niemann-Pick disease type A (NPDA) is a rare and severe genetic disorder characterized by the body's inability to properly metabolize a lipid called sphingomyelin. This leads to its accumulation within various organs, primarily the liver, spleen, and brain. NPDA is an autosomal recessive disorder, meaning both copies of the responsible gene must be mutated for the condition to manifest. Infants suffering from the illness typically exhibit symptoms within the first few months of life. These indications include progressive enlargement of the liver and spleen, difficulty feeding, failure to thrive, and developmental delays. Various other neurological symptoms, such as loss of muscle tone (hypotonia) and an exaggerated startle response, become apparent as the disorder progresses. NPDA has a rapid and devastating course, often leading to severe impairment of neurological function and a shortened lifespan. Diagnosing this disease involves a combination of clinical evaluation, imaging studies, and biochemical testing of blood and tissue samples.

The escalating incidences of genetic mutations affecting the SMPD1 gene, which can lead to the harmful accumulation of sphingomyelin in various body organs, predominantly the liver, spleen, and brain, are primarily driving the Niemann-Pick disease type A market. In addition to this, the inflating utilization of effective therapeutic interventions, including enzyme replacement therapy and substrate reduction, for



managing the condition and impeding its progression, is also creating a positive outlook for the market. Moreover, the widespread adoption of supportive treatments, such as respiratory assistance, nutritional support, and seizure management, is further bolstering the market growth. These measures aid in ameliorating symptoms, enhancing the quality of life, and extending survival rates for individuals suffering from NPDA. Apart from this, the rising usage of advanced technologies like biomarker identification and genetic screening, since they help to provide early diagnosis and timely intervention, is acting as another significant growth-inducing factor. Additionally, the emerging research and development activities, which are aimed at uncovering novel therapeutic avenues, such as small-molecule inhibitors to treat the condition, are also augmenting the market growth. Furthermore, the increasing application of gene therapy that involves the introduction of functional genes to replace or rectify the aberrant ones responsible for the disorder, is expected to drive the Niemann-Pick disease type A market during the forecast period.

IMARC Group's new report provides an exhaustive analysis of the Niemann-Pick disease type A 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 type A 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 type A 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

Japan

Analysis Covered Across Each Country

Historical, current, and future epidemiology scenario

Historical, current, and future performance of the Niemann-Pick disease type A market Historical, current, and future performance of various therapeutic categories in the market

Sales of various drugs across the Niemann-Pick disease type A market Reimbursement scenario in the market

In-market and pipeline drugs

Competitive Landscape:

This report also provides a detailed analysis of the current Niemann-Pick disease type A 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
Key Questions Answered in this Report:
Market Insights

How has the Niemann-Pick disease type A 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 type A 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 type A 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 type A across the seven major markets?

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

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

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

What is the size of the Niemann-Pick disease type A 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 type A?

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

Niemann-Pick Disease Type A: 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 type A 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 type A market?



What are the key regulatory events related to the Niemann-Pick disease type A market? What is the structure of clinical trial landscape by status related to the Niemann-Pick disease type A market?

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

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



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