

## Acid Sphingomyelinase Deficiency Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034

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## **Abstracts**

The 7 major acid sphingomyelinase deficiency markets are expected to exhibit a CAGR of 5.76% during 2024-2034.

The acid sphingomyelinase deficiency market has been comprehensively analyzed in IMARC's new report titled "Acid Sphingomyelinase Deficiency Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034". Acid sphingomyelinase deficiency is a rare inherited lysosomal storage disorder that affects the metabolism of lipids in cells. In individuals suffering from the ailment, a biological substance called sphingomyelin accumulates within various cells and tissues of the body, particularly in the liver, spleen, lungs, and brain. This can cause progressive damage to these organs, leading to several health complications, like life-threatening lung failure. The most common indications associated with the illness are abdominal distension and discomfort, enlarged liver and spleen, seizures, failure to thrive, difficulty breathing, recurrent respiratory infections, developmental delays, loss of muscle tone, decreased blood cells, cherry-red spots in the eye, bone abnormalities, etc. The diagnosis of acid sphingomyelinase deficiency is mainly based on the evaluation of presenting symptoms, medical history, and physical examination. The healthcare professional may further perform imaging techniques, like X-rays or magnetic resonance imaging, to determine organ involvement and monitor disease progression in patients. In some cases, a bone marrow aspiration or biopsy may be done to assess the storage of sphingomyelin in the cells.

The increasing prevalence of alterations in the gene, which encodes instructions for the production of an enzyme that breaks down lipids in the body, is primarily driving the acid sphingomyelinase deficiency market. In addition to this, the inflating utilization of



substrate reduction therapy, since it involves using drugs that decrease the formation of sphingomyelin, thereby reducing the burden of accumulated lipids in the cells, is also creating a positive outlook for the market. Moreover, the widespread adoption of hematopoietic stem cell transplantation, owing to its various advantages, such as halting disease progression, stabilizing neurological symptoms, and preventing organ damage, is further bolstering the market growth. Apart from this, the rising application of gene editing techniques to precisely modify or replace the faulty gene with a functional one is acting as another significant growth-inducing factor. Additionally, the emerging popularity of molecular chaperone therapy, which utilizes small molecules or other therapeutic agents to stabilize misfolded proteins, enhancing their activity and functionality, is expected to drive the acid sphingomyelinase deficiency market during the forecast period.

IMARC Group's new report provides an exhaustive analysis of the acid sphingomyelinase deficiency 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 acid sphingomyelinase deficiency 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 acid sphingomyelinase deficiency 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 acid sphingomyelinase deficiency market

Historical, current, and future performance of various therapeutic categories in the market

Sales of various drugs across the acid sphingomyelinase deficiency market Reimbursement scenario in the market

In-market and pipeline drugs

Competitive Landscape:

This report also provides a detailed analysis of the current acid sphingomyelinase deficiency 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 acid sphingomyelinase deficiency 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 acid sphingomyelinase deficiency market across the seven major markets in 2023 and what will it look like in 2034?

What is the growth rate of the acid sphingomyelinase deficiency 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 acid sphingomyelinase deficiency across the seven major markets?

What is the number of prevalent cases (2018-2034) of acid sphingomyelinase deficiency by age across the seven major markets?

What is the number of prevalent cases (2018-2034) of acid sphingomyelinase deficiency by gender across the seven major markets?

How many patients are diagnosed (2018-2034) with acid sphingomyelinase deficiency across the seven major markets?

What is the size of the acid sphingomyelinase deficiency 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 acid sphingomyelinase deficiency?

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

Acid Sphingomyelinase Deficiency: 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 acid sphingomyelinase deficiency 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 acid sphingomyelinase deficiency market?

What are the key regulatory events related to the acid sphingomyelinase deficiency market?

What is the structure of clinical trial landscape by status related to the acid sphingomyelinase deficiency market?

What is the structure of clinical trial landscape by phase related to the acid sphingomyelinase deficiency market?

What is the structure of clinical trial landscape by route of administration related to the acid sphingomyelinase deficiency market?



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