

Global Sanfilippo Syndrome Market: Key Trends, Market Share, Growth Drivers, And Forecast For 2025-2034

*The Business Research Company's
Sanfilippo Syndrome Global Market
Report 2025 – Market Size, Trends, And
Forecast 2025-2034*

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KINGDOM, May 30, 2025

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growth can be attributed to increasing awareness of rare genetic disorders, increased funding for rare disease research by governments, expansion of patient advocacy groups and support organizations, progression in genetic screening programs, and the growing practice in orphan drug designations for rare diseases.

What Is The Anticipated Growth Rate And Market Size Of
The Global Sanfilippo Syndrome Market?

The sanfilippo syndrome market is projected to experience
accelerated growth over the next few years. By 2029, it is

estimated to reach \$13.79 billion, with a CAGR of 10.5%. This forecast period's growth is attributed to the development in personalized medicine strategies for sanfilippo syndrome, increased acceptance of gene therapy as a curative approach, rising demand for targeted drug delivery systems, increasing adoption of stem cell therapies with enhanced efficacy, and the global expansion of newborn screening programs. Major trends likely to emerge during the forecast period include advancements in gene therapy technologies, innovations in stem cell therapy, targeted drug delivery systems, advancements in blood-brain barrier BBB penetration strategies, and developments within neuroprotective small molecules.

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What Are The Key Drivers Propelling The Growth Of The Sanfilippo Syndrome Market?

The rise in the focus on gene therapies is expected to boost the growth of the sanfilippo syndrome market further. Gene therapies constitute medical treatments that aim to modify or replace faulty genes to prevent or treat diseases. The appeal of gene therapies lies in their potential to treat genetic disorders, rare diseases, and chronic conditions at the root cause - at the molecular level. For example, gene therapies can treat Sanfilippo syndrome by delivering functional copies of the defective gene, which can restore enzyme production, decreasing the build-up of harmful substances in cells. Backing this statement, the American Society of Gene & Cell Therapy reported in January 2024 that the number of gene therapies in Phase III clinical trials increased by 10% in the fourth quarter. This marks the first quarterly growth of this magnitude since the third quarter of 2022.

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Which Key Industry Players Are Dominating The Sanfilippo Syndrome Market?

Leading companies in the sanfilippo syndrome market include Takeda Pharmaceutical Company Limited, BioMarin Pharmaceutical Inc., Qiagen N.V., Natera Inc., Guardant Health Inc., Novogene Corporation, Ultragenyx Pharmaceutical Inc., JCR Pharmaceuticals Co. Ltd., Oxford Nanopore Technologies plc, Pacific Biosciences of California Inc., Denali Therapeutics Inc., MacroGen Inc., REGENXBIO Inc., Personalis Inc., Veritas Genetics Inc., Greenwood Genetic Center, ArmaGen Inc., Abeona Therapeutics Inc., Orchard Therapeutics plc, Allievex Corporation.

What Trends Are Emerging In The Sanfilippo Syndrome Industry?

Companies leading the sanfilippo syndrome market are concentrating on creating innovative treatments such as gene therapies. These are poised to enhance patient outcomes and slow disease progression by correcting or replacing faulty genes in the body. To illustrate, in February 2025, Ultragenyx Pharmaceutical Inc., a US-based biopharmaceutical company, announced promising findings on UX111 ABO-102, an AAV gene therapy for sanfilippo syndrome Type A MPS IIIA. The therapy demonstrated significant improvements in Bayley-III raw scores for cognitive abilities, and both receptive and expressive communication. Additionally, it led to a drastic decrease in cerebrospinal fluid CSF heparan sulfate HS levels, which correlated with improved clinical outcomes.

How Is The Global Sanfilippo Syndrome Market Segmented?

Segments for the sanfilippo syndrome market are:

- 1 By Treatment: Enzyme Replacement Therapy, Gene Therapy, Genistein, Other Treatments
- 2 By Diagnosis: Glycosaminoglycan Analysis GAG Analysis, Genomic DNA Sequencing, Activity

Assay, Other Diagnosis

3 By End-User: Hospitals, Research Institutions

Subsegments include:

1 By Enzyme Replacement Therapy: Intravenous Enzyme Replacement Therapy, Intrathecal Enzyme Replacement Therapy, Intracerebroventricular ICV Enzyme Replacement Therapy

2 By Gene Therapy: Adeno-Associated Virus AAV-Based In Vivo Gene Therapy, Lentiviral Vector-Based Ex Vivo Gene Therapy, Non-Viral Vector Gene Delivery

3 By Genistein: Oral Genistein Supplementation, High-Dose Genistein Regimens, Low-Dose Genistein Regimens

4 By Other Treatments: Substrate Reduction Therapy SRT, Stem Cell Therapy, Anti-Inflammatory Agents, Symptomatic Management Drugs.

Where Does The Largest Sanfilippo Syndrome Market Reside?

In 2024, North America was the largest region in the sanfilippo syndrome market. Other regions covered in the report include Asia-Pacific, Western Europe, Eastern Europe, South America, the Middle East, and Africa.

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