+357 96 030922
info@marketpublishers.com

Global Neurometabolic Disorders Market Size study, by Type (Amino Acid Disorders, Organic Acidemias, Fatty Acid Oxidation Disorders, Mitochondrial Disorders), by Diagnosis Method, Therapeutic Approaches, End User and Regional Forecasts 2022-2032

https://marketpublishers.com/r/G47C38339083EN.html
Date: May 2025
Pages: 285
Price: US$ 3,218.00 (Single User License)
ID: G47C38339083EN

Abstracts

The Global Neurometabolic Disorders Market is valued approximately at USD 7.77 billion in 2023 and is anticipated to grow with a healthy growth rate of more than 6.02% over the forecast period 2024–2032. Neurometabolic disorders, a diverse group of rare, genetically driven conditions affecting the brain's metabolic function, have emerged as a critical frontier in modern medical research and care delivery. These conditions, often manifesting early in life, are driven by defects in metabolic pathways involving amino acids, fatty acids, and mitochondrial functions. What sets this market apart is the urgency and complexity of care required, which has catalyzed innovation across diagnostic technologies and therapeutic modalities. A growing global awareness, increased newborn screening programs, and expanding access to genomic medicine are reshaping patient journeys from delayed diagnosis to early intervention—unlocking the potential for improved long-term outcomes.

As the landscape of rare diseases evolves, the neurometabolic disorders market has found momentum propelled by surging investments in genomic sequencing, molecular diagnostics, and innovative therapies. Diagnostic methods such as neuroimaging, biochemical testing, and next-generation sequencing are now playing pivotal roles in enabling clinicians to pinpoint rare inborn errors of metabolism with enhanced accuracy. Moreover, pharmacological advancements such as gene therapy, enzyme replacement, and dietary interventions are not only addressing symptoms but are now beginning to

+357 96 030922
info@marketpublishers.com

modify disease course in select conditions. Governments and private players alike are funding research pipelines and launching patient support initiatives, which are contributing to increased therapeutic approvals and robust product pipelines.

However, despite remarkable progress, the market continues to be hindered by several structural challenges. A significant barrier remains the high cost of advanced diagnostics and specialized therapies—frequently unaffordable or inaccessible in low- and middle-income regions. Additionally, the rarity and variability of neurometabolic disorders often result in limited clinical data, hindering large-scale trials and slowing regulatory approval. The intricacies of managing these disorders also demand multidisciplinary expertise, yet such teams are not universally available. These limitations underscore the need for innovative business models, cross-border collaborations, and real-world evidence frameworks to accelerate progress and drive equitable care access.

Yet, in the face of these hurdles, several dynamic forces continue to expand the opportunity space. The emergence of precision medicine and gene editing tools such as CRISPR has opened the door to potentially curative therapies, especially for monogenic neurometabolic conditions. Simultaneously, increasing academic-industry partnerships and digital health integration are fostering decentralized diagnostic platforms and home-based treatment options—particularly important for pediatric patients with chronic care needs. Pharmaceutical companies are increasingly prioritizing rare disease portfolios, encouraged by orphan drug designations and regulatory incentives from the U.S. FDA and European Medicines Agency, thereby contributing to heightened R&D activity across this niche but impactful market.

From a regional standpoint, North America currently commands a leading share of the neurometabolic disorders market, supported by favorable reimbursement policies, sophisticated healthcare infrastructure, and pioneering research hubs in the United States and Canada. Europe follows closely, bolstered by expansive newborn screening programs, rare disease registries, and public health funding. Meanwhile, Asia Pacific is expected to register the fastest growth over the forecast period, with rising healthcare expenditure, rapid adoption of genetic diagnostics, and a growing footprint of multinational pharmaceutical players in countries like India, China, and Japan. Latin America and the Middle East & Africa, although currently underpenetrated, are progressively integrating rare disease management into their national health strategies, signaling a gradual but important shift toward inclusive access.

Major market player included in this report are:

+357 96 030922
info@marketpublishers.com

Sanofi S.A.

BioMarin Pharmaceutical Inc.

Takeda Pharmaceutical Company Limited

Ultragenyx Pharmaceutical Inc.

AbbVie Inc.

Genzyme Corporation

Amicus Therapeutics, Inc.

Horizon Therapeutics plc

Aeglea BioTherapeutics, Inc.

Retrophin, Inc.

BridgeBio Pharma, Inc.

Orchard Therapeutics

Alexion Pharmaceuticals, Inc.

Stealth BioTherapeutics Corp.

Acer Therapeutics Inc.

The detailed segments and sub-segment of the market are explained below:

By Type

Amino Acid Disorders

Organic Acidemias

+357 96 030922
info@marketpublishers.com

- Fatty Acid Oxidation Disorders
- Mitochondrial Disorders
- Neurodegenerative Disorders

By Diagnosis Method

- Neuroimaging
- Biochemical Testing
- Genetic Testing
- Electroencephalography
- Lumbar Puncture

By Therapeutic Approaches

- Dietary Management
- Gene Therapy
- Enzyme Replacement Therapy
- Supportive Therapies
- Pharmacological Treatment

By End User

- Hospitals
- Diagnostic Laboratories

+357 96 030922
info@marketpublishers.com

Research Institutes

Homecare Settings

Pharmaceutical Companies

By Region:

North America

U.S.

Canada

Europe

UK

Germany

France

Spain

Italy

Rest of Europe

Asia Pacific

China

India

Japan

+357 96 030922
info@marketpublishers.com

Australia

South Korea

Rest of Asia Pacific

Latin America

Brazil

Mexico

Rest of Latin America

Middle East & Africa

Saudi Arabia

South Africa

Rest of Middle East & Africa

Years considered for the study are as follows:

Historical year – 2022

Base year – 2023

Forecast period – 2024 to 2032

Key Takeaways:

Market Estimates & Forecast for 10 years from 2022 to 2032.

Annualized revenues and regional level analysis for each market segment.

+357 96 030922
info@marketpublishers.com

Detailed analysis of geographical landscape with Country level analysis of major regions.

Competitive landscape with information on major players in the market.

Analysis of key business strategies and recommendations on future market approach.

Analysis of competitive structure of the market.

Demand side and supply side analysis of the market.

Companies Mentioned

Sanofi S.A.

BioMarin Pharmaceutical Inc.

Takeda Pharmaceutical Company Limited

Ultragenyx Pharmaceutical Inc.

AbbVie Inc.

Genzyme Corporation

Amicus Therapeutics, Inc.

Horizon Therapeutics plc

Aeglea BioTherapeutics, Inc.

Retrophin, Inc.

BridgeBio Pharma, Inc.

Orchard Therapeutics

+357 96 030922
info@marketpublishers.com

Alexion Pharmaceuticals, Inc.

Stealth BioTherapeutics Corp.

Acer Therapeutics Inc.

+357 96 030922
info@marketpublishers.com

Contents

+357 96 030922
info@marketpublishers.com

CHAPTER 3. GLOBAL NEUROMETABOLIC DISORDERS MARKET DYNAMICS

CHAPTER 4. GLOBAL NEUROMETABOLIC DISORDERS MARKET INDUSTRY ANALYSIS

+357 96 030922
info@marketpublishers.com

CHAPTER 5. GLOBAL NEUROMETABOLIC DISORDERS MARKET SIZE & FORECASTS BY TYPE 2022–2032

CHAPTER 6. GLOBAL NEUROMETABOLIC DISORDERS MARKET SIZE & FORECASTS BY DIAGNOSIS METHOD 2022–2032

CHAPTER 7. GLOBAL NEUROMETABOLIC DISORDERS MARKET SIZE & FORECASTS BY THERAPEUTIC APPROACHES 2022–2032

+357 96 030922
info@marketpublishers.com

+357 96 030922
info@marketpublishers.com

CHAPTER 10. COMPETITIVE INTELLIGENCE

+357 96 030922
info@marketpublishers.com

CHAPTER 11. RESEARCH PROCESS

+357 96 030922
info@marketpublishers.com

List Of Tables

LIST OF TABLES

+357 96 030922
info@marketpublishers.com

List Of Figures

LIST OF FIGURES

+357 96 030922
info@marketpublishers.com

I would like to order

Product name: Global Neurometabolic Disorders Market Size study, by Type (Amino Acid Disorders, Organic Acidemias, Fatty Acid Oxidation Disorders, Mitochondrial Disorders), by Diagnosis Method, Therapeutic Approaches, End User and Regional Forecasts 2022-2032

Product link: https://marketpublishers.com/r/G47C38339083EN.html

Price: US$ 3,218.00 (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 https://marketpublishers.com/r/G47C38339083EN.html