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# Global Fabry disease - 2024 -2031

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## Abstracts

Fabry Disease Treatment Market Size

The Global Fabry Disease Treatment Market size reached USD 1,876.7 million in 2022 and is projected to witness lucrative growth by reaching up to USD 3,287.2 million by 2031. The global Fabry disease treatment market is expected to exhibit a CAGR of 7.4% during the forecast period (2024-2031). The key market trend is the emergence of precision medicine and personalized therapies.

For instance, in May 2023, 4D Molecular Therapeutics, Inc., a clinical-stage biotherapeutics company, the Company is initially focused on five clinical-stage product candidates in three therapeutic areas for both rare and large market diseases such as ophthalmology, pulmonology, and cardiology (Fabry disease cardiomyopathy).

Fabry disease, also known as Anderson-Fabry disease, is a rare genetic disorder that belongs to a group of conditions known as lysosomal storage disorders. It is caused by mutations in the GLA gene, which results in a deficiency or malfunction of the enzyme alpha-galactosidase A (AGA). This enzyme is responsible for breaking down a fatty substance called globotriaosylceramide (Gb3) within cells.

The Fabry disease treatment market is driven by factors such as increasing awareness and diagnosis, increasing demand for novel therapies such as chaperone therapies and enzyme replacement therapies, advancements in treatment options, increasing R&D activities, and technological advancements.

For instance, in February 2023, The novel recombinant human ?–Galactosidase–A (?–Gal–A) enzyme is being investigated as a PEGylated enzyme replacement therapy (ERT) for Fabry disease. The treatment is intended to help address unmet medical needs for these patients, such as progressive kidney decline.

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## Fabry Disease Treatment Market Dynamics

### Increasing Demand for Novel Therapies

The increasing demand for novel therapies such as chaperone therapies and enzyme replacement therapies are the major factors driving the market share during the forecast period. Chaperone therapies, also known as pharmacological chaperone therapies, offer a personalized treatment approach for Fabry disease by targeting specific genetic mutations.

These therapies aim to stabilize and enhance the function of the patient's own defective enzyme, enabling it to effectively break down the accumulated substances. The demand for chaperone therapies is increasing due to their potential benefits in addressing the underlying molecular defects associated with specific mutations, leading to improved treatment outcomes.

### High Cost of the Treatment and Limited Treatment Options

The high cost of the treatment and limited treatment options are the major factors hampering the market growth during the forecast period. Fabry disease treatments, including enzyme replacement therapies (ERT) and chaperone therapies, can be expensive. The high cost of these therapies may pose financial challenges for patients, healthcare systems, and payers. Affordability concerns can limit patient access and uptake of treatments, impacting market demand.

Fabry disease patients who have advanced disease manifestations or organ damage may have limited treatment options. Existing therapies may not fully address the complexities and challenges associated with the advanced stages of the disease. The lack of effective treatments for advanced cases can limit market growth and patient outcomes.

## Fabry Disease Treatment Market Segment Analysis

The global Fabry disease treatment market is segmented based on drugs, treatment, route of administration, distribution channel, and region.

### Based on Treatment, the Enzyme Replacement Therapy (ERT) Segment is Expected to Dominate the Market Share

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Based on the treatment, the enzyme replacement therapy (ERT) segment is expected to dominate the market share during the forecast period. The market for enzyme replacement therapy holds the 65.2% of the market share in 2022. ERT has been widely used and accepted as a standard treatment for Fabry disease for many years.

It has a proven track record of effectiveness in reducing symptoms, improving quality of life, and slowing disease progression. The long-standing use and established efficacy of ERT have contributed to its dominance in the market.

Fabry Disease Treatment Market Geographical Share

North America Holds the Largest Market Share Due to the Increasing Adoption of Novel Therapies and the Presence of a Strong Healthcare Infrastructure

North America holds the largest market share during the forecast period due to the increasing adoption of novel therapies and the presence of strong healthcare infrastructure. The region holds the 43.2% market share for the Fabry disease treatment market in 2022.

North America, particularly the United States, has a relatively higher healthcare expenditure compared to other regions. The increased healthcare spending provides better access to advanced treatments, including novel therapies, for Fabry disease patients. The higher healthcare expenditure positively impacts market growth and the adoption of innovative treatments in North America.

Fabry Disease Treatment Market Key Players

The major global players include Sanofi S.A, Takeda Pharmaceutical Company Ltd, Teva Pharmaceutical Industries Ltd., Amicus Therapeutics Inc., Amgen Inc., Bristol-Myers Squibb Company, Neuraltus Pharmaceuticals Inc., Novartis AG, Pfizer Inc., and Idorsia Pharmaceuticals Ltd.

COVID-19 Impact on Fabry Disease Treatment Market

The COVID-19 pandemic has had a significant impact on the Fabry disease treatment market. The pandemic led to disruptions in healthcare services worldwide. Hospitals and healthcare facilities were overwhelmed with COVID-19 patients, and resources were redirected to address the immediate crisis. This diversion of resources and focus

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on pandemic management resulted in a temporary disruption of routine medical services, including the diagnosis and treatment of rare diseases like Fabry disease.

The economic impact of the COVID-19 pandemic resulted in financial challenges for healthcare systems, patients, and payers. Affordability concerns may have influenced treatment decisions and market dynamics.

Russia-Ukraine Conflict Analysis

The Russia-Ukraine war significantly impacted the Fabry disease treatment market growth. The Russia-Ukraine conflict may indirectly impact research and development activities related to Fabry disease treatments. Institutions and organizations involved in research collaborations or clinical trials that have a presence in conflict-affected areas may face challenges in conducting studies, accessing funding, or collaborating with international partners.

Geopolitical conflicts can affect global investor confidence and risk perceptions. If the conflict creates broader market uncertainties, investors may become more cautious and risk-averse. This could potentially impact investments in healthcare companies involved in the development and commercialization of Fabry disease treatments.

Key Developments

In May 2023, Sangamo Therapeutics, Inc., a genomic medicine company, announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to isaralgagene civaparvovec, or ST-920, a wholly owned gene therapy product candidate for the treatment of Fabry disease.

In May 2023, Protalix BioTherapeutics, a biopharmaceutical company, and Chiesi Group, a specialty pharmaceutical company developed a drug called Elfabrio is approved by FDA for treating Fabry disease, a rare inherited metabolic disorder. The drug, Elfabrio, is an enzyme replacement therapy.

Why Purchase the Report?

To visualize the global Fabry disease treatment market segmentation based on drugs, treatment, route of administration, distribution channel, and region, as well as understand key commercial assets and players.

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Identify commercial opportunities by analyzing trends and co-development.

Excel data sheet with numerous data points of Fabry disease treatment market-level with all segments.

PDF report consists of a comprehensive analysis after exhaustive qualitative interviews and an in-depth study.

Product mapping available as Excel consisting of key products of all the major players.

The global Fabry disease treatment market report would provide approximately 54 tables, 46 figures, and 195 Pages.

Target Audience 2024

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# Contents

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# List Of Tables

## LIST OF TABLES

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# List Of Figures

## LIST OF FIGURES

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