Rare Disease Treatment - Market Share Analysis, Industry Trends & Statistics, Growth Forecasts 2019 - 2029

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Report description:

The Rare Disease Treatment Market size is estimated at USD 216.65 billion in 2024, and is expected to reach USD 380.62 billion by 2029, growing at a CAGR of 11.93% during the forecast period (2024-2029).

The COVID-19 pandemic had a profound impact on the rare disease treatment market owing to the cancellations of elective procedures, including treatment and diagnostic procedures, worldwide. For instance, a review article published in the Frontiers in Public Health Journal in April 2021 reported the challenges faced by patients affected by rare diseases. The article concluded that regular clinical services, counseling, and therapies were extremely inadequate. Additionally, clinicians, researchers, and scientists working on rare genetic diseases faced extra challenges due to the pandemic since research projects and clinical trials for rare and genetic diseases were suspended during the COVID-19 pandemic. However, the market has been recovering following the lifting of restrictions. The market is expected to show a stable growth rate during the forecast period.

In addition, the increase in the number of rare disease cases and initiatives favoring vaccinations, rising R&D activities for novel therapeutics and drugs, and an increase in the number of new drug launches and favorable government policies are actively affecting the growth of the market studied.

The definition of rare diseases has distinctions in different parts of the world. The United States Food and Drug Administration defines a rare disease as a disease or condition that affects less than 200,000 people in the United States. The European Commission Public Health states that a rare disease affects no more than one person in 2000 in the European Union. According to Health Canada, the rare disease affects less than five persons in 10,000 in Canada. According to an article published in March 2021, over 6,000 different rare diseases are thought to exist, and more than 70% of rare diseases are hereditary, and the majority of them have a chronic course. According to the February 2022 update of rarediseaseday.org, about 300 million people around

the world are living with rare diseases, which is quite high. Thus, the demand for effective and advanced treatment solutions for rare diseases is expected to increase due to the high burden and growing awareness about these diseases. This is expected to fuel growth in the market studied over the forecast period.

Various governments around the world have been formulating several initiatives for the treatment of rare diseases. For instance, the Indian government's Ministry of Health and Family Welfare's National Policy for Rare Diseases 2021 aimed at lowering the incidence and prevalence of rare diseases based on an integrated and comprehensive preventive strategy. This encompasses awareness generation, premarital, post-marital, pre-conception, and post-conception screening and counseling programs to prevent births of children with rare diseases, and within the constraints on resources and competing health care priorities, enable access to affordable health care to patients of rare diseases who are amenable to one-time treatment or relatively low-cost therapy.

Additionally, in May 2022, the Center for Drug Evaluation and Research (CDER) of the US FDA launched a new program for expediting the development of new treatment options for rare diseases, the Accelerating Rare disease Cures (ARC) Program. Such developments are expected to propel the market's growth during the forecast period.

Additionally, the innovation in treatment and increasing product approvals, along with partnerships and acquisitions by key players are helping in the market's growth. For instance, in May 2022, the European Medicines Agency (EMA) approved xenpozyme (olipudasealfa), a therapy for the treatment of non-central nervous system (CNS) manifestations of Acid Sphingomyelinase Deficiency (ASMD), a rare and progressive genetic disease. EMA specified that xenpozyme was the first ASMD-specific treatment in the European Union developed to replace patients' deficient or defective enzyme, acid sphingomyelinase (ASM), and thereby reduce fat accumulation within cells and relieve some of the symptoms of the disease. Such approvals are expected to augment the market's growth during the forecast period.

The increase in the necessity of rare disease treatment worldwide is expected to propel the growth of the rare disease treatment market. However, a lack of awareness regarding rare disease treatment and the high cost of treatment is expected to hinder the market's growth during the forecast period.

Rare Disease Treatment Market Trends

The Biologics Segment is Expected to Hold a Major Market Share in the Rare Disease Treatment Market

Biologics are the drugs that target specific parts of the immune system to treat disease. Biologics are proteins, antibodies, peptides, and some vaccines that are usually injected or infused because they cannot be absorbed orally.

According to the Regulatory Affairs Professionals Society (RAPS), 26 of 50 drugs (52%) in 2021 were approved as orphan drugs for rare diseases. In previous years, the proportion of orphan drugs approved has been inconsistent, with FDA identifying 31 of 53 drugs (58.4%) in 2020 as having been approved by the Center for Drug Evaluation and Research (CDER) as orphan drugs.

An article published in the MDPI Journal of Biologics in August 2021 studied the main biological treatments currently available for inflammatory bowel disease treatment due to the extremely important current role of biologics. The study showed that various biologic therapies, such as anti-TNF-? therapy, anti-integrin therapy, anti-cytokine therapy, and other new biologics, are widely used to treat rare inflammatory bowel disease. The study indicated that further studies are needed to better understand the action mechanism of the biological drugs, which will help understand how to improve the efficacy and safety of the treatment methods.

The strategic initiatives adopted by key players, such as product launches and approvals, partnerships, and acquisitions, are also

fueling the segment's growth. For instance, in November 2022, CanSino Biologics Inc. entered into a strategic collaboration agreement with AstraZeneca to improve Chinese patients' access to the diagnosis, prevention, and treatment of rare diseases. The collaboration helps AstraZeneca develop the biopharmaceutical ecosystem and diagnose and treat rare diseases. Such collaborations among market players regarding innovations in biologics are expected to propel the segment's growth during the forecast period.

North America is Expected to Hold a Significant Share in the Rare Disease Treatment Market

The North American region is expected to occupy a major share in the rare disease treatment market owing to the high prevalence of rare diseases in the region, such as Huntington's disease, spina bifida, fragile X syndrome, Guillain-Barre syndrome, Crohn's disease, cystic fibrosis, and Duchenne muscular dystrophy, high awareness about these diseases, and the presence of a robust healthcare system for the diagnosis and treatment of rare diseases.

According to the Genetic and Rare Diseases (GARD) Information Center in May 2022, 1 in 10 Americans (or 30 million people) have a rare disease, and there are approximately 7,000 known rare diseases. In addition, according to the Canadian Organization for Rare Disorders (CORD) Report 2021, every year, about 1 in 12 people in Canada are affected by rare diseases, and about 80% of these diseases are caused due to genetic changes. Hence, owing to the high burden of rare diseases in the region, the market is expected to have significant growth over the forecast period.

Furthermore, in September 2022, the Critical Path for Rare Neurodegenerative Diseases (CP-RND), a public-private partnership aimed at fostering the development of treatments for amyotrophic lateral sclerosis (ALS) and other rare neurodegenerative diseases, was launched by the US Food and Drug Administration and the National Institutes of Health (NIH). Additionally, in October 2022, Takeda Canada Inc. released its report that highlighted the need to develop a single, pan-Canadian definition of a rare disease to help accelerate and support the launch of Canada's Rare Disease strategy. With this, Canada is taking a meaningful step ahead in developing a national strategy for rare diseases, which is expected to increase people's awareness about rare disease treatment and contribute to regional market growth.

Rare Disease Treatment Industry Overview

The rare disease treatment market is competitive and consists of several major players. In terms of market share, a few major players dominate the market. Some companies currently dominating the market are Amgen Inc., AstraZeneca (Alexion Pharmaceuticals Inc.), Bristol-Myers Squibb Company, Biomarin Pharmaceuticals, and Bayer AG, among others. Market players readily adopt multiple initiatives, such as mergers and acquisitions and product launches, to strengthen their position in the market.

Additional Benefits:

  The market estimate (ME) sheet in Excel format
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Table of Contents:

Rare Disease Treatment - Market Share Analysis, Industry Trends & Statistics, Growth Forecasts 2019 - 2029

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