

Global Rare Diseases Treatment Market Size study & Forecast, by Therapeutic Area (Cancer, Neurological Conditions, Cardiovascular Conditions, Musculoskeletal Conditions, Hematologic Disorders, Infectious Diseases, Metabolic disorders, Endocrine disorders, Others) by Route of Administration (Oral, Injectable, Others), by Drug Type (Biologics, Biosimilar, Small Molecule), by Distribution Channel (Hospital Pharmacy, Specialty Pharmacy, Online Pharmacy) and Regional Analysis, 2022-2029

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### **Abstracts**

Global Rare Diseases Treatment Market is valued at approximately USD 119.6 billion in 2021 and is anticipated to grow with a healthy growth rate of more than 12.8% over the forecast period 2022-2029. Rare Diseases are diseases that affect a small percentage of people. It is coded as ICD-11 by the world health organization, ICD-11 consists of around 5500 rare diseases. Rare disease can also be termed as an orphan disease, a condition that lacks a market size large enough to attract funding and resources for the development of remedies, barring the government from giving favorable economic terms for developing and commercializing such treatments. According to the US Food and Drug Administration, a rare disease is one that affects less than two lakh persons in the country. Increasing approval and launch of new orphan drugs, increasing prevalence of rare diseases, government support and initiatives for the treatment of rare diseases, increasing research and development in the sector, and favorable government policies are the factors contributing to the growth of the market.



According to the National Institutes of Health, One in ten Americans (about 30 million people) have a rare condition, and there are approximately 7,000 identified rare diseases in the US. Additionally, In January 2022, Amgen announced the approval of LUMAKRAS® drug in Japan for the treatment of rare cancer, and in October 2021, Scemblix drug was approved by the Food and Drug Administration for patients with chronic-phase (CP)-CML as a third line of treatment. Increasing approval and launch of new orphan drugs and increasing prevalence of rare diseases are the major factors driving the growth of the market. Furthermore, Increasing initiatives by the government and increasing awareness of rare diseases are the factors creating major market opportunities in the forecast period. However, the high cost of Rare Diseases Treatment stifles market growth throughout the forecast period of 2022-2029.

The key regions considered for the Global Rare Diseases Treatment Market study includes Asia Pacific, North America, Europe, Latin America, and Rest of the World. North America dominated the market in terms of revenue, owing to the dominance of rare diseases, easy accessibility to products, new product approval, and increasing government support and investment in the region. Asia Pacific is expected to grow with the highest CAGR during the forecast period, owing to factors such as rising government initiatives, increasing prevalence of orphan diseases, geographic expansion of key players, and active participation of government.

Major market player included in this report are:

F. Hoffmann-La Roche Ltd.

Pfizer, Inc

**PTC Therapeutics** 

AstraZeneca

Novartis AG

Novo Nordisk Pharmatech A/S

Takeda Pharmaceutical Company Limited

Bayer AG

Merck & Co., Inc.

Bristol Myers Squibb Company

### Recent Developments in the Market:

In July 2021, AstraZeneca acquired Alexion Pharmaceuticals, Inc. to enjoy a stronger scientific foothold in immunology and to continue to lead the way in the research and development of drugs for people with rare diseases through Alexion's cutting-edge complement-biology platform and a strong pipeline.

In November 2022, The FDA received a new Biologics License Application (BLA) for



PRX-102 (pegunigalsidasealfa) from ProtalixBiotherapeutics Inc. and Chiesi Global Rare Diseases for the treatment of adult Fabry disease patients.

Global Rare Diseases Treatment Market Report Scope:

Historical Data 2019-2020-2021

Base Year for Estimation 2021

Forecast period 2022-2029

Report Coverage Revenue forecast, Company Ranking, Competitive Landscape, Growth factors, and Trends

Segments Covered Therapeutic Area, Route of Administration, Drug Type, Distribution Channel, Region

Regional Scope North America; Europe; Asia Pacific; Latin America; Rest of the World Customization Scope Free report customization (equivalent up to 8 analyst's working hours) with purchase. Addition or alteration to country, regional & segment scope\*

The objective of the study is to define market sizes of different segments & countries in recent years and to forecast the values to the coming years. The report is designed to incorporate both qualitative and quantitative aspects of the industry within countries involved in the study.

The report also caters detailed information about the crucial aspects such as driving factors & challenges which will define the future growth of the market. Additionally, it also incorporates potential opportunities in micro markets for stakeholders to invest along with the detailed analysis of competitive landscape and Therapeutic Area offerings of key players. The detailed segments and sub-segment of the market are explained below:

By Therapeutic Area:

Cancer

**Neurological Conditions** 

Cardiovascular Conditions

Musculoskeletal Conditions

Hematologic Disorders

Infectious Diseases

Metabolic disorders

**Endocrine disorders** 

Others

By Drug Type:

**Biologics** 

Biosimilar



### Small Molecule

By Route of Administration:

Oral

Injectable

Others

By Distribution Channel:

**Hospital Pharmacy** 

**Specialty Pharmacy** 

Online Pharmacy

By Region:

North America

U.S.

Canada

Europe

UK

Germany

France

Spain

Italy

ROE

Asia Pacific

China

India

Japan

Australia

South Korea

**RoAPAC** 

Latin America

Brazil

Mexico

Rest of the World



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