

# **Rare Disease Therapeutics Market - Global Industry Size, Share, Trends, Opportunity, and Forecast, 2018-2028 Segmented By Therapeutic Area (Hematologic Diseases, Cancer, Infectious Diseases, Cardiovascular Diseases, Metabolic Diseases, Endocrine Diseases, Musculoskeletal Diseases, Others), By Route of Administration (Injectable, Oral, Others), By Drug Type (Biologics, Biosimilar, Small Molecules), By Distribution Channel (Specialty Pharmacies, Hospital Pharmacies, Online Pharmacies), By Region, By Competition.**

<https://marketpublishers.com/r/R27D253C8D69EN.html>

Date: October 2023

Pages: 170

Price: US\$ 4,900.00 (Single User License)

ID: R27D253C8D69EN

## **Abstracts**

Global Rare Disease Therapeutics Market has valued at USD 98.61 billion in 2022 and is anticipated to reach a CAGR of 10.42% through 2028. The global rare disease therapeutic market is a dynamic and increasingly significant sector in the realm of healthcare. Rare diseases, also known as orphan diseases, are characterized by their low prevalence in the population, affecting a limited number of individuals. Despite their rarity, collectively, rare diseases impact millions of people worldwide. This market is defined by its focus on developing and providing specialized treatments for these often life-altering and life-threatening conditions.

One of the key drivers of this market's growth is the advances in genetic research and understanding. As scientists delve deeper into the genetic underpinnings of rare diseases, they uncover potential targets for innovative therapies, including gene therapies and precision medicine approaches. These breakthroughs have opened new

avenues for therapeutic development and offer renewed hope for patients with previously untreatable rare diseases. Regulatory incentives, such as the Orphan Drug Act in the United States and equivalent laws in other regions, have played a pivotal role in spurring investment in rare disease therapeutics. These incentives provide financial and regulatory advantages to pharmaceutical companies and researchers focused on developing treatments for rare diseases, making it a more attractive endeavor. Furthermore, increased awareness of rare diseases, driven by patient advocacy groups and healthcare organizations, has led to greater recognition of the unmet medical needs within this space. This heightened awareness has resulted in more resources being directed toward rare disease research and treatment development.

Targeted therapies designed specifically for rare diseases are another driving force behind market growth. These therapies are tailored to the unique genetic and molecular characteristics of the disease, promising improved efficacy and fewer side effects compared to traditional treatments. Expanding patient populations, as more rare disease cases are identified and diagnosed, further amplify the market's significance. Moreover, gene therapy advancements, characterized by innovative approaches like CRISPR-Cas9, have the potential to revolutionize rare disease treatment by directly addressing the genetic causes of these conditions.

Collaborations between academia and industry, as well as the involvement of global healthcare initiatives, are fostering a collaborative environment that accelerates rare disease therapeutic development. The market for rare disease therapeutics, driven by scientific progress, regulatory support, and a growing commitment to addressing the needs of rare disease patients, continues to evolve and expand, offering renewed hope and improved quality of life for those affected by these conditions.

## Key Market Drivers

### Rising Number of Patients with Rare Diseases

There are a lot of rare diseases in the medical world. Some are well-known, like cystic fibrosis or cat eye syndrome, while others are more obscure. Most cancers are rare and there are rare diseases of the brain and nervous system, metabolic diseases, chromosome disorders, skin, bone and skeletal, and diseases that affect the heart, blood vessels, lungs, kidneys and other organs and systems. A lot of rare diseases are named after the doctors who first diagnosed them, while others are named after patients or even the hospitals where they were first diagnosed.

An article published in the Journal of Rare Disorders in 2020 estimates that approximately 7,000 rare diseases have been diagnosed, with approximately 70% of them having no treatment available. As a result, research into unmet need of rare disease treatments will ensure that the market continues to expand in the future.

It is estimated that 6% to 8% of the population of the European Union is afflicted with a rare disease, as reported by EURORDIS. Furthermore, EURORDIS states that between 50-60% of rare diseases begin in infancy, and some of these conditions can be fatal. Consequently, it is expected that in the coming years, the demand for certain therapeutic drugs will rise significantly due to the growing pediatric population affected by rare diseases.

### Initiatives taken by Government and Market Players

The growth of the market is expected to be supported by government regulations that promote the development of products, such as the orphan drug law, which allocates the classification of an orphan drug to drug candidates developed by pharmaceutical companies. This program facilitates the treatment of orphan diseases through the advancement of research and development.

Additionally, in 2021, Center for Drug Evaluation and Research (CDER) approved about 26 new orphan drugs to treat rare diseases. Some of these new orphan drugs include Lumakras, Scelendrix, Welireg, Amondys, Cytalux, Besremi, Empavelli, Evkeeza, Exkivity, fexinodazole, and Zymonta.

The COVID-19 “Critical Relief” program was launched in April 2020 by the National Policy for rare disorders (NPRD) for patients with rare diseases affected by the pandemic. Under this program, the NPRD provided orphan disease patients and guardians with financial assistance of up to USD 1,000 per year to cover their non-medical needs. These programs are expected to increase the adoption of rare disease treatment in the coming years.

The Indian government's National Policy on ‘Rare Diseases 2021’ was designed to reduce the prevalence and incidence of rare diseases through an integrated and comprehensive prevention strategy. This includes awareness generation, pre-marriage, post-marriage, pre-pregnancy, and post-pregnancy screening and counselling programs to prevent the birth of children with rare conditions, and, within the limitations of resources and conflicting health care priorities, to provide affordable health care to those with rare diseases who are eligible for one-time treatments or relatively

inexpensive therapy.

In 2020, the U.S. FDA approved drugs to treat a disease with bladder dysfunction called neurogenic detrusor overactivity, observed in patients with spinal cord injury and multiple sclerosis.

In May 2022, the U.S. Food and Drug Administration (FDA) created a new program to accelerate the development of new treatments for rare diseases called the “Accelerating Rare Disease Cures” (ARC) Program, which is managed by the Center for Drug Evaluation and Research (CDER).

In May 2022, the European Medicines Agency (EMA) granted a marketing authorization for Xenpozyme (olipudasealfa), a therapy for the treatment of Non-Central Nervous System (NCS), manifestations of Acid Sphingomyelinase Deficiency (ASMD), a rare and degenerative genetic disorder.

### Increasing Demand of Non-Biological Drugs to Treat Rare Diseases

Non-biologic drugs for rare diseases are therapeutic agents that have been chemically synthesized or produced through conventional pharmaceutical processes, rather than being obtained from biological sources. These drugs are commonly referred to as small molecule drugs and are the most widely used and recognized drug class in medicine.

Non-biologics drug segment is expected to grow at an impressive rate during the forecast period, on account of the immunogenicity and minimal side effects associated with such products. These drugs are deemed effective for treating certain conditions including APDS (activated phosphoinositide 3-kinase delta syndrome) and Felty Syndrome. These factors, alongside cost-effectiveness and non-complexity in clinical studies, are projected to augment the consumption of non-biologic medications among rare disease patients.

Non-biological medicinal products are generally composed of molecules with well-characterized chemical structure. These molecules are usually synthesized in the laboratory through chemical reactions. Most non-biological medications are orally administered, i.e., taken by mouth in tablet, capsule, or liquid form. This is a convenient and self-administered route of administration for patients.

Non-biologic drugs have the potential to address a broad range of molecular and cellular pathways and are employed in the treatment of a variety of medical conditions,

such as rare diseases. Non-biological drugs are known to be highly stable and can be kept at room temperature without the need for refrigeration. Furthermore, the manufacturing process for these drugs is well-established, making them more suitable for large-scale production.

Once the patent protection of a non-biologic drug has expired, other pharmaceutical companies can produce and sell generic versions of the drug, thus increasing its availability and affordability.

Small molecule drugs are capable of functioning as inhibitors of enzymes that are involved in the process of disease. For example, imatinib is used to treat certain forms of leukemia and gastroenterological stromal tumors. Synthetic hormones are also used to treat rare endocrine disorders, such as hypothyroidism, with the aid of levothyroxine, and adrenal insufficiencies with the aid of hydrocortisone.

Certain small molecule drugs have the potential to modulate the immune system response, thus making them useful for the treatment of rare autoimmune conditions.

### Growing Burden of CNS disorders can Drive the Development of Precision Treatments for Rare Diseases

As the incidence and prevalence of rare diseases in the central nervous system (CNS) increase, there is an amplified need to gain a better understanding of the genetic, molecular, and biochemical substrates of these diseases. Genetics and neuroscience have enabled researchers to gain a greater understanding of the complexities of rare CNS disorders, allowing for the identification of disease-inducing mutations and pathways.

Rare disease treatment market value from the central nervous system is expected to accelerate in the forecast period, given the rising prevalence of disorders that affect nerve cells in the spinal cord and brain, causing loss of muscle control. Based on data from the National Library of Medicine, amyotrophic lateral sclerosis affects around 5 in 100,000 people worldwide. Moreover, the strong focus on the development of precision medicine will further drive the demand for therapies to treat rare diseases.

Precision medicine is a form of medical treatment that is tailored to the unique needs of each individual patient, considering factors such as genetics, lifestyle, and environment. In rare diseases of the central nervous system (CNS), where each condition is caused by a distinct genetic mutation, targeted therapies are becoming increasingly feasible.

Understanding the genetic makeup of a patient's disorder allows clinicians to design targeted therapies that address specific molecular defects.

Precision medicine is heavily reliant on biomarkers. These are things that can be measured to show the presence or progress of a disease. Developing precision treatments for rare diseases of the central nervous system often involves finding and testing biomarkers related to certain disorders. These biomarkers can help early diagnosis, track disease progress, and measure how well treatment is working.

As treatment options for rare CNS diseases are limited, precision medicine approaches involve examining the potential of drugs that have been developed for other conditions to treat a rare disease. By elucidating the molecular pathways associated with both the rare condition and other common conditions, researchers may be able to identify drugs that could potentially treat the rare CNS disorder.

The prevalence of rare diseases in the central nervous system (CNS) has prompted researchers and pharmaceutical manufacturers to pay greater attention to these conditions. The development of precision medicine techniques has made it simpler to conduct specific research on rare diseases, thus accelerating the development of drugs and overall market growth.

## Key Market Challenges

### Accurate Diagnosis

Finding a diagnosis is the key to successful treatment and, in some cases, clinical trial involvement. However, accurate and timely diagnosis of a rare condition can be challenging for patients and in some cases an accurate diagnosis may take up to five years.

In rare cases, patients may present signs and symptoms that a medical professional may not be able to identify as being related to or caused by the disease. In such cases, extensive genetic testing may be conducted to identify genetic variations that may be associated with the manifestation of the disease. It is always beneficial to ensure that the patient is either assigned to a clinical geneticist or/or a physician who is familiar with the difficulties associated with interpreting genetic data.

### Patient Availability and Recruitment



Recruiting patients to participate in clinical trials in the field of orphan medicinal products is a challenging task. Not only is it challenging to determine the target patient population, but also finding investigators who specialize in treating these patients is challenging. These difficulties may pose a recruitment risk and necessitate a customized feasibility approach to reduce risk for each rare disease study.

Because the patient population is likely to be geographically distributed, patients may be required to travel considerable distances to access the study site for an orphan disease clinical trial. While travel reimbursement could potentially reduce this risk, organizations should also explore novel solutions and technologies to collect data from home and minimize site visits.

### Endpoint Selection

When designing therapeutics for rare conditions, it is important to ensure that clinical study endpoints measure how the drug changes the progression of patient's disease. To distinguish a drug effect from standard disease progression, clinical studies and endpoint design must adequately reflect regulatory requirements and payer considerations.

It is recommended that sponsors collaborate with regulatory agencies to determine efficacy endpoints prior to the clinical study. The Food and Drug Administration organizes disease-specific forums to identify and discuss endpoints that are relevant from the patient or caregiver's point of view; these endpoints may include verified patient-reported outcomes that include the patient's voice. Rare disease often requires participation from numerous sites across many regions

### Key Market Trends

#### Growing Product Innovation

Rising demand for rare diseases has led to the development of various therapies for the disease. For instance, in February 2023, Sanofi is planning to launch two of its medicines for rare diseases in India early this year or next year. The company has been recommended by the Subject Expert Committee for the import and placing on the market of the two novel products in India: Nexviazyme (AveloglucoSidase Alfa powder) for the treatment of Pompe disease and Xenpozyme and Olipudase Alfa powder, for the treatment of Niemann Pick disease (ASMD). According to a company note, both products have orphan drug status and are approved in several countries including the

United States, Australia, the European Union, the United Kingdom, and Japan. In addition, the company has waivers for late-stage clinical trials (Phase III and IV).

### Advancements In Precision Medicine (PPM), Big Data, and Genomics

There are no indications that the rare disease space will experience a decline in popularity in the future, as the current drug development landscape is well-suited to the emerging field of medicine. The development of Personalized Precision Medicine (PPM), Big Data, and Genomics has enabled a significant improvement in the targeting of challenging diagnostic and therapeutic forms of disease, while a better comprehension of the genetic substrates of pathologies has enabled the delineation and targeting of rare disorders

### Regulatory Game-Changers

The 1983 enactment of the United States Orphan Drug Act is widely regarded as the turning point in the history of rare disease drug development. The legislation sought to address market failure by providing incentives to pharmaceutical companies that would otherwise be reluctant to invest in a drug that could potentially benefit only a small number of patients. According to pharmaceutical trade organization (PhRMA), more than 600 orphan drugs have received FDA approval since Orphan Drug Act's inception, yet only 5 percent of rare conditions have an FDA-approved treatment, meaning there's still plenty of untapped growth potential for years to come. For instance, in 2022, FDA announced the launch of the new Accelerating Rare Disease Cure (ARC) Program.

### Companies Launching Initiative Programs

There are various initiatives being taken by companies to help people living with rare diseases. For instance, in 2022, Horizon launched new support initiatives for adults and teens living with rare disease. Being a rare disease sufferer can be challenging and lonely. To provide relief to the estimated 500-600 individuals living with cystic fibrosis in the United States, Horizon Therapeutic launched two distinct initiatives. Additionally in 2021, Horizon Therapeutics announced the launch of monthly virtual storytelling experience, #RAREis, to highlight the voices and stories of the community of children living with rare diseases and to raise awareness and funds for Make-A-Wish Mid-Atlantic and Make-A-Wish Canada. This photobooth is part of Horizon's global mission to support the wishes of children living with life-altering rare diseases and to bring smiles to their faces during a difficult time. Further, in support for the rare disease community, Horizon's U.S headquarters in Deerfield, Illinois and global headquarters in



Dublin, Ireland was lit in purple, pink, green, and blue, the official colors of rare disease day.

## Segmental Insights

### Therapeutic Area Insights

Hematologic diseases, which encompass a wide range of conditions affecting the blood and related tissues, hold a dominant position in the global rare disease therapeutic market for several compelling reasons. Firstly, these diseases collectively represent a significant proportion of rare diseases globally, making them a substantial part of the market. Examples include sickle cell anemia, hemophilia, and various rare blood disorders.

Secondly, advancements in research and therapeutic development have propelled the emergence of novel treatments and therapies for hematologic rare diseases. Gene therapy and precision medicine approaches, in particular, have shown promising results in addressing previously untreatable or poorly managed conditions. This therapeutic innovation has attracted substantial investment and interest from pharmaceutical companies and researchers. Furthermore, hematologic rare diseases often have a significant impact on patients' quality of life and longevity, driving both medical and societal urgency for effective treatments. This sense of urgency fosters collaborations among healthcare providers, researchers, and advocacy groups, further accelerating therapeutic development efforts.

Lastly, regulatory agencies, recognizing the unmet medical need in hematologic rare diseases, have implemented expedited approval pathways, facilitating quicker access to therapies. These factors collectively make hematologic rare diseases a dominant force in the global rare disease therapeutic market, driving research, innovation, and improved patient outcomes.

### Route of Administration Insights

Injectables hold a dominant position in the global rare disease therapeutic market for several compelling reasons. Firstly, many rare diseases are characterized by complex pathophysiology and require precise and controlled drug delivery, making injectable formulations an optimal choice. This route of administration ensures that the medication reaches the bloodstream rapidly, allowing for more immediate therapeutic effects, crucial in managing severe or life-threatening rare diseases.

Secondly, injectables are often preferred in cases where oral or other delivery methods may be less effective due to issues like poor absorption, gastrointestinal complications, or the need for high bioavailability. In the context of rare diseases, where treatment options are limited, injectables provide a reliable means of delivering therapies directly to the bloodstream, optimizing their efficacy. Furthermore, regulatory agencies have recognized the importance of injectables in the treatment of rare diseases by expediting approvals for innovative therapies. This has encouraged pharmaceutical companies and researchers to focus on developing injectable treatments for rare diseases, driving further advancements and investments in this area.

Lastly, the high specificity and targeted delivery achievable with injectables make them a favored choice for therapies, including enzyme replacement therapies and gene therapies, which are essential in managing many rare diseases. As a result, injectables continue to dominate the global rare disease therapeutic market, offering hope and improved outcomes for patients with these challenging conditions.

## Regional Insights

North America occupies a dominant position in the global rare disease therapeutic market for several compelling reasons. Firstly, the region benefits from a robust healthcare infrastructure and advanced research facilities, which foster a fertile ground for the development and adoption of rare disease treatments. North America boasts a wealth of renowned medical institutions, pharmaceutical companies, and research organizations dedicated to addressing rare diseases.

Secondly, the region's commitment to innovation is exemplified by its significant investments in research and development. Government agencies, private foundations, and pharmaceutical companies in North America allocate substantial funding towards rare disease research and therapeutic development, incentivizing the creation of cutting-edge treatments. Additionally, North America possesses a sizeable patient population, making it an attractive market for pharmaceutical companies to invest in rare disease therapies. The Orphan Drug Act in the United States, for instance, provides incentives for drug developers to focus on rare diseases, further stimulating research and commercialization efforts.

Furthermore, North America's regulatory environment is conducive to rare disease therapeutic development, with expedited approval processes and orphan drug designations facilitating quicker access to innovative treatments. These factors,

combined with a culture of medical innovation and research excellence, solidify North America's dominant role in the global rare disease therapeutic market.

### Key Market Players

Novartis AG

AstraZeneca PLC

Pfizer Inc.

Sanofi SA

AbbVie Inc

Bristol-Myers Squibb Co

Bayer AG

F Hoffmann-La Roche Ltd.

Amgen Inc

Eisai Co Ltd

Novo Nordisk A/S

### Report Scope:

In this report, the Global Rare Disease Therapeutics Market has been segmented into the following categories, in addition to the industry trends which have also been detailed below:

#### Rare Disease Therapeutics Market, By Therapeutic Area:

Hematologic Diseases

Cancer

Infectious Diseases

Cardiovascular Diseases

Metabolic Diseases

Endocrine Diseases

Musculoskeletal Diseases

Others

Rare Disease Therapeutics Market, By Route of Administration:

Injectable

Oral

Others

Rare Disease Therapeutics Market, By Drug Type:

Biologics

Biosimilar

Small Molecules

Rare Disease Therapeutics Market, By Distribution Channel:

Specialty Pharmacies

Hospital Pharmacies

Online Pharmacies

Rare Disease Therapeutics Market, By Region:

North America

United States

Canada

Mexico

Europe

France

United Kingdom

Italy

Germany

Spain

Asia-Pacific

China

India

Japan

Australia

South Korea

South America

Brazil

Argentina

Colombia

Middle East & Africa

South Africa

Saudi Arabia

UAE

Competitive Landscape

Company Profiles: Detailed analysis of the major companies present in the Rare Disease Therapeutics Market.

Available Customizations:

Global Rare Disease Therapeutics market report with the given market data, Tech Sci Research offers customizations according to a company's specific needs. The following customization options are available for the report:

Company Information

Detailed analysis and profiling of additional market players (up to five).



## Contents

### **1. PRODUCT OVERVIEW**

- 1.1. Market Definition
- 1.2. Scope of the Market
  - 1.2.1. Markets Covered
  - 1.2.2. Years Considered for Study
  - 1.2.3. Key Market Segmentations

### **2. RESEARCH METHODOLOGY**

- 2.1. Objective of the Study
- 2.2. Baseline Methodology
- 2.3. Key Industry Partners
- 2.4. Major Association and Secondary Sources
- 2.5. Forecasting Methodology
- 2.6. Data Triangulation & Validations
- 2.7. Assumptions and Limitations

### **3. EXECUTIVE SUMMARY**

- 3.1. Overview of the Market
- 3.2. Overview of Key Market Segmentations
- 3.3. Overview of Key Market Players
- 3.4. Overview of Key Regions/Countries
- 3.5. Overview of Market Drivers, Challenges, Trends

### **4. VOICE OF CUSTOMER**

### **5. GLOBAL RARE DISEASE THERAPEUTICS MARKET OUTLOOK**

- 5.1. Market Size & Forecast
  - 5.1.1. By Value
- 5.2. Market Share & Forecast
  - 5.2.1. By Therapeutic Area (Hematologic Diseases, Cancer, Infectious Diseases, Cardiovascular Diseases, Metabolic Diseases, Endocrine Diseases, Musculoskeletal Diseases, Others)
  - 5.2.2. By Route of Administration (Injectable, Oral, Others)

- 5.2.3. By Drug Type (Biologics, Biosimilar, Small Molecules)
- 5.2.4. By Distribution Channel (Specialty Pharmacies, Hospital Pharmacies, Online Pharmacies)
- 5.2.5. By Region
- 5.2.6. By Company (Shares of Top 5 Market Players)
- 5.3. Market Map
  - 5.3.1. By Therapeutic Area
  - 5.3.2. By Route of Administration
  - 5.3.3. By Drug Type
  - 5.3.4. By Distribution Channel
  - 5.3.5. By Region

## **6. NORTH AMERICA RARE DISEASE THERAPEUTICS MARKET OUTLOOK**

- 6.1. Market Size & Forecast
  - 6.1.1. By Value
- 6.2. Market Share & Forecast
  - 6.2.1. By Therapeutic Area
  - 6.2.2. By Route of Administration
  - 6.2.3. By Drug Type
  - 6.2.4. By Distribution Channel
  - 6.2.5. By Country
    - 6.2.5.1. United States Rare Disease Therapeutics Market Outlook
      - 6.2.5.1.1. Market Size & Forecast
        - 6.2.5.1.1.1. By Value
      - 6.2.5.1.2. Market Share & Forecast
        - 6.2.5.1.2.1. By Therapeutic Area
        - 6.2.5.1.2.2. By Route of Administration
        - 6.2.5.1.2.3. By Drug Type
        - 6.2.5.1.2.4. By Distribution Channel
    - 6.2.5.2. Canada Rare Disease Therapeutics Market Outlook
      - 6.2.5.2.1. Market Size & Forecast
        - 6.2.5.2.1.1. By Value
      - 6.2.5.2.2. Market Share & Forecast
        - 6.2.5.2.2.1. By Therapeutic Area
        - 6.2.5.2.2.2. By Route of Administration
        - 6.2.5.2.2.3. By Drug Type
        - 6.2.5.2.2.4. By Distribution Channel
    - 6.2.5.3. Mexico Rare Disease Therapeutics Market Outlook

- 6.2.5.3.1. Market Size & Forecast
  - 6.2.5.3.1.1. By Value
- 6.2.5.3.2. Market Share & Forecast
  - 6.2.5.3.2.1. By Therapeutic Area
  - 6.2.5.3.2.2. By Route of Administration
  - 6.2.5.3.2.3. By Drug Type
  - 6.2.5.3.2.4. By Distribution Channel

## **7. EUROPE RARE DISEASE THERAPEUTICS MARKET OUTLOOK**

- 7.1. Market Size & Forecast
  - 7.1.1. By Value
- 7.2. Market Share & Forecast
  - 7.2.1. By Therapeutic Area
  - 7.2.2. By Route of Administration
  - 7.2.3. By Drug Type
  - 7.2.4. By Distribution Channel
  - 7.2.5. By Country
    - 7.2.5.1. France Rare Disease Therapeutics Market Outlook
      - 7.2.5.1.1. Market Size & Forecast
        - 7.2.5.1.1.1. By Value
      - 7.2.5.1.2. Market Share & Forecast
        - 7.2.5.1.2.1. By Therapeutic Area
        - 7.2.5.1.2.2. By Route of Administration
        - 7.2.5.1.2.3. By Drug Type
        - 7.2.5.1.2.4. By Distribution Channel
    - 7.2.5.2. Germany Rare Disease Therapeutics Market Outlook
      - 7.2.5.2.1. Market Size & Forecast
        - 7.2.5.2.1.1. By Value
      - 7.2.5.2.2. Market Share & Forecast
        - 7.2.5.2.2.1. By Therapeutic Area
        - 7.2.5.2.2.2. By Route of Administration
        - 7.2.5.2.2.3. By Drug Type
        - 7.2.5.2.2.4. By Distribution Channel
    - 7.2.5.3. United Kingdom Rare Disease Therapeutics Market Outlook
      - 7.2.5.3.1. Market Size & Forecast
        - 7.2.5.3.1.1. By Value
      - 7.2.5.3.2. Market Share & Forecast
        - 7.2.5.3.2.1. By Therapeutic Area

- 7.2.5.3.2.2. By Route of Administration
- 7.2.5.3.2.3. By Drug Type
- 7.2.5.3.2.4. By Distribution Channel
- 7.2.5.4. Italy Rare Disease Therapeutics Market Outlook
  - 7.2.5.4.1. Market Size & Forecast
    - 7.2.5.4.1.1. By Value
  - 7.2.5.4.2. Market Share & Forecast
    - 7.2.5.4.2.1. By Therapeutic Area
    - 7.2.5.4.2.2. By Route of Administration
    - 7.2.5.4.2.3. By Drug Type
    - 7.2.5.4.2.4. By Distribution Channel
- 7.2.5.5. Spain Rare Disease Therapeutics Market Outlook
  - 7.2.5.5.1. Market Size & Forecast
    - 7.2.5.5.1.1. By Value
  - 7.2.5.5.2. Market Share & Forecast
    - 7.2.5.5.2.1. By Therapeutic Area
    - 7.2.5.5.2.2. By Route of Administration
    - 7.2.5.5.2.3. By Drug Type
    - 7.2.5.5.2.4. By Distribution Channel

## **8. ASIA PACIFIC RARE DISEASE THERAPEUTICS MARKET OUTLOOK**

- 8.1. Market Size & Forecast
  - 8.1.1. By Value
- 8.2. Market Share & Forecast
  - 8.2.1. By Therapeutic Area
  - 8.2.2. By Route of Administration
  - 8.2.3. By Drug Type
  - 8.2.4. By Distribution Channel
  - 8.2.5. By Country
    - 8.2.5.1. China Rare Disease Therapeutics Market Outlook
      - 8.2.5.1.1. Market Size & Forecast
        - 8.2.5.1.1.1. By Value
      - 8.2.5.1.2. Market Share & Forecast
        - 8.2.5.1.2.1. By Therapeutic Area
        - 8.2.5.1.2.2. By Route of Administration
        - 8.2.5.1.2.3. By Drug Type
        - 8.2.5.1.2.4. By Distribution Channel
    - 8.2.5.2. India Rare Disease Therapeutics Market Outlook

- 8.2.5.2.1. Market Size & Forecast
  - 8.2.5.2.1.1. By Value
- 8.2.5.2.2. Market Share & Forecast
  - 8.2.5.2.2.1. By Therapeutic Area
  - 8.2.5.2.2.2. By Route of Administration
  - 8.2.5.2.2.3. By Drug Type
  - 8.2.5.2.2.4. By Distribution Channel
- 8.2.5.3. South Korea Rare Disease Therapeutics Market Outlook
  - 8.2.5.3.1. Market Size & Forecast
    - 8.2.5.3.1.1. By Value
  - 8.2.5.3.2. Market Share & Forecast
    - 8.2.5.3.2.1. By Therapeutic Area
    - 8.2.5.3.2.2. By Route of Administration
    - 8.2.5.3.2.3. By Drug Type
    - 8.2.5.3.2.4. By Distribution Channel
- 8.2.5.4. Japan Rare Disease Therapeutics Market Outlook
  - 8.2.5.4.1. Market Size & Forecast
    - 8.2.5.4.1.1. By Value
  - 8.2.5.4.2. Market Share & Forecast
    - 8.2.5.4.2.1. By Therapeutic Area
    - 8.2.5.4.2.2. By Route of Administration
    - 8.2.5.4.2.3. By Drug Type
    - 8.2.5.4.2.4. By Distribution Channel
- 8.2.5.5. Australia Rare Disease Therapeutics Market Outlook
  - 8.2.5.5.1. Market Size & Forecast
    - 8.2.5.5.1.1. By Value
  - 8.2.5.5.2. Market Share & Forecast
    - 8.2.5.5.2.1. By Therapeutic Area
    - 8.2.5.5.2.2. By Route of Administration
    - 8.2.5.5.2.3. By Drug Type
    - 8.2.5.5.2.4. By Distribution Channel

## **9. SOUTH AMERICA RARE DISEASE THERAPEUTICS MARKET OUTLOOK**

- 9.1. Market Size & Forecast
  - 9.1.1. By Value
- 9.2. Market Share & Forecast
  - 9.2.1. By Therapeutic Area
  - 9.2.2. By Route of Administration

9.2.3. By Drug Type

9.2.4. By Distribution Channel

9.2.5. By Country

9.2.5.1. Brazil Rare Disease Therapeutics Market Outlook

9.2.5.1.1. Market Size & Forecast

9.2.5.1.1.1. By Value

9.2.5.1.2. Market Share & Forecast

9.2.5.1.2.1. By Therapeutic Area

9.2.5.1.2.2. By Route of Administration

9.2.5.1.2.3. By Drug Type

9.2.5.1.2.4. By Distribution Channel

9.2.5.2. Argentina Rare Disease Therapeutics Market Outlook

9.2.5.2.1. Market Size & Forecast

9.2.5.2.1.1. By Value

9.2.5.2.2. Market Share & Forecast

9.2.5.2.2.1. By Therapeutic Area

9.2.5.2.2.2. By Route of Administration

9.2.5.2.2.3. By Drug Type

9.2.5.2.2.4. By Distribution Channel

9.2.5.3. Colombia Rare Disease Therapeutics Market Outlook

9.2.5.3.1. Market Size & Forecast

9.2.5.3.1.1. By Value

9.2.5.3.2. Market Share & Forecast

9.2.5.3.2.1. By Therapeutic Area

9.2.5.3.2.2. By Route of Administration

9.2.5.3.2.3. By Drug Type

9.2.5.3.2.4. By Distribution Channel

## **10. MIDDLE EAST & AFRICA RARE DISEASE THERAPEUTICS MARKET OUTLOOK**

10.1. Market Size & Forecast

10.1.1. By Value

10.2. Market Share & Forecast

10.2.1. By Therapeutic Area

10.2.2. By Route of Administration

10.2.3. By Drug Type

10.2.4. By Distribution Channel

10.2.5. By Country



#### 10.2.5.1. South Africa Rare Disease Therapeutics Market Outlook

##### 10.2.5.1.1. Market Size & Forecast

###### 10.2.5.1.1.1. By Value

##### 10.2.5.1.2. Market Share & Forecast

###### 10.2.5.1.2.1. By Therapeutic Area

###### 10.2.5.1.2.2. By Route of Administration

###### 10.2.5.1.2.3. By Drug Type

###### 10.2.5.1.2.4. By Distribution Channel

#### 10.2.5.2. Saudi Arabia Rare Disease Therapeutics Market Outlook

##### 10.2.5.2.1. Market Size & Forecast

###### 10.2.5.2.1.1. By Value

##### 10.2.5.2.2. Market Share & Forecast

###### 10.2.5.2.2.1. By Therapeutic Area

###### 10.2.5.2.2.2. By Route of Administration

###### 10.2.5.2.2.3. By Drug Type

###### 10.2.5.2.2.4. By Distribution Channel

#### 10.2.5.3. UAE Rare Disease Therapeutics Market Outlook

##### 10.2.5.3.1. Market Size & Forecast

###### 10.2.5.3.1.1. By Value

##### 10.2.5.3.2. Market Share & Forecast

###### 10.2.5.3.2.1. By Therapeutic Area

###### 10.2.5.3.2.2. By Route of Administration

###### 10.2.5.3.2.3. By Drug Type

###### 10.2.5.3.2.4. By Distribution Channel

## 11. MARKET DYNAMICS

### 11.1. Drivers

### 11.2. Challenges

## 12. MARKET TRENDS & DEVELOPMENTS

### 12.1. Recent Development

### 12.2. Mergers & Acquisitions

### 12.3. Technology Launches

## 13. COMPETITIVE LANDSCAPE

### 13.1. Novartis AG

- 13.1.1. Business Overview
- 13.1.2. Treatment Offerings
- 13.1.3. Recent Developments
- 13.1.4. Key Personnel
- 13.1.5. SWOT Analysis
- 13.2. AstraZeneca PLC
- 13.3. Pfizer Inc
- 13.4. Sanofi SA
- 13.5. AbbVie Inc
- 13.6. Bristol-Myers Squibb Co
- 13.7. Bayer AG
- 13.8. F Hoffmann-La Roche Ltd
- 13.9. Amgen Inc
- 13.10. Eisai Co Ltd
- 13.11. Novo Nordisk A/S

## **14. STRATEGIC RECOMMENDATIONS**

## **15. ABOUT US & DISCLAIMER**

## I would like to order

Product name: Rare Disease Therapeutics Market - Global Industry Size, Share, Trends, Opportunity, and Forecast, 2018-2028 Segmented By Therapeutic Area (Hematologic Diseases, Cancer, Infectious Diseases, Cardiovascular Diseases, Metabolic Diseases, Endocrine Diseases, Musculoskeletal Diseases, Others), By Route of Administration (Injectable, Oral, Others), By Drug Type (Biologics, Biosimilar, Small Molecules), By Distribution Channel (Specialty Pharmacies, Hospital Pharmacies, Online Pharmacies), By Region, By Competition.

Product link: <https://marketpublishers.com/r/R27D253C8D69EN.html>

Price: US\$ 4,900.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

[info@marketpublishers.com](mailto:info@marketpublishers.com)

## Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/R27D253C8D69EN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:  
Last name:  
Email:  
Company:  
Address:  
City:  
Zip code:  
Country:  
Tel:  
Fax:  
Your message:

**\*\*All fields are required**

Customer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970