

Rare Diseases Treatment Market Size, Share & Trends Analysis Report By Therapeutic Area (Cancer, Musculoskeletal Conditions), By Route of Administration, By Drug Type, By Distribution Channel, By Region, And Segment Forecasts, 2022 - 2030

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Abstracts

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Rare Diseases Treatment Market Growth & Trends

The global rare diseases treatment market size is expected to reach USD 335.84 billion by 2030, according to a new study by Grand View Research Inc., expanding at a CAGR of 12.8% from 2022 to 2030. Increasing prevalence of rare diseases contributes to industry growth. According to the National Center for Advancing Translational Sciences (NCATS), over 30 million of the U.S. population is estimated to be affected by rare diseases in 2022. Increasing prevalence of people living with orphan diseases is anticipated to increase the sales and demand for orphan medicines over the forecast period.

The presence of supportive regulations by government authorities, such as the Orphan Drug Act that support product development by offering the orphan drug designation to potential drug candidates developed by pharmaceutical companies, is expected to support industry growth. This initiative boosts the research and development in the field of orphan disease treatment.

In April 2020, the National Organization for Rare Disorders (NORD) launched the

“COVID-19 critical relief” program for patients with rare diseases affected by the COVID-19 pandemic. Under this program, the NORD provided monetary assistance of up to USD 1,000 annually to orphan disease patients and their caregivers for fulfilling their medical and non-medical needs. Thus, the availability of such programs is anticipated to boost the adoption of rare disease treatment over the forecast period.

Moreover, companies have undertaken initiatives such as collaborations and partnerships for the development, manufacturing, and commercialization of products in the international market. For instance, in August 2020, Sarepta Therapeutics, Inc. entered into a collaboration with the University of Florida for the development of novel genetic medicines for the treatment of patients with various orphan diseases, including Duchenne Muscular Dystrophy (DMD).

However, a lack of accurate and early diagnosis of rare diseases can often present a challenge for orphan disease patients, and in some cases, an accurate diagnosis is not obtained for as long as five years. In addition, patient eligibility and recruitment of eligible patients for clinical trials for rare disease treatment may restrain the industry growth. Not only finding the target population is difficult but finding physicians who treat these patients is also complex, thus leading to delays in the new product approval and launch in the market.

Rare Diseases Treatment Market Report Highlights

By therapeutic area, cancer segment held the largest revenue share in 2021 due to high prevalence of cancer indications such as multiple myeloma, acute lymphoblastic leukemia, chronic myeloid leukemia, and esophageal cancer and wide availability of orphan drugs for the treatment of patients with these conditions

By route of administration, the injectable segment dominated the market in 2021 and is expected to witness significant growth owing to high safety, efficacy, and tolerability associated with this route of administration among rare disease patients

By drug type, the biologics segment dominated the market in 2021 due to the presence of supportive reimbursement policies and the higher efficacy of biologics as disease-modifying therapies for the treatment of rare diseases

By distribution channel, the specialty pharmacy segment dominated the market

in 2021 due to government initiatives to support specialty pharmacies that provide orphan drugs. For instance, in July 2022, Utilization Review Accreditation Commission (URAC) awarded Maxor Specialty Pharmacy with the rare diseases pharmacy center of excellence for providing high-quality orphan drugs to patients

Asia Pacific is expected to witness lucrative growth during the forecast period. The growth of the region can be attributed to the entry of new products into the region. According to Illness Challenged Foundation, China had approved new 87 orphan drugs for 47 rare diseases by the end of 2021

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