

Orphan Drug Market Poised for Growth as Demand for Rare Disease Treatments Rises

Discover insights into the global orphan drug market, driven by rising demand for treatments of rare diseases

VANCOUVER, BC, CANADA, June 28, 2024 /EINPresswire.com/ -- The global [orphan drug market](#) size was USD 154.20 Billion in 2022 and is expected to register a rapid revenue CAGR of 12.3 % during the forecast period. The global orphan drug market is experiencing significant growth driven

by rising occurrences of rare diseases and supportive regulatory frameworks worldwide. Orphan drugs, specifically designed to treat uncommon medical conditions affecting a small population, are gaining traction due to increased R&D investments and regulatory incentives.

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Market Drivers

The market's expansion is propelled by a surge in rare disease cases globally, with approximately 30 million Americans and 400 million people worldwide affected by over 7,000 identified rare diseases. Governments, particularly in developed countries, are offering regulatory incentives to encourage orphan drug development under laws like the Orphan Drug Act in the U.S. and similar regulations in Europe. This support aims to address the unmet medical needs of patients with rare disorders.

Market Developments

Recent advancements in biologics, including vaccines and monoclonal antibodies, dominate the orphan drug market, reflecting their efficacy in treating rare conditions. Major pharmaceutical



players are intensifying their focus on biologics, driving innovation and expanding therapeutic options for patients.

Market Challenges

Despite growth opportunities, the high cost of orphan drugs remains a significant barrier, particularly in developing regions where reimbursement challenges may limit patient access. This disparity underscores the need for sustainable pricing models to ensure broader affordability.

Segment Insights

Biologics: Biologics constitute the largest segment within the orphan drug market, supported by a diverse range of therapeutic options including immune modulators and growth factors. Regulatory approvals and ongoing clinical trials continue to bolster the segment's growth.

Oncology: The oncology segment, driven by advancements in cancer therapies and increased investment in rare cancer treatments, is anticipated to grow steadily. Recent FDA approvals, such as the designation for Evorpaccept in treating acute myeloid leukemia, highlight ongoing progress in rare cancer therapies.

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End-Use Insights

Hospital Pharmacies: Hospital pharmacies lead in orphan drug distribution, catering to the need for intravenous administration and specialized patient care. The segment benefits from the growing elderly population requiring chronic disease management, thus supporting market expansion.

Future Outlook

Looking ahead, the orphan drug market is poised for continued growth, supported by ongoing research initiatives, expanding therapeutic pipelines, and evolving regulatory landscapes. As innovations in biologics and targeted therapies accelerate, stakeholders are expected to collaborate more closely to address affordability challenges and ensure equitable access to orphan drugs worldwide.

Orphan Drug Top Companies and Competitive Landscape

The global orphan drug market is fragmented, with large and medium-sized players accounting for the majority of market revenue. Major players are deploying various strategies, entering into

mergers & acquisitions, strategic agreements & contracts, developing, testing, and introducing more effective products in the market.

Pfizer Inc.

AbbVie Inc.

Novartis AG

Amryt Pharma plc

Amgen Inc.

GSK plc.

JOHNSON & JOHNSON

Sanofi

Hoffmann-La Roche Ltd

Takeda Pharmaceutical Company Limited.

Teva Pharmaceutical Industries Ltd.

Bristol-Myers Squibb Company

Biogen

Merck & Co., Inc.

Jazz Pharmaceuticals, Inc,

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Orphan Drug Latest Industry News

On 24 April 24, 2023, Genentech, a member of the Roche Group announced three new partnerships focused on improving brain health outcomes. These industry-leading initiatives are designed to quantify and assess the complexity, economic impact and patient and care partner burden of brain health conditions and generate robust data around the nuanced dynamics of neurologic care.

On January 16, 2023, CARsgen Therapeutics Holdings Limited collaborated with Huadong Medicine (Hangzhou) Co., Ltd. for the commercialization of CARsgen's BCMA CAR-T product CT053 in mainland China. Under the terms of the agreement, CARsgen will receive an upfront payment of around USD 28 million and is eligible to receive regulatory and commercial milestone payments up to USD 140 million.

For the purpose of this report, Emergen Research has segmented the global orphan drug market on the basis, drug type, therapy end-use, and region:

Drug Type Outlook (Revenue, USD Billion; 2019-2032)

Biologics

Non-Biologics

Therapy Outlook (Revenue, USD Billion; 2019-2032)

Oncology

Hematology

Neurology

Infectious Diseases

Metabolic Disorders

Endocrinology

Immunology

Others

End-use Outlook (Revenue, USD Billion; 2019-2032)

Hospital Pharmacies

Retail Pharmacies

Online Pharmacies

Others

Regional Outlook (Revenue, USD Billion; 2019–2032)

North America

U.S.

Canada

Mexico

Europe

Germany

France

UK

Italy

Spain

Benelux

Rest of Europe

Asia Pacific

China

India

Japan

South Korea

Rest of APAC

Latin America

Brazil

Rest of LATAM

Middle East & Africa

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UAE

South Africa

Turkey

Rest of Middle East & Africa

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