

Demand for Rare Disease Pharmaceutical Service Market is forecasted to reach a value of US \$217.36 billion by 2029

The Business Research Company's Rare Disease Pharmaceutical Service Global Market Report 2025 – Market Size, Trends, And Global Forecast 2025-2034

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[/EINPresswire.com/](#) -- How Large Will The Rare Disease Pharmaceutical Service Market Be By 2025?

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The market size of the pharmaceutical service sector for rare diseases has seen a significant increase over the past few years. Predictions indicate that it is likely to expand from \$117.70 billion in 2024 to \$133.40 billion in 2025, experiencing a compound annual growth rate (CAGR) of

13.3%. The factors contributing to this growth during the historic period include heightened emphasis on genetic research, increased use of advanced diagnostic methods, a surge in patient registry numbers, enhanced partnerships between pharmaceutical and biotech firms, and a noticeable increase in the understanding of the incentives associated with orphan drugs.

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Anticipations are high for a robust expansion of the rare disease pharmaceutical service market in the upcoming years, with an estimated value of \$217.37 billion by 2029, reflecting a compound annual growth rate (CAGR) of

13.0%. This predicted evolution during the forecast timeline can be linked to the heightened incorporation of AI in the discovery of drugs, the escalating requirement for individualised treatment options, amplified investment in clinical trials concerning rare diseases, the rising application of real-world evidence in decision-making, and strengthened regulatory backing for therapies targeting rare diseases. Prominent trends likely to be witnessed throughout the forecast period are the evolution in AI-based drug discovery, integration of digital health platforms, technological progression in patient surveillance, progress in therapies guided by

biomarkers, and notable advancements in decentralized clinical trials.

Download a free sample of the rare disease pharmaceutical service market report:

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What Are The Major Driving Forces Influencing The Rare Disease Pharmaceutical Service Market Landscape?

The emergence of personalized medicine is set to trigger an upswing in the rare disease pharmaceutical service market. Tailoring medical care to a person's genetic blueprint, lifestyle, and environment, personalized medicine offers a sharpened and more potent method of treatment. Advances in genomics, biomarker studies, and data analytics, which can be adapted to an individual's genetic components, have propelled this shift. It leads to improved results, lessens side effects and caters to unaddressed patient necessities more accurately than conventional treatments. The Pharmaceutical Service facilitates personalized medicine by crafting targeted treatments, utilizing genetic and biomarker information, and offering bespoke treatment plans that cater to patient-specific needs. As per the Personalized Medicine Coalition, a nonprofit based in the US, the FDA sanctioned 16 innovative personalized treatments for rare disease sufferers in 2023, a substantial increase from the six approvals in 2022. This upsurge in personalized medicine underscores the expansion of the rare disease pharmaceutical service market.

Who Are The Top Players In The Rare Disease Pharmaceutical Service Market?

Major players in the Rare Disease Pharmaceutical Service Global Market Report 2025 include:

- Johnson & Johnson
- Merck & Co. Inc.
- Pfizer Inc.
- AbbVie Inc.
- Sanofi S.A.
- Novartis AG
- AstraZeneca PLC
- GlaxoSmithKline PLC (GSK)
- Takeda Pharmaceutical Company Limited
- Amgen Inc.

What Are The Prominent Trends In The Rare Disease Pharmaceutical Service Market?

Leading businesses in the [rare disease pharmaceutical service sector](#) are now focusing on bringing forward innovative treatment methods such as acetyllecine. The aim is to enhance the efficacy of treatments, improve patient results, and cater to unmet needs related to rare neurological and metabolic disorders. Acetyllecine, a modified form of amino acid (N-acetyl-L-leucine), serves as a therapeutic agent to reinforce motor function and neurological health, and maintain balance in impacted patients. For example, in September 2024, IntraBio, a biopharmaceutical organization from the US, achieved FDA approval for AQNEURSA. This was

the first exclusive therapy developed for Niemann-Pick Disease Type C, a rare and fatal neurodegenerative condition. This achievement holds great importance as the treatment illustrated quick and substantial improvement in neurological symptoms, providing a novel therapeutic choice for children and adults living with this uncommon disease.

Market Share And Forecast By Segment In The Global Rare Disease Pharmaceutical Service Market

The rare disease pharmaceutical service market covered in this report is segmented as

- 1) By Types: Drug Discovery, Clinical Trials, Other Types
- 2) By Application: Congenital And Genetic Diseases, Tumors And Cancer, Endocrine Diseases
- 3) By End User: Pharmaceutical Companies, Biotechnology Companies, Academic And Research Institutions, Hospitals And Specialty Clinics, Contract Research Organizations (CROs) And Contract Development And Manufacturing Organizations (CDMOs), Diagnostic Laboratories And Genetic Testing Centers

Subsegments:

- 1) By Drug Discovery: Target Identification And Validation, Preclinical Research And Toxicology Studies, Biomarker Discovery And Validation, Genomic And Proteomic Analysis, Computational Drug Design And Modeling, High-Throughput Screening (HTS), Lead Optimization And Candidate Selection
- 2) By Clinical Trials: Clinical Trial Design And Protocol Development, Patient Recruitment And Retention, Site Management And Monitoring, Regulatory Affairs And Compliance Support, Data Management And Biostatistics, Pharmacovigilance And Safety Monitoring, Real-World Evidence (RWE) And Post-Marketing Studies
- 3) By Other Types: Market Access And Pricing Strategy, Health Economics And Outcomes Research (HEOR), Regulatory Affairs And Compliance Services, Manufacturing And CMC (Chemistry, Manufacturing, and Controls) Support, Medical Affairs And Scientific Communication, Post-Marketing Surveillance, Consulting And Strategic Advisory

View the full rare disease pharmaceutical service market report:

<https://www.thebusinessresearchcompany.com/report/rare-disease-pharmaceutical-service-global-market-report>

Rare Disease Pharmaceutical Service Market Regional Insights

In 2024, North America dominated the rare disease pharmaceutical service market as the largest region. The Rare Disease Pharmaceutical Service Global Market Report 2025 includes several regions such as Asia-Pacific, Western Europe, Eastern Europe, South America, the Middle East, and Africa.

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