

Comprehensive Report on the Rare Disease Clinical Trials Market: Opportunities and Challenges

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

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/EINPresswire.com/ -- What Is The Expected Cagr For The [Rare Disease Clinical Trials Market Through 2025?](#)

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In the past few years, the market size for clinical trials targeting rare diseases has seen a quick expansion. It is projected to increase from \$12.06 billion in 2024 to about \$13.30 billion in 2025 with a compound annual growth rate (CAGR) of 10.3%. The growth during the historical period

can be explained by the growing awareness of genetic disorders among healthcare practitioners, boosted demand for specialized patient monitoring in trials, an uptick in the development of tailored medicine approaches, an expansion in the acceptance of patient-focused trial methodologies, and a rising need for international multi-center clinical studies.

Expectations are high for a swift expansion in the rare disease clinical trials market size in the upcoming years. By 2029, the market is projected to reach \$19.45 billion, with a

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substantial compound annual growth rate (CAGR) of 10.0%. Factors propelling this growth in the forecast period encompass the escalating number of rare disease therapies undergoing clinical trials, heightened regulatory assistance for the approval of orphan drugs, an upturn in disease-specific patient registries and databases, a surge in international clinical trials and multi-place studies, and additional funding endeavors for the infrastructure relating to rare disease research. Significant trends anticipated during the forecast include the sophisticated use of decentralized trial models, fresh approaches in patient recruitment strategies, the amalgamation of real-world evidence in trial setups, advancements in precision medicine techniques, and the

escalating incorporation of digital health technologies.

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What Are The Driving Factors Impacting The Rare Disease Clinical Trials Market?

The growth of the rare disease clinical trials market is likely to be fuelled by the surging popularity of personalized medicine. Personalized medicine is a medical approach that customizes treatments to singular patient profiles, taking into account genetic factors, lifestyle, and environment. The adoption of personalized medicine has grown due to its success in delivering better treatment outcomes, by tailoring treatments to each patient's specific profiles, enhancing efficacy and safety. Rare disease clinical trials are enhancing the progress of personalized medicine by yielding insights that are specific to individual patients; these insights instrumental in the creation of targeted therapies, precisely designed to suit each patient's exclusive genetic and molecular make-up. For instance, in February 2024, the Personalized Medicine Coalition (PMC), a non-profit entity based in the US, reported that the Food and Drug Administration noted that personalized medicines constituted 34% of all approvals in 2022, a figure that rose to 38% the following year. As such, it's evident that the increase in personalized medicine is fuelling the expansion of the rare disease clinical trials market.

Which Players Dominate The Rare Disease Clinical Trials Industry Landscape?

Major players in the Rare Disease Clinical Trials Global Market Report 2025 include:

- F. Hoffmann-La Roche AG
- Pfizer Inc.
- Novartis AG
- AstraZeneca plc
- Takeda Pharmaceutical Company Limited
- IQVIA Holdings Inc.
- Laboratory Corporation of America Holdings
- ICON plc
- Moderna Inc.
- Charles River Laboratories International Inc.

What Are The Main Trends, Positively Impacting The [Growth Of Rare Disease Clinical Trials Market?](#)

Leading organizations in the market of rare disease clinical trials are directing their efforts towards trailblazing initiatives, such as implementing a customer-centric strategy to present bespoke trial templates, boosting patient interaction, and speeding up the creation of targeted treatments for uncommon diseases. A customer-centric strategy points to the act of placing the needs, objectives, and preferences of biotech and pharmaceutical customers at the forefront in order to deliver customized and potent clinical trial solutions. For example, in March 2025, Evestia Clinical Limited, an American pharmaceutical research corporation, was revealed as the

fresh brand identity of EMAS Pharma. This rebranding is an intentional ploy to fast-track the company's global spread. The firm is dedicated to expediting the creation of treatments for rare ailments by offering expert-backed, bespoke clinical trial solutions adapted to the singular challenges of these diseases. This focus is a fundamental aspect of its brand identity and service offering.

Global Rare Disease Clinical Trials Market Segmentation By Type, Application, And Region
The rare disease clinical trials market covered in this report is segmented as

- 1) By Phase: Phase I, Phase II, Phase III, Phase IV
- 2) By Therapeutic Area: Oncology, Cardiovascular Disorders, Neurological Disorders, Infectious Disease, Genetic Disorders, Autoimmune And Inflammation, Hematologic Disorders, Musculoskeletal Disorders, Other Therapeutic Areas
- 3) By Study Design: Interventional, Observational, Expanded Access
- 4) By End-User: Pharmaceutical Companies, Biotechnology Companies, Research Institutes, Other End-Users

Subsegments:

- 1) By Phase I: First In Human Trials, Dose Escalation Studies, Safety And Tolerability Studies
- 2) By Phase II: Proof Of Concept Studies, Dose Response Studies, Efficacy And Safety Studies
- 3) By Phase III: Randomized Controlled Trials, Comparative Effectiveness Studies, Large Scale Multicenter Trials
- 4) By Phase IV: Post Marketing Surveillance, Long Term Safety Studies, Real World Evidence Studies

View the full rare disease clinical trials market report:

<https://www.thebusinessresearchcompany.com/report/rare-disease-clinical-trials-global-market-report>

Which Region Holds The Largest Market Share In The Rare Disease Clinical Trials Market?

In 2024, North America held the top spot in terms of size in the global rare disease clinical trials market. The report on rare disease clinical trials encompasses all regions, namely Asia-Pacific, Western Europe, Eastern Europe, North America, South America, Middle East and Africa.

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