

# Orphan Drugs Service Market to Reach US \$80.62 Billion by 2029

*The Business Research Company's  
Orphan Drugs Service Global Market  
Report 2025 – Market Size, Trends, And  
Global Forecast 2025-2034*

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Orphan Drugs Service Global Market Report 2025

What Is The Estimated Industry Size Of [Orphan Drugs Service Market?](#)

The market size of orphan drugs services has experienced speedy expansion in recent years. The market is projected to widen from \$37.05 billion in 2024 to \$43.40 billion in 2025, marking a CAGR of 17.1%. Factors such as the escalating prevalence of rare diseases, increasing government aid, the rise of patient advocacy group initiatives, a growing focus on unresolved medical requirements, and escalating cooperation between healthcare providers and researchers have significantly contributed to this growth during the historic period.

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Accelerated expansion is anticipated in the orphan drugs service market in the upcoming years, as it is projected to

reach \$80.62 billion in 2029 with a compound annual growth rate (CAGR) of 16.7%. The predicted growth during the forecast period can be linked to an upswing in biopharmaceutical research investments, the expanding need for precision medicine, the growing acceptance of advanced clinical trial models, an increase in finances from venture capital firms, and a heightened focus on global regulatory harmonization. Key trends for the forecast period encompass advancements in drug discovery platforms, cell, and gene therapy service innovations, resources directed towards artificial intelligence for clinical trials, progress in patient selection based on biomarkers, and novel integration of digital health for patient monitoring.

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### What Are The Major Factors Driving The [Orphan Drugs Service Global Market Growth](#)?

The rise in funding for orphan diseases is contributing significantly to the expansion of the orphan drugs service market. Orphan diseases, while impacting a small section of the populace, often less than 1 in 2,000 individuals, are typically chronic, progressive and can be life-threatening or disabling. The investments in such illnesses have increased due to the recognition of existent medical needs and the prospect of high yields through regulatory incentives like orphan drug classification, market exclusivity, and reduced production overheads. An increase in funding for these under-researched diseases facilitates the growth of orphan drug services by boosting the financing for research and development. As a result, treatments for previously ignored conditions can now be developed. The process also speeds up the availability of drugs by aiding clinical trials, regulatory approvals, and patient access, thereby improving therapy alternatives for people with rare diseases. As an example, a US-based non-profit organization, Global Genes, reported in April 2024 that pharmaceutical firms had secured \$7.1 billion for orphan disease drug development in the first quarter of 2024. This was a significant 307% rise from the \$1.8 billion raised during the same timeframe the previous year. Consequently, the rising funding for rare diseases is stimulating the growth of the orphan drugs service market.

### Who Are The Leading Companies In The Orphan Drugs Service Market?

Major players in the Orphan Drugs Service Global Market Report 2025 include:

- Johnson & Johnson Services Inc.
- F. Hoffmann-La Roche Ltd.
- Pfizer Inc.
- AbbVie Inc.
- Bayer AG
- Sanofi S.A.
- Novartis AG
- AstraZeneca PLC
- The Bristol-Myers Squibb Company
- GSK plc.

### What Are Some Emerging Trends In The Orphan Drugs Service Market?

Leading organizations in the orphan drugs service market are prioritizing the acquisition of regulatory approvals to accelerate development and make rare disease treatments more readily available. Such approvals are formal authorization from government bodies that legally permit the testing, production, and marketing of medications and treatments. For example, Askbio GmbH, a gene therapy company based in the U.S., was given an orphan drug designation by the European Commission in February 2023 for their experimental gene therapy, AB-1003, for limb-girdle muscular dystrophy. This adeno-associated virus (AAV)-based therapy is aimed at replenishing FKBP enzyme activity in muscle cells using one-time intravenous infusion. The European Commission and the U.S. Food and Drug Administration (FDA) have both bestowed orphan drug designation to AB-1003, along with the FDA granting it rare pediatric disease and

fast track designations, emphasizing the critical unmet medical demands. The drug is currently undergoing a Phase 1/2 clinical trial (LION-CS101) to assess its safety and efficacy in adults diagnosed with limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9).

What Are The Primary Segments Covered In The Global Orphan Drugs Service Market Report?

The orphan drugs service market covered in this report is segmented as

- 1) By Type: Congenital Disease, Genetic Disease, Tumors And Cancer, Chronic, Other Types
- 2) By Therapeutic Area: Oncology, Metabolic Disorders, Neurological Disorders, Hematology, Infectious Diseases, Other Rare Diseases
- 3) By Application: Adult, Children

Subsegments:

- 1) By Congenital Disease: Heart Defects, Cleft Lip And Palate, Spina Bifida, Down Syndrome
- 2) By Genetic Disease: Cystic Fibrosis, Huntington Disease, Sickle Cell Anemia, Muscular Dystrophy
- 3) By Tumors And Cancer: Leukemia, Lymphoma, Neuroblastoma, Sarcoma
- 4) By Chronic: Rare Metabolic Disorders, Rare Endocrine Disorders, Rare Cardiovascular Disorders, Rare Neurological Disorders
- 5) By Other Types: Rare Infectious Diseases, Rare Immunological Disorders, Rare Dermatological Conditions, Rare Ophthalmological Disorders

View the full orphan drugs service market report:

<https://www.thebusinessresearchcompany.com/report/orphan-drugs-service-global-market-report>

Which Region Is Forecasted To Grow The Fastest In The Orphan Drugs Service Industry?

In the year 2024, North America held the leading position in the global market for orphan drugs service. It is anticipated that Asia-Pacific will witness the most rapid growth over the projected timeframe. The report on orphan drugs service encompasses several regions including Asia-Pacific, Western Europe, Eastern Europe, North America, South America, the Middle East, and Africa.

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Speak With Our Expert:

Saumya Sahay

Americas +1 310-496-7795

Asia +44 7882 955267 & +91 8897263534

Europe +44 7882 955267

Email: [saumyas@tbrc.info](mailto:saumyas@tbrc.info)

The Business Research Company - [www.thebusinessresearchcompany.com](http://www.thebusinessresearchcompany.com)

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Oliver Guirdham

The Business Research Company

+44 7882 955267

[info@tbrc.info](mailto:info@tbrc.info)

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