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US Orphan Designated Drugs Market Opportunity, Drugs Sales, Price, Dosage & Clinical Trials Insight 2030

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Abstracts

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US Orphan Designated Drugs Market Opportunity, Drugs Sales, Price, Dosage & Clinical Trials Insight 2030 Report Offering & Highlights:

US Orphan Designated Drugs Market Opportunity: > US$ 190 Billion By 2030

Insight On FDA Designated Orphan Drugs In Clinical Trials: > 850 Orphan Drugs

Clinical Trials Insight By Company, Indication, Phase & Priority Status

Insight On FDA Designated Marketed Orphan Drugs: > 500 Orphan Drugs

Pricing & Dosage Insight: > 400 Marketed Orphan Drugs

US, Global, Regional, Annual Sales Insight (2019 – Q1’2025): >150 Orphan Drugs

Sales, Price & Dosage Data Represented In More Than 1000 Charts & Tables

Orphan Designation Insight By Indication, Company, Trial Phase, Marketed Drugs Represented In 1000 Tables

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Research Methodology:

At Kuick Research, we begin by outlining the foundation of our research methodology, which is driven by an integrated approach that combines multiple data dimensions. In our work, we ensure that the report captures market estimation, regional analysis, and a carefully constructed competitive landscape. We take pride in the detailed inclusion of key components such as the assessment of available information, clinical trial data, and the nuances of dosing and pricing in the US. Every piece of data is purposefully gathered and validated to ensure that our findings are as robust as possible, reflecting the multifaceted nature of the market landscape.

This report on the US orphan designated drugs market is the result of comprehensive primary and secondary research, encompassing over 1400 FDA designated orphan drugs, alongside in-depth analysis of their pricing, dosing, and sales data. Market size, marketed drugs regional sales analysis and recent trends are also included in the report. To ensure the accuracy and reliability of our analysis on US orphan designated drugs pricing and market performance, we leveraged an extensive array of sources, including company reports, exchange filings, annual and quarterly reports, and official press releases.

Over 50000 distinct web links were reviewed for comprehensive clinical trial information.

For annual, quarterly, global and regional sales analysis, more than 1500 PDF documents were analyzed.

More than 2000 distinct web links were examined to gather detailed drug pricing and dosage information

More than 400 orphan designated drugs specific websites were accessed for drug profiling

More than 2000 distinct web links were accessed to validate FDA designated orphan drug indications by indications and developer.

Report Overview:

Since the enactment of the Orphan Drug Act in 1983, the drug development landscape

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for rare diseases in the US has changed significantly. The law was enacted to incentivize pharmaceutical companies to concentrate on the development of drugs for rare diseases, i.e., diseases that afflict fewer than 200,000 individuals. Prior to the Act, these diseases had been virtually ignored because of the low economic enticements to firms to develop them. The expenses for developing medications were significant but the patient base was small, creating uncertainty about the monetary returns. The Orphan Drug Act altered this situation by providing a range of incentives, the most significant among which was a seven-year market exclusivity following approval. This assisted in the making of development of treatments for rare diseases economically viable.

More drugs have over the years acquired orphan drug status. To date, hundreds of drugs in development have been assigned such status with the indications ranging from rare tumors to genetic conditions, cardiovascular diseases, and neurologic conditions. The FDA orphan drug designation not only encouraged the development of novel treatments but also has resulted in some unexpected commercial success stories. Those drugs initially designed for limited patient populations now are showing to be profitable products, reversing the opinion that orphan drugs cannot exhibit good market performance.

One of the best examples is Merck's Keytruda (pembrolizumab), an immune checkpoint inhibitor that originally received orphan drug designation in 2012 for malignant melanoma. Since then, Keytruda has had its indications broadened to include various cancers, such as esophageal carcinoma, lymphoma, and lung cancer, among others, many of which are orphan diseases. Although an orphan drug, Keytruda is now amongst the world's best-selling cancer medications, with 2024 revenue of almost US$ 29.4 billion, an astonishing 18% increase over last year. Importantly, more than 60% of this originated from the US alone. This business success demonstrates how orphan drugs, in the long run, can discover larger markets and generate sizeable returns.

Likewise, Gilead's Biktarvy, an HIV drug, was given orphan drug designation and is now one of the company's best-selling drugs. In 2024, it recorded US$ 13.4 billion in sales, up 13% from the previous year. Both drugs underscore how treatments for rare diseases can evolve from specialty therapies into big moneymakers.

The success of these drugs in the market shows that orphan drug development can be both clinically effective and profitable. With appropriate incentives, companies are more likely to invest in the treatment of rare diseases, even if the number of patients is small. The incentives created by the FDA and other regulatory authorities, including the

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potential for market exclusivity and fast-track review, enable firms to break even on their investment and reap huge profits in the long term. These advantages have motivated numerous firms to enter the orphan drug area, particularly since rare diseases are increasingly being understood as a result of advances in genomics and biotechnology.

In addition, the development of the orphan drug market has contributed to the rise in collaborations between pharmaceutical firms. Collaborations can assist in the division of the cost and the acceleration of the development of new medicines. An example is the partnership between Cadrenal Therapeutics and Abbott, established in March 2025 to aid in the development of tecarfarin, a new oral anticoagulant, in patients with HeartMate 3™ Left Ventricular Assist Devices (LVADs). Tecarfarin, granted orphan drug status, is under investigation in a clinical trial for enhancing anticoagulation benefit in patients with advanced heart failure. This alliance reflects the increased trend of grouping together resources and expertise to make therapies for rare conditions, as the scientific and logistical hurdles associated with orphan drug development can be significant.

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