

The Impact of Orphan Drug Legislation on Rare Disorders

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Description

Rare diseases, also known as orphan diseases, affect a small percentage of the population. Despite their rarity, there are thousands of different rare diseases, collectively impacting millions of individuals worldwide. The development of treatments for these conditions has historically been neglected due to the high cost and low potential for financial return. This situation led to the creation of orphan drug legislation, which provides incentives for pharmaceutical companies to develop therapies for rare diseases. This article research the history, impact, and future prospects of orphan drug legislation. The term "orphan drug" emerged in the 1980s, reflecting the lack of interest from pharmaceutical companies in developing treatments for rare diseases. The costs of Research and Development (R&D) for new drugs are immense, and for conditions affecting fewer than 200,000 individuals (the threshold for a disease to be considered rare in the United States), the potential market is too small to justify these costs. As a result, many patients with rare diseases had little hope for effective treatments. In response to this issue, the United States passed the Orphan Drug Act (ODA) in 1983. The primary aim of the ODA was to stimulate the development of drugs for rare diseases by offering various incentives to pharmaceutical companies. These incentives included tax credits for clinical testing, grant funding, assistance with the drug approval process, and seven years of market exclusivity following approval. The success of the ODA inspired similar legislation in other regions, including the European Union, Japan, and Australia.

Incentives and benefits

The incentives provided under orphan drug legislation have proven essential in encouraging the development of treatments for rare diseases. Market exclusivity, one of the most significant incentives, ensures that the company has a temporary monopoly on the drug, allowing them to recoup their investment. This exclusivity is particularly beneficial because it protects the drug from competition from generic versions for a set period, usually seven years in the U.S. and ten years in the EU. Tax credits for clinical testing reduce the financial burden on companies during the expensive phases of drug development. These credits can

cover a substantial portion of the costs associated with clinical trials, making it more feasible for smaller biotech firms to invest in orphan drugs. Additionally, grants and research funding from government agencies and non-profit organizations provide critical support for early-stage research, which is often the most uncertain and risky phase of drug development. Regulatory assistance, including protocol assistance and fee waivers, simplifies the approval process for orphan drugs. This support helps streamline the path to market, reducing the time and cost associated with regulatory compliance. Furthermore, the orphan drug designation can increase the visibility and credibility of a drug, attracting potential investors and partners.

Impact on drug development

Since the enactment of orphan drug legislation, there has been a significant increase in the number of drugs developed for rare diseases. Before the ODA, only a handful of orphan drugs were available. By 2023, over 1,000 orphan drugs had been approved by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) had authorized more than 200 orphan medicinal products. The development of orphan drugs has led to life-changing treatments for many patients with rare diseases. For instance, enzyme replacement therapies for lysosomal storage disorders, such as Gaucher disease and Fabry disease, have drastically improved patient outcomes. Similarly, advancements in gene therapy have provided potential cures for genetic disorders like Spinal Muscular Atrophy (SMA) and certain types of inherited blindness. Orphan drug legislation has also spurred innovation in the pharmaceutical industry. The focus on rare diseases has driven the development of novel therapeutic approaches, including gene therapy, RNA-based treatments, and personalized medicine. These innovations have not only benefited patients with rare diseases but have also advanced the broader field of medicine. Despite the success of orphan drug legislation, several challenges and criticisms remain. One major concern is the high cost of orphan drugs. While the incentives provided under the ODA and similar laws have facilitated the development of these drugs, they have also led to high prices.