

Overview

The conversation around pharmaceutical companies and their responsibility to provide patients with affordable drug treatments and therapies never stops. Orphan Drugs are often a neglected topic when this conversation inevitably arises. With millions of Americans suffering from diseases that lack an FDA-approved treatment, who is expected to bear that responsibility? In our latest Basic, "Orphan Drugs: The Fight to Treat Rare Diseases", we review the past and future of the Orphan Drug Act and the Orphan Drug Tax Credit, providing an assessment of what lies ahead for those who depend most on the development of these life-saving treatments.

What are Orphan Drugs?

Orphan Drugs are pharmaceuticals created with the intent of treating diseases so rare that many manufacturers are hesitant to devote the large amount of resources needed to develop them due to a lack of return on investment.

The process of developing pharmaceuticals that treat public health concerns is long and expensive. Most processes average ten years and cost tens of millions of dollars. All of this time and money can be invested by drug companies, researchers, and public health experts with no guarantee of success. As such, developing a drug that's used to treat a rare disease with relatively few diagnosed patients poses the risk of not recouping all of the capital, research, and resources that went into developing the drug.

Background

In 1983, in an effort to incentivize the development of new treatments for rare and fatal diseases, Congress passed the Orphan Drug Act (ODA). When the ODA was signed into law in 1983, there was widespread agreement among policy makers, medical professionals, clinical researchers and developers, and many others that something needed to be done to spur research and investment for rare disease therapies and treatments. A rare disease is one that affects fewer than 200,000 people but the National Institutes of Health (NIH) estimates that there are somewhere between 6,000 and 7,000 rare diseases affecting nearly 30 million Americans. In other words, rare diseases affect 1 in 10 Americans.

The ODA was designed to alleviate the risk a company may face if they invested resources heavily in the pursuit of a therapy that would be used to treat a rare disease. Companies were finally able to undertake risky development programs that included expensive clinical trials with a low probability of success, and not fear a total loss. The ODA provided some security and assurance of a company's ability to recover its investment in an effort to make life-saving treatments available to the public.

Center Forward Basics

Center Forward brings together members of Congress, not-for-profits, academic experts, trade associations, corporations and unions to find common ground. Our mission: to give centrist allies the information they need to craft common sense solutions, and provide those allies the support they need to turn those ideas into results.

In order to meet our challenges we need to put aside the partisan bickering that has gridlocked Washington and come together to find common sense solutions.

For more information, please visit www.center-forward.org

Key Facts

- **30 million Americans** are affected by a rare disease
 - That's **1/10** living in the U.S.
- Approximately **7,000** rare diseases are known, but only about **5%** have a FDA treatment approved
 - **85-90%** of rare diseases are serious or life-threatening
- **80%** of rare diseases are genetic in origin
- Before the passage of the Orphan Drug Act in 1983, only **40 orphan drugs** had been approved for commercial use.
 - Since its passage, just under **500 additional orphan drugs** have been granted FDA approval

Orphan Drug Tax Credit

The Orphan Drug Tax Credit (ODTC) was a temporary benefit included in the 1983 passage of the ODA that was ultimately made a permanent component of the tax code in 1997. The ODTC is the real tool in the ODA that makes research and development of orphan drugs possible. The ODTC is a research and development credit that allows drug manufacturers to claim a credit for 50% of certain research and clinical trial costs associated with orphan drugs.

If the ODTC were eliminated from the tax code, pharmaceutical companies could once again face burdensome costs for orphan drug development, ultimately reducing overall research and development. A June 2015 [Ernst & Young study](#) estimated that without the ODTC, '57 fewer, or 33% less, new orphan drugs would be approved over the next 10 years.'

Recent Changes

The ODA and ODTC may be little known provisions of federal law; however, the benefits and protections of the law are widely utilized by drug researchers and manufacturers. Opponents of the tax credit argue that it has been wildly successful for pharmaceutical corporations. They point to specific examples of several drugs bringing in billions annually while only being approved to treat a handful of diseases. Other opponents simply argue that the law has been on the books since the early 1980s and it was time for an update.

Before Congress passed recent tax legislation and the president signed it into law, pharmaceutical companies were able to claim a credit for 50% of certain research and clinical trial costs associated with orphan drugs. While the ODTC was eliminated in the House version of tax reform, the Senate version sought to preserve the tax credit by reducing the rate to 27.5%. This was no surprise, however, as Senator Orrin Hatch of Utah who chairs the tax-writing Senate Committee on Finance was an original cosponsor of the ODA. He has, though, asked the Government Accountability Office to investigate possible abuses associated with the ODC and the ODTC.

Supporters of the ODTC argue that while they agreed the overall corporate tax rate should be lowered, eliminating or reducing this particular tax credit would seriously limit their ability to develop and deliver life-saving treatments. The ODTC is a critical incentive and without it, drug manufacturers will likely not invest in these endeavors as they currently are.

When House and Senate conferees met to work out the differences between the two drafts of the bill, they settled on allowing the ODTC to remain, but reduced the rate from 50% to 25% for all qualified testing expenses. Legislators estimated that the bill as agreed upon would save the federal government roughly \$32.5 billion from 2018 to 2027. These changes were made as a part of the recent tax bill signed by President Trump in 2017.

Definitions

Rare Disease: A disorder or condition that affects less than 200,000 persons in the United States, as defined by the Orphan Drug Act of 1983.

Other Resources

- BIO and Ernst and Young
 - [Impact of the Orphan Drug Tax Credit on treatments for rare diseases](#)
- GlobalGenes
 - [Section 2: Difference in Orphan Drug Development and Approval](#)
- NORD
 - [Trends in Orphan Drug Costs and Expenditures Do Not Support Revisions in the Orphan Drug Act: Background and History](#)
- OrphanNet
 - [About Orphan Drugs](#)
- U.S. FDA
 - [Designating an Orphan Product: Drugs and Biological Products](#)
 - [Developing Products for Rare Diseases & Conditions](#)

Links to Other Resources

- BIO and Ernst & Young — Impact of the Orphan Drug Tax Credit on treatments for rare diseases
<https://www.bio.org/sites/default/files/EY%20BIO%20Orphan%20Drug%20Tax%20Credit%20Report%202015%2006%2016.pdf>
- Global Genes — Section 2: Difference in Orphan Drug Development and Approval
<https://globalgenes.org/toolkits/drug-development-overview/how-is-drug-development-for-rare-diseases-different/>
- National Organization for Rare Disorders (NORD) — Trends in Orphan Drug Costs and Expenditures Do Not Support Revisions in the Orphan Drug Act: Background and History
https://rarediseases.org/wp-content/uploads/2017/10/NORD-IMS-Report_FNL.pdf
- OrphanNet — About Orphan Drugs
http://www.orpha.net/consor/cgi-bin/Education_AboutOrphanDrugs.php?lng=EN&stapage=ST_EDUCATION_EDUCATION_ABOUTORPHANDRUGS
- U.S. Food and Drug Administration (FDA) — Designating an Orphan Product: Drugs and Biological Products
<https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/default.htm>
- U.S. Food and Drug Administration (FDA) — Developing Products for Rare Diseases & Conditions
<https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/ucm2005525.htm>