

INCENTIVES PROVIDED BY THE ORPHAN DRUG ACT

The Orphan Drug Act (ODA) was passed by Congress in recognition of the need to incentivize innovative rare disease drug development. In the decade before the ODA was passed, the FDA had approved very few therapies to treat rare diseases. This left millions of patients within the United States, and the rest of the world, without treatment.¹



Incentives of the **Orphan Drug Act** include:



Exclusivity

The FDA grants **market exclusivity for 7 years to the first sponsor** of an orphan drug that receives FDA approval for a designated rare disease or condition, this is compared to the 5 years of exclusivity that sponsors receive for drugs that treat common diseases.



Tax Credit

A sponsor may claim **tax credits of up to 25%** for expenditures incurred during the clinical trials. The tax credit allows companies to save money on qualifying clinical trial costs that they can then use for further development of their therapy.



Waiver of Prescription Drug User Fees

The sponsor's fee as outlined in the Prescription Drug User Fee Act (PDUFA Fees) at the time of submitting a marketing application to FDA may be **waived** for a designated rare disease product,² **reducing** the costs associated with the FDA approval process for a new orphan drug. Waivers of PDUFA fees, like the tax credit, allow companies to put the money they save towards **developing** new therapies.

Additional incentives that also encourage rare disease drug development, include:

Orphan Products Grant Program — **GRANTS**

are available to help defray the costs of clinical



research needed to investigate orphan products.

Rare Disease Priority Review Voucher

Program — Under the Federal Food, Drug, and Cosmetic Act (FD&C Act), FDA can award **PRIORITY REVIEW VOUCHERS** to sponsors of rare pediatric disease product applications that meet certain criteria. Under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed



to receive a priority review of a subsequent marketing application for a different product.

¹ FDA at Rare Disease Day, accessed April 03, 2018.

² <https://www.fda.gov/forindustry/developingproductsforrare diseasesconditions/ucm239698.htm>

THE ORPHAN DRUG ACT: MAKING THE IMPOSSIBLE HAPPEN FOR RARE DISEASE PATIENTS

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The Orphan Drug Tax Credit is one of the only tax credits that actually saves lives. It also gives hope to the nearly 95 percent of individuals with rare diseases without a treatment that one day they too will have a treatment, or even cure.

— Letter from 140 patient advocacy groups. September, 2017

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1983

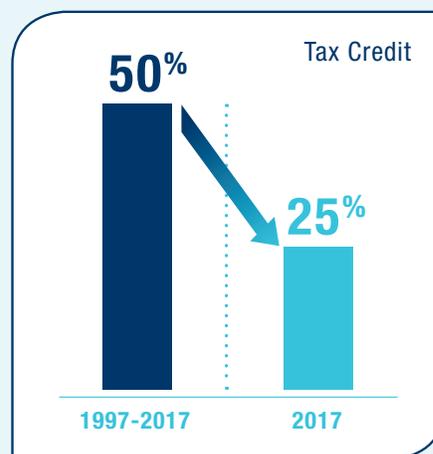
The Orphan Drug Act (ODA) was enacted in 1983 to **incentivize drug development for rare diseases**. The ODA mobilized the biopharmaceutical industry with several incentives that ameliorated the risk of drug development for rare diseases. One of these key incentives was the Orphan Drug Tax Credit.

1997

The Orphan Drug Tax Credit was made permanent in 1997 and it **provided a tax credit of 50% percent** of the qualified clinical testing expenses incurred with respect to FDA-designated orphan drugs”?

2017

During the **2017 tax reform debate, the Orphan Drug Tax Credit was almost eliminated**; due to a concerted effort the tax credit was saved but it was halved from 50 to 25 percent. This change will make it harder to raise the capital required to develop a single drug to treat a disease with a small patient populations.



We will not fully understand the economic and social impact of this change in the rare disease investment space and in the community of patients with rare diseases for many years to come.



In order to continue to incentivize rare disease drug development, the current Orphan Drug Tax Credit must be maintained.