Rare Disease
Company Coalition

Orphan Drug Tax Credit is a Proven Catalyst for Investment and Treatment Development

1983

Year ODA established by Congress

~95%

Percentage of rare diseases that lack an FDA-approved drug

The Rare Disease Company Coalition calls on Congress to preserve this important tax credit vital for developing new treatments for people living with rare diseases.

The Orphan Drug Act (ODA) was established by Congress in 1983 and subsequently created the Orphan Drug Tax Credit (ODTC) for the advancement of rare disease treatments. The ODTC is a key provision of the ODA and promotes research for rare diseases by lowering development costs for manufacturers. It is an important incentive for companies working to find rare disease treatments, as it reduces the significant financial cost and risk that comes with the research and development of new therapies for small patient populations.

The ODTC is a vital lifeline for rare disease patients and should be protected to preserve hope for the rare disease community: Given that approximately 95% of rare diseases of rare diseases lack an FDA-approved drug, the proposal to reduce the ODTC would have a devastating impact on orphan drug development in the U.S. and on millions of Americans living with a rare disease. We must preserve innovation and not lose the gains that have been made since the passage of the ODA.

#OneRareVoice

ODTC Has Proven Effective for Driving Investment into Therapy Development

- Rare diseases have small patient populations, resulting in increased risk and higher research and development costs. The ODTC incentivizes biotechnology companies to invest in the development of treatments that are not otherwise economically viable.
- At the time the ODTC was established, only 38 drugs had been approved by the U.S. Food and Drug Administration (FDA) for rare diseases. Today, there are more than 650 drugs approved to treat rare diseases demonstrating the effectiveness of the ODTC.

Congress Should Further Access to Rare Disease Treatments

- Policies that arbitrarily restrict eligibility of the ODTC to only include qualified clinical testing expenses for the first approved orphan use or indication of a new drug would lead to less drug development. In this case, the tax credit would not be available for clinical testing expenses to evaluate whether existing therapies can treat children in addition to adults, or whether existing therapies can treat additional rare conditions.
- When a drug is approved, it has been established as a safe and effective treatment option for one disease. Though a company still must invest significantly to establish the drug's safety and efficacy for each additional patient population, it is both prudent and typical to determine if the drug is applicable to other disease states. Approximately 25 percent of rare disease therapies have been approved to treat two or more orphan indications.

38

Number of drugs that had been approved for rare diseases at passage of ODTC in 1983

>650

Number of drugs that have been approved for rare diseases today because of ODTC

~25%

Percentage of rare disease therapies that are approved to treat two or more orphan indications

Rare Disease Company Coalition

We are dedicated to being a reliable and trusted resource for our leaders in Congress and the Administration, and a productive partner to rare disease-minded industry, academic and patient groups, by educating on the issues and opportunities that affect rare disease companies and advancing our shared mission to improve the lives of people living with rare diseases.

For more information, please visit www.rarecoalition.com or contact info@rarecoalition.com.
Follow us on Twitter at @RareCoalition.

#OneRareVoice

05_2022