

Building on 40 Years of the Orphan Drug Act

40 years later, the Orphan Drug Act (ODA) continues to foster innovation and investment in research and development for rare disease treatments.



200+

New treatments created by
RDCC member companies
utilizing ODA incentives



In the next 40 years, Congress should take steps to protect and build on the policies that have enabled progress for rare disease patients – not chip away at them.

Celebrating Success

By its nature, the rare disease drug research and development process incurs more risk. Treatments are manufactured for limited population sizes, but their development costs remain the same or higher than drugs for larger populations due to the lack of natural history, complex diagnosis, small population sizes for clinical trials, patients for participation in clinical trials, and the often-unprecedented regulatory pathway.

In 1983, the ODA was passed to incentivize investment in rare disease research and development where there was little research being done and almost no treatment options

for those diagnosed with a rare disease. Prior to the ODA, only 38 orphan drugs had been approved by the FDA. Today, more than 650 medicines have been approved for rare disease patients—an astounding success. As a result, the experience of receiving a rare disease diagnosis has changed dramatically since the ODA's passage – from despairing of ever having a treatment to having hope that even if a treatment is not currently available, researchers even now may be uncovering a path to develop a safe and effective medicine for your condition.

In the next 40 years, Congress should protect and build on the policies that have enabled this progress, not chip away at them.

The advances accrued over the past 40 years would not have been achieved without the supportive policy and regulatory environment established by the ODA. To safeguard the future of rare disease research, it is imperative that Congress preserve and strengthen the ODA.

Looking Ahead

More work is needed. As we recognize the ODA's role in incentivizing biomedical innovation in this much needed area, we acknowledge that at least 95% of rare diseases still lack treatment today, necessitating urgent policy considerations to bring treatments to the 1 in 10 Americans suffering from a rare disease.

The RDCC and our partners in the rare disease community call on lawmakers to protect the existing policies that

provide incentives for rare disease drug research and development, and to establish new incentives that will enable additional breakthroughs for patients.

RDCC members are at the forefront of these medical innovations, pioneering advances in molecular biology and targeted biotechnology development to bring treatments to rare disease patients. In fact, RDCC members are currently developing more than 200 promising rare disease treatments, which speaks to the success of the ODA.

We Call on Policymakers to:

Restore the OTDC to 50% of applicable R&D costs

The Orphan Drug Tax Credit (ODTC) is vital for incentivizing companies to invest in R&D for rare disease treatments that would otherwise be economically unviable. When established in 1983, companies were encouraged to claim up to 50% of expenditures incurred during clinical trials. The amount of the ODTC was reduced in 2017 under the Tax Cut and Jobs Act from 50 percent to 25 percent, yet remains a critical program for sustained innovation and investment for innovator companies that focus on life-changing development programs for treatments for rare diseases.

Rather than reducing the level of the ODTC, leaders in Congress should increase and strengthen the credit, making it financially viable for companies to pursue rare disease drug development.

Bolster the ODTC to its original 50% of qualified R&D costs by advancing legislation to ensure stability and protect advances made in rare diseases research and development.

Support exemptions for rare disease treatments in the IRA and future drug pricing policies

The Inflation Reduction Act (IRA)'s Drug Price Negotiation Program takes a positive step forward by exempting drugs indicated for only one rare disease or condition from negotiation, but further action is needed to ensure future research for additional rare diseases and conditions.

Support exemptions for rare disease treatments in the IRA and future drug pricing policies to ensure that future research for new rare indications is not put at risk.

Incentivize companies to complete promising clinical trials that were put on pause during the pandemic

The ODA recognizes the importance of exclusivity for drugs with orphan designation as a critical incentive to make drug development viable and is especially important as companies attempt to resume clinical trials following the COVID-19 public health emergency. Clinical trials were harder to complete given the health concerns of higher-risk rare disease patients, and companies faced added financial challenges from new safety and logistical challenges as well as greater uncertainty in trial continuation. Extending exclusivity for orphan drugs disrupted during the pandemic will incentivize companies to complete these trials and bring new treatments and cures to patients battling rare diseases.

Incentivize companies to pursue R&D in rare disease drug development, inherent with added obstacles added from the COVID-19.

Incentivize R&D by making permanent the Priority Review Voucher (PRV)

The FDA's priority review vouchers (PRV) provide a critical and cost-effective incentive to spur the investment in high-risk development of treatments. By making this program permanent, Congress can further offset the high cost and risk of rare disease drug development. Additionally, Congress should build upon this successful model and create additional systems that allow orphan drug manufacturers to receive vouchers that provide regulatory advantages, such as expedited FDA approval.

Make permanent the priority review vouchers (PRVs) that are set to expire between 2024-2026 and consider new types of incentive modeled on the PRV.



**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](https://twitter.com/RareCoalition).

1:10

One in ten people lives with a rare disease and half are children

~95%

Percentage of rare diseases that have no FDA-approved treatment options

38

Orphan drugs approved by the FDA in 1983

>650

Orphan drugs approved by the FDA in 2023



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#OneRareVoice