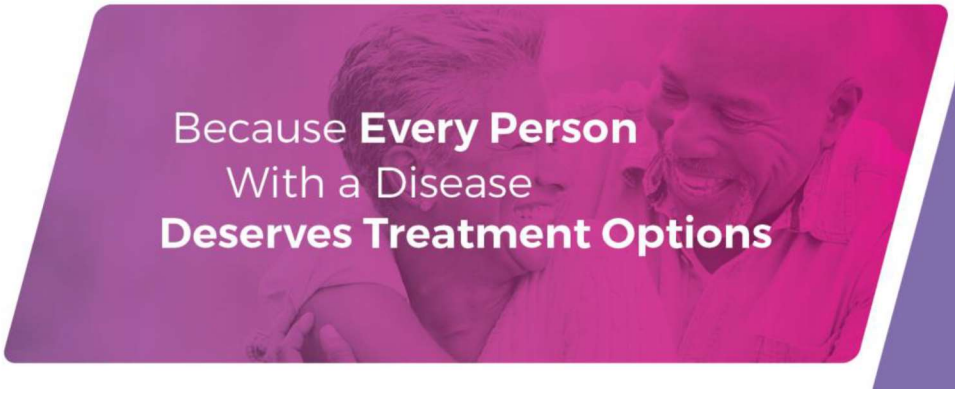
Save Rare Treatments



Date: August 2, 2024

The Honorable Diana DeGette
House Energy and Commerce Committee
2313 Rayburn House Office Building
United States House of Representatives
Washington, DC 20515

The Honorable Larry Bucshon
House Energy and Commerce Committee
2111 Rayburn House Office Building
United States House of Representatives
Washington, DC 20515

Sent Via Electronic Transmission

Dear Representatives DeGette and Bucshon:

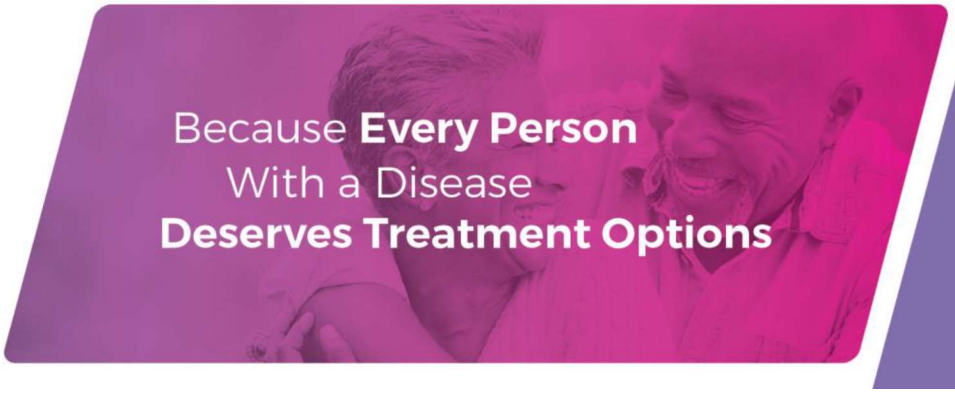
The Save Rare Treatments Task Force, a multi-sector public policy and advocacy collaboration of organizations representing individuals living with rare diseases, biopharmaceutical innovators, and other stakeholders, appreciates your continued dedication to accelerating the discovery, development, and delivery of life-saving treatments and cures for patients.

The Task Force is pleased to offer a response to your Request for Information on policies for consideration on what Congress can do to bring about the next generation of treatments. The Task Force recommends you consider policies to protect key research incentives and propel rare disease biomedical research into the future, including changes designed to make needed adjustments to the Orphan Drug Exclusion in the Medicare Drug Price Negotiation Program (MDPNP).

Individuals with rare diseases have significant unmet needs in access to therapies targeted to treat their conditions. There are approximately 30 million Americans living with a rare disease, and of the estimated 10,000 known rare diseases, 95 percent lack an FDA-approved treatment.[1] Without further innovation and protections for existing incentives that reward rare disease drug development, these individuals will not receive the treatments and cures that are possible and needed to improve their lives.

While we still have a long way to go in bringing treatments to individuals with rare diseases, it is important to remember that Congress designed an incentive structure through the *Orphan Drug Act* (ODA) that has accelerated rare disease research over the last 40 years. Congress passed the ODA in 1983 because individuals with rare diseases were being left out of significant medical advances due to the barriers to investment in the research and development needed to bring new therapies to market.[2] The ODA's incentives have had a marked impact on accelerating the development of treatments for rare diseases.[1] Since its passage, more than 880 drugs have been approved for more than 1,200 indications,[3][4] including 20 new FDA approvals for rare diseases in 2022 alone.[5] By contrast, prior to the passage of the ODA, only 38 *total* drugs treating rare diseases entered the market.[6]

[1] ODA incentives include market exclusivity, tax credits, user fee exemptions, and grants for clinical testing for drugs designated with orphan status.



Now, Congressional action is needed to preserve the rate of progress made in developing treatments for rare diseases. The structure of the Orphan Drug Exclusion in the MDPNP undermines the incentives of the ODA by exposing orphan drugs to negotiation eligibility prematurely. The exclusion is only available when an orphan drug is designated for a singular rare disease or condition, and when FDA approval for the drug is solely within that designation. This overly narrow exclusion ignores the value of research undertaken today that results in multiple designations and approvals for a product to treat multiple rare diseases.[7]

Without Congressional intervention, we believe the development of treatments for rare diseases that was made possible by the bipartisan ODA will suffer. An analysis of orphan drug development predicted that there will be a 40 percent reduction in orphan FDA approvals between 2026 and 2035, with 14 of the 34 orphan drugs from the manufacturing cohort analyzed failing to make it to market because of the MDPNP.[8] Pharmaceutical manufacturers have also begun announcing decisions to rescind development based on the Orphan Drug Exclusion.[910]

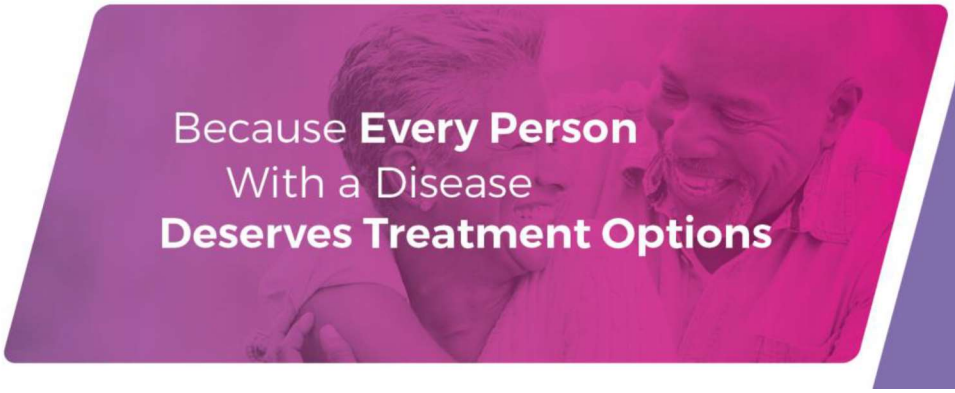
As you consider what policies are needed to further the *21st Century Cures* and *Cures 2.0* goals of accelerating biomedical research and increasing patient access to novel therapies, we urge you to adopt policies that ensure rare disease innovation continues to be incentivized. Congressional action is necessary to restore the spirit of the research and development incentives in the ODA. The Task Force strongly supports the *Optimizing Research Progress Hope And New* (ORPHAN) *Cures Act* (H.R. 5539 / S. 3131). The *ORPHAN Cures Act* is designed to ensure that eligibility for the Orphan Drug Exclusion allows products to remain excluded from negotiation so long as their FDA approved uses are exclusively for rare diseases, as well as clarifies that the clock for negotiation eligibility starts at the date of a product's first non-rare approval.

This bipartisan, bicameral legislation will correct the problems with the Orphan Drug Exclusion, fostering vitally needed research and development of new treatments for rare diseases. The Task Force encourages your consideration of this proposal as you work with stakeholders to determine the most critical policy elements needed for future drug development and increased patient access.

We appreciate your consideration of our input and look forward to working with you throughout the legislative process. Please reach out to Shannon Deere at shannon.deere@leavittpartners.com with any inquiries for the Task Force.

Sincerely,

Save Rare Treatments Task Force



[1] National Center for Advancing Translational Sciences. Delivering Hope for Rare Diseases. (2023). https://ncats.nih.gov/sites/default/files/NCATS_RareDiseasesFactSheet.pdf

[2] Swann, J. (2018). The Story Behind the Orphan Drug Act. U.S. Food and Drug Administration. https://www.fda.gov/industry/fdas-rare-disease-day/story-behind-orphan-drug-act#:~:text=That%20law%2C%20the%20Orphan%20Drug,for%20research%20and%20development%20expenses

[3] FDA. Search orphan drug designations and approvals. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/.

[4] Fermaglich, L.J. and Miller, K.L. (2023, June 23). A comprehensive study of the rare diseases and conditions targeted by orphan drug designations and approvals over the forty years of the Orphan Drug Act. *Orphanet Journal of Rare Diseases*. BioMed Central2023. https://ojrd.biomedcentral.com/articles/10.1186/s13023-023-02790-7.

[5] Lore, A. (2023). Celebrating 40 years of the Orphan Drug Act on Rare Disease Day. PhRMA. https://phrma.org/blog/celebrating-40-years-of-the-orphan-drug-act-on-rare-disease-day

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[7] In a study of the nearly 500 orphan drugs approved between 1990-2022, 15 percent had been approved for multiple rare diseases. Miller, K. L. and Lanthier, M. (2024, Jan 8). Orphan Drug Label Expansions: Analysis Of Subsequent Rare And Common Indication Approvals. *Health Affairs*, 43 (1). https://www.healthaffairs.org/doi/10.1377/hlthaff.2023.00219.

[8] Gassull, D., Bowen, H., & Schulthess, D. (2023, Jun 1). IRA's Impact on the US Biopharma Ecosystem. Vital Transformation. https://vitaltransformation.com/wp-content/uploads/2023/10/VT-BIO_IRA_v14.pdf.

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[10] Zhang, R. C., Cohrs Zhang, R. (2023). Genentech weighs slow-walking ovarian cancer therapy to make more money under drug pricing reform. STAT. https://www.statnews.com/2023/08/10/genentech-drug-price-cancer/.