Save Rare Treatments

Date: February 6, 2025

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Washington, DC 20515

Congressman Don Davis
United States House of Representatives
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Congressman Kevin Hern
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Congresswoman Mariannette Miller-Meeks
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Congressman Shri Thanedar
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Congressman Gus Bilirakis
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Congressman Josh Gottheimer
United States House of Representatives
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Sent Via Electronic Transmission

Dear Congressmen Joyce, Davis, Hern, Peters, Hudson, Bilirakis, Gottheimer, Keating, and Miller-Meeks,

The Save Rare Treatments Task Force strongly supports your bipartisan legislation, the Optimizing Research Progress Hope And New (ORPHAN) Cures Act (H.R. 946). The ORPHAN Cures Act is a critically needed, targeted solution to protect incentives for the research and development of medicines to treat rare disease and to accelerate the creation of the next generation of treatments and cures.

The Task Force is a public policy alliance comprised more than 30 organizations representing individuals living with rare diseases, biopharmaceutical innovators, and health sector leaders committed to effectuating a technical fix of the Orphan Drug Exclusion in the Medicare Drug

Price Negotiation Program. The Orphan Drug Exclusion as it exists today unintentionally undermines the continued discovery and development of new treatments for people living with rare diseases which erodes the success more than 40 years of bipartisan research incentives.

Research incentives are desperately needed to fuel new treatments in rare disease. Approximately 30 million Americans have a rare disease, and roughly half of patients diagnosed with rare diseases are children.[i] Yet, of the estimated 10,000 rare diseases and rare cancers, less than 10 percent have an FDA approved treatment.[ii] This means most Americans with a rare disease have no treatment specifically designed to treat their condition. Millions of Americans are waiting for continued research and development to make new treatments available.

Congress has long recognized the benefits of a policy environment which allows rare disease innovation to flourish. With an understanding of the challenges in research rare disease treatments and the low number of treatments approved for individuals with rare disease, Congress passed bipartisan legislation in 1983 specifically designed to provide needed incentives to kickstart innovation.[iii] Since then, the number of federally approved orphan drugs increased by 1,576 percent – from 38 to more than 880 drugs that treat more than 1,200 rare disorders.[iv] This progress underscores the need for Congress to preserve incentives for research and development of treatments for rare disease.

Today, the ORPHAN Cures Act is urgently needed to ensure the future of these bipartisan incentives and address unintended consequences which are negatively impacting the research landscape. Currently, orphan drugs are only excluded from price negotiation when they are designated for a single rare disease or condition, and when FDA approvals are solely within that designation. Additionally, the date used to begin a drug's tolling toward negotiation begins on the date of its first approval, even if that approval is for a rare disease.

The ORPHAN Cures Act will correct both of these issues by (1) ensuring that the Orphan Drug Exclusion from negotiation eligibility allows products to remain excluded so long as their FDA approved uses are exclusively for rare diseases and (2) clarifying that the clock for negotiation eligibility starts at the date of a product's first non-rare approval. These narrowly targeted changes will ensure incentives remain to prioritize investment into rare disease treatments.

Across the rare disease community, individuals living with rare disease and their families are concerned about the impact the Orphan Drug Exclusion is already having on investments and pipeline decisions for rare disease treatments. By one estimate, price negotiation would lead to a reduction in research and development spending by $663 billion, resulting in 135 fewer new drugs being approved through 2039.[v] The ORPHAN Cures Act is critical for reversing this trend.

Save Rare Treatments

The Task Force thanks you for your bipartisan leadership to bring hope to the rare disease community by dramatically improving the environment for research and development of evidence-based, FDA-approved therapies. We look forward to working with other members of Congress on a bipartisan basis to help advance the ORPHAN Cures Act through the legislative process to ensure that strong incentives for research and development are adopted so that individuals with rare diseases continue to benefit from new treatments to meet their needs.

Sincerely,

Save Rare Treatments Task Force

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[i] Wan, E. L., Elkaim, Y., Gao, W., Yoon, R. (2023) Zebras Among Us: Advocating for the 30 Million Americans Living with Rare Disease. *Medical science educator*. 33(5), 1239- 1242. doi:10.1007/s40670-023-01856-2.

[ii] National Center for Advancing Translational Sciences. (2023*). Rare Disease Day at NIH 2023: Putting Hope Into Action. National Institute of Health*. https://ncats.nih.gov/news-events/news/rare-disease-day-at-NIH-2023-putting-hope-into-action.

[iii] The Orphan Drug Act, 42 U.S.C. § 360bb (1983).

[iv] "Recognizing the 40th Anniversary of the Orphan Drug Act, the Rare Disease Company Coalition Calls on Policymakers to Renew Commitment to Advancing Innovation for Rare Disease Patients." (2023). Rare Disease Company Coalition.

[v] Philipson, T., Ling, Y., & Chang, R. (2022). "The Impact of Recent White House Proposals on Cancer Research." University of Chicago.