

Save Rare Treatments



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New Task Force Endorses Legislation to Save Rare Disease Treatments

(WASHINGTON, D.C.) – [The Save Rare Treatments Task Force](#), a new multi-sector public policy and advocacy collaboration convened by Leavitt Partners, an HMA Company, publicly launched this week and today released a letter of support for recently introduced, bipartisan, bicameral legislation. The Task Force is comprised of organizations representing persons living with rare diseases, biopharmaceutical innovators, and other health sector leaders committed to modifying the Medicare Drug Price Negotiation Program’s (MDPNP) overly narrow Orphan Drug Exclusion that undermines the discovery and development of new treatments for persons living with rare diseases.

“Approximately 30 million Americans have a rare disease,” the members wrote. “Yet, of the estimated 10,000 rare diseases, 95 percent lack a Food and Drug Administration (FDA)-approved treatment. That means most Americans with a rare disease have no treatment specifically designed to treat their condition. Millions of Americans require continued research and development to make new treatments available” they wrote.

“The Save Rare Treatments Task Force appreciates that the *ORPHAN Cures Act* is the solution tailored to correct the MDPNP’s Orphan Drug Exclusion, which undermines incentives to research and develop drugs for rare diseases,” Task Force members said. “Anything less than a framework that restores incentives for rare disease research and development will not solve the problem,” members wrote. “We have seen rare disease drug developments scaled back in the wake of this new law, and it is time to reverse this trend,” they wrote. The members thanked the Senators and Congressmen for the legislation, calling it a “critical step forward in Congress correcting the Orphan Drug Exclusion to ensure that the research and development incentives that have succeeded for decades are maintained” in the future. “We appreciate your bipartisan leadership and look forward to working with other members of Congress on a

bipartisan basis to help advance this important legislation through the legislative process,” they wrote.

“Rare disease patients, caregivers, and the entire rare disease community is concerned about the impact the Orphan Drug Exclusion is already having on investments and pipeline decisions for rare disease treatments,” the members wrote. “Individuals living with rare diseases and their families need and deserve continued research, development, and innovation to enable the advancement of new, life-sustaining, and life-saving treatments,” they wrote.

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About the Medicare Drug Price Negotiation Program

In 2022, Congress created the “Medicare Drug Price Negotiation Program.” This program requires CMS to set prices for certain drugs covered under Medicare Part B (physician-administered drugs) and Part D (retail prescription drugs), starting with 10 high-spending, single-source drugs for 2026 and increasing cumulatively each year. By 2031, 100 drugs will be subjected to price negotiations. The Program excludes orphan drugs from price negotiation eligibility only when they are designated for a single rare disease or condition. However, this exclusion is limited to drugs treating a single rare disease. This narrow exclusion discourages research and development of treatments for other rare diseases.

About the Save Rare Treatments Task Force

The Task Force was created in Fall 2023 to work with Congress and the Administration to ensure that the Orphan Drug Exclusion under the Medicare Drug Price Negotiation Program allows products to remain excluded from negotiation eligibility so long as their FDA approved uses are exclusively for rare diseases. The Task Force’s goal is to restore the spirit of the research and development incentives in the Orphan Drug Act that have succeeded for more than 40 years so more persons living with rare diseases can have treatment options. The Task Force is convened and managed by [Leavitt Partners](#), an HMA Company.