Save Rare Treatments

Date: November 16, 2023

Senator John Barrasso
United States Senate
307 Dirksen Senate Office Building
Washington, DC 20510

Senator Tom Carper
United States Senate
513 Hart Senate Office Building
Washington, DC 20510

Congressman John Joyce
United States House of Representatives
152 Cannon House Office Building
Washington, DC 20515

Congressman Wiley Nickel
United States House of Representatives
1133 Longworth House Office Building
Washington, DC 20515

Sent Via Electronic Transmission

Dear Senator Barrasso, Senator Carper, Congressman Joyce, and Congressman Nickel,
The Save Rare Treatments Task Force, a new multi-sector public policy and advocacy collaboration, is pleased to offer its strong support for your bipartisan legislation, the *Optimizing Research Progress Hope And New (ORPHAN) Cures Act* (H.R. 5539 / S. 3131). 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) narrow Orphan Drug Exclusion that undermines the discovery and development of new treatments for persons living with rare diseases.

As you know, approximately 30 million Americans have a rare disease. 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.

Today, the *ORPHAN Cures Act* is urgently needed to modify the MDPNP's Orphan Drug Exclusion. Currently, the MDPNP excludes orphan drugs from negotiation eligibility only when they are designated for a single rare disease or condition, and when FDA approvals are solely within that designation. The *ORPHAN Cures Act*: (1) ensures 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) clarifies that the clock for negotiation eligibility starts at the date of a product's first non-rare approval.

The *ORPHAN Cures Act* will correct these problems and continue this vital era of needed research and development of new treatments for rare diseases. Prior to the *Orphan Drug Act of 1983*, FDA had approved only 38 treatments for rare diseases. Today, there are more than 550 treatments approved for more than 1,100 indications.[i] These new treatments have brought help and hope to millions of Americans and are an example of the kinds of innovation that Congress can help foster through smart, targeted, bipartisan policy.

The changes envisioned in the *ORPHAN Cures Act* are timely. Rare disease patients, caregivers, and the entire rare disease community are concerned about the impact the Orphan Drug Exclusion is already having on investments and pipeline decisions for rare disease treatments. We have seen rare disease drug developments scaled back in the wake of this new law, and it is time to reverse this trend. 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.

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. Anything less than a framework that restores incentives for rare disease research and development will not solve the problem.

Thank you for this 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. 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.

Sincerely,

Save Rare Treatments Task Force

[i] https://www.fda.gov/news-events/fda-voices/fda-continues-important-work-advance-medical-products-patients-rare-diseases.