Save Rare Treatments

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Save Rare Treatments Task Force Applauds Senators Barrasso and Heinrich for Introducing the ORPHAN Cures Act

Patient-Focused Task Force Thanks Senators for Bipartisan Legislation that Will Remove Barriers to Research and Development of New Rare Disease Treatments

The Save Rare Treatments Task Force thanks Senators John Barrasso (R-WY) and Martin Heinrich (D-NM) for their bipartisan introduction of the ORPHAN Cures Act (S.1862). The Optimizing Research Progress Hope And New (ORPHAN) Cures Act is a critical legislative fix to unintended consequences in current law targeted to ensure that innovations in rare disease treatments will continue to be incentivized.

The Save Rare Treatments Task Force encourages Congress to take action on the ORPHAN Cures Act so that this needed legislation can become law. The Task Force is a collaboration of people with rare diseases, patient advocacy organizations representing people with rare diseases, rare disease biopharmaceutical innovators, and other health sector leaders dedicated to expanding access to rare disease treatments by protecting and fostering innovation that leads to new options and expanded indications for approved treatments.

Strong research and development 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. Yet, of the estimated 10,000 rare diseases and rare cancers, less than 10 percent have an FDA-approved treatment for that disease. This means most Americans with a rare disease have no treatment specifically designed to treat their condition. Millions of Americans are putting their hope in continued research and development to enable new treatments for them or their loved one.

Growing evidence is already showing the harm to innovation as a result of the Inflation Reduction Act (IRA) and its overly narrow Orphan Drug Exclusion in the Medicare Drug Price Negotiation Program. Some troubling trends include:

- By one estimate from University of Chicago researchers, 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.
- Recently published research from the National Pharmaceutical Council shows a significant decrease in the percentage of second orphan designations, down 48 percent after the IRA's passage. This means that fewer investments are going toward expanded uses of FDA-approved drugs to treat more rare diseases with critical unmet needs.

- A new tracker evaluating the impacts of the IRA on rare disease drug development shows that, since passage of the law, 10 research programs have been discontinued and 7 drugs have been discontinued.

Today, the ORPHAN Cures Act is urgently needed to correct unintended consequences which are having concrete, negative impacts on innovation. This legislation will provide targeted solutions that (1) ensure that products that are exclusively for rare diseases remain protected from negotiation and (2) clarify that the timeline used to determine when an orphan drug may become eligible for negotiation begins at the product's first non-rare disease approval.

The Task Force is comprised of more than 40 organizations, and with the majority of its member organizations being patient advocacy organizations representing people with rare diseases and rare cancers. The Task Force is united in its mission to see the ORPHAN Cures Act enacted to protect research and development of new treatments for people with rare diseases.

Below are statements of support from individual Task Force members:

> "As a Spinal Muscular Atrophy patient with 25 years of clinical research experience, I know intimately that access to rare disease drugs—and even more so when it is *timely*—can be life-saving and life-transforming. My family started the SMA Foundation after my diagnosis in 2001, spearheading the now three safe and effective SMA drugs on the market, taking the disease from fatal to chronic. My parents were told at diagnosis I would live to college if I was lucky; instead, I graduated at the top of my class from Yale, have a full-time job, just got married, and am starting my doctoral studies in the fall. When I think of the 95% of rare diseases still untreated, I hope that SMA can underscore the importance of protecting the true potential of rare disease drug innovation, which is why I ardently support the introduction of the Optimizing Research Progress Hope And New Cures Act and thank Senators Barrasso and Heinrich for their bipartisan leadership. SMA has come far, but patients like me still need supportive treatment; by the time the first SMA treatment was available, I had lost the ability to walk, fight the common cold independently, and had taken 20 trips to the operating room. The ORPHAN Cures Act ensures that all rare disease patients in need of treatment are able to benefit from U.S. innovation to ultimately enjoy the same hopes, dreams, and lives as any other American is afforded." – **Arya Singh, public health researcher and rare disease advocate**

> "The Bonnell Foundation thanks Senators Barrasso and Heinrich for introducing the ORPHAN Cures Act. As the mother of two daughters living with cystic fibrosis, my hope is that policymakers will do what is needed to ensure that they have access to treatments specifically tailored to treat their mutations. The first step in ensuring access to these treatments is providing incentives so that researchers can innovate and build on successes of previously approved treatments. Without the ORPHAN Cures Act, there is little incentive to study a drug's impact on multiple rare diseases, and longstanding, bipartisan incentives that have benefited people with rare diseases – including my daughters – will be undercut. I highly encourage the

Committee to advance the provisions from ORPHAN Cures so that families like mine can have peace of mind." – **Laura Bonnell, CEO of the Bonnell Foundation**

"The orphan drug designation has been instrumental in providing life-saving treatments for people with rare cancers, with oncology having among the most orphan drug designations and approvals. The ORPHAN Cures Act will ensure this progress continues so that all people diagnosed with a rare disease can benefit from meaningful innovation." – **Kim Czubaruk, JD, Associate Vice President of Policy, Cancer*Care***

"The Chondrosarcoma Foundation supports introduction of the ORPHAN Cures Act in the Senate. Chondrosarcoma is a rare and aggressive bone cancer with no approved targeted therapies and limited treatment options beyond surgery. Patients often face delayed diagnoses, a lack of clinical trials, and minimal research investment due to the rarity of the disease. The ORPHAN Cures Act offers hope by addressing these systemic barriers and creating pathways to accelerate the development of treatments for ultra-rare conditions like chondrosarcoma. We urge continued bipartisan support to ensure patients are no longer left behind because their disease is too rare to attract innovation." – **Traci Hurley, MD, Executive Director, Chondrosarcoma Foundation**

"CureLGMD2i Foundation appreciates Senators Barrasso and Heinrich for their work to support the rare disease community through bipartisan introduction of the ORPHAN Cures Act. People with limb girdle muscular dystrophy (LGMD) – like my daughter – and their families rely upon the research and development incentives for orphan drugs that have led to a significant increase in FDA approvals in rare disease. Potential treatments that are currently in development for LGMD2I/R9 would not be possible without these incentives, and without urgent Congressional action, others with progressive genetic disorders will be left without the innovation needed to foster more treatments. We cannot allow these incentives to be undercut, and I urge other members of Congress to act swiftly to ensure that ORPHAN Cures Act becomes law." – **Kelly Brazzo, Co-Founder, President/CEO, CureLGMD2i Foundation**

"Many thanks to the cosponsors of the ORPHAN Cures Act. Your courage shows you understand how critical rare disease treatments are. As a patient- and care partner–led organization, we witness the devastating impact of rare diseases—and the compounded harm when no treatments exist. Second designations are vital: they fuel learning, reduce costs, and save lives. The Inflation Reduction Act unintentionally disrupted this, cutting second designations by 48% (from 12.1% to 6.3%). This drives up healthcare costs and, tragically, costs lives. I've seen this firsthand—my wife has a rare form of vasculitis. Without the medication she is on (second-designation treatment), she wouldn't survive more than five years. Please ensure the ORPHAN Cures Act is included in reconciliation." – **Pam Squires, Program Development Officer, Eosinophilic & Rare Disease Cooperative**

"We applaud the introduction of the ORPHAN Cures Act under the leadership of Senator Barrasso and Senator Heinrich. This legislative fix is critical to ensure that the development of therapies for rare cancer and disease communities is incentivized, whether it's for one or multiple indications. As the use of genetics and genomics in medicine has increased, we have seen tremendous growth in lifesaving targeted therapies for smaller populations. PARP inhibitors, for those with BRCA mutations in the hereditary ovarian and pancreatic cancer communities, are a prime example of success. Unfortunately, that progress is currently threatened; this legislation must be enacted to continue building on the advancements rare disease populations have experienced." – **Lisa Schlager, Vice President, Public Policy, FORCE: Facing Our Risk of Cancer Empowered**

"Firefly Fund supports strongly supports the Senate's introduction of the ORPHAN Cures Act. Time is of the essence when it comes to treating rare and ultra-rare genetic neurodegenerative disorders like Niemann-Pick Disease Type C (NPC). Children with NPC progressively lose their fine motor skills and muscle coordination, and decline cognitively, losing their ability to communicate with the world. Most children with the disease do not survive to adulthood. We thank Senators Barrasso and Heinrich for introducing legislation that will protect innovation into life-changing and life-saving treatments, and give people with rare diseases and their families hope for a better tomorrow." – **Pam Andrews, Executive Director, Firefly Fund**

"Introducing and passing the ORPHAN Cures Act is vital for the rare disease community, especially for those living with rare liver diseases. These patients often face years without a diagnosis, and even when they finally get one, they're met with a devastating reality: no approved treatments, limited research, and few clinical trials. The ORPHAN Cures Act helps protect the research incentives that make progress possible. For rare liver disease patients, this bill is about ensuring that necessary treatments can be adapted and repurposed for conditions beyond their original use. When research incentives are stripped away, innovation stalls, and patients with rare conditions are left behind. This legislation does not increase spending, instead, it safeguards what's already working. Continued investment in rare disease innovation drives job growth, strengthens the biomedical economy, and ultimately benefits all patients. It is essential to keep talking about the ORPHAN Cures Act and getting it across the finish line." – **Larry Holden, CEO of Global Liver Institute**

"The IgA Nephropathy Foundation thanks Senators Barrasso and Heinrich for their introduction of the ORPHAN Cures Act. People with rare diseases like IgA nephropathy deserve to have meaningful hope that research and development will result in new treatments, and longstanding incentives included in the Orphan Drug Act are critical to ensuring the future of innovative treatments. Without the ORPHAN Cures Act, those incentives will continue to be undercut, meaning fewer people with rare diseases will have access to life-changing therapies. We appreciate the leadership of the Senators in prioritizing the needs of people with rare diseases, and look forward to working with policymakers to ensure that ORPHAN Cures becomes law." – **Stuart Miller, Director of Strategic Relationships, IgA Nephropathy Foundation**

"Prevent Blindness applauds Senators Barrasso and Heinrich for introducing the ORPHAN Cures Act. On behalf of Americans across the lifespan who live with one of 550 known rare eye conditions, we are proud to join leading medical groups in support of this legislation for the promise and potential of innovative treatments for people who live with vision loss and eye disease resulting from a rare eye condition." – **Jeff Todd, President and CEO, Prevent Blindness**

"At United Porphyrias Association, we represent families living with the Porphyrias - some of the rarest and most painful diseases known to medicine. For conditions like erythropoietic protoporphyria, patients often wait a decade or more for a correct diagnosis, and even then, most have no approved treatment. The ORPHAN Cures Act is not just policy, it's a promise to the 95% of rare disease communities still waiting for a therapeutic breakthrough to go from isolation to living a full life. We're grateful to Senators Barrasso and Heinrich for championing this fix, and we urge Congress to act swiftly to preserve hope for millions." – **Kristen Wheeden, President, United Porphyrias Association**

The Save Rare Treatments Task Force thanks Senators Barrasso and Heinrich for their leadership in introducing the ORPHAN Cures Act. We urge Congress to advance this bill without delay to protect research and development for new treatments for millions of Americans.

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