Save Rare Treatments

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Save Rare Treatments Task Force Supports Including the ORPHAN Cures Act in House Budget Reconciliation Legislation

Patient-Focused Task Force Says Legislation is Critical to Protect Research and Development of New Treatments for Americans with Rare Disease

The Save Rare Treatments Task Force strongly supports the inclusion of the ORPHAN Cures Act (H.R. 946) as part of the House budget reconciliation package. The ORPHAN Cures Act is a bipartisan, critically necessary, targeted legislative fix to unintended consequences in current law. The ORPHAN Cures Act will help millions of Americans with rare diseases by restoring research and development incentives to develop the next generation of treatments and cures. Today, an estimated 30 million Americans have a rare disease, half of whom are children. Sadly, only 5 percent have an FDA-approved treatment for their disease. Without the policy changes in the ORPHAN Cures Act, more than 28 million Americans with rare diseases would have new treatments placed even further out of reach.

The Save Rare Treatments Task Force encourages the House Energy & Commerce Committee to advance the provisions of the ORPHAN Cures Act so that this needed legislation can eventually 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.

The Task Force seeks to correct the overly narrow Orphan Drug Exclusion in the Medicare Drug Price Negotiation Program to (1) ensure that products that are exclusively for rare diseases remain protected from negotiation and (2) clarify 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 Energy & Commerce Committee's legislative proposal includes technical fixes to both of these limitations, comprehensively addressing unintended consequences that will have a negative impact on rare disease research and development.

The Task Force is led by more than 25 patient advocacy organizations representing people with rare diseases and rare cancers who believe that the ORPHAN Cures Act is needed to protect innovation and promote access to treatments for people with rare diseases.

Below are statements from individual Task Force members:

Arya Singh, public health researcher and rare disease advocate, said: “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 Optimizing Research Progress Hope And New Cures Act in the reconciliation package. 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."

Laura Bonnell, CEO of the Bonnell Foundation, wrote: “The Bonnell Foundation applauds the Energy and Commerce Committee for including proposals designed to foster innovation for people with rare diseases. 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.”

Sally Werner, CEO of the Cancer Support Community, noted: “Cancer Support Community supports the inclusion of the Optimizing Research Progress Hope And New (ORPHAN) Cures Act in the reconciliation package. This legislation encourages the development and availability of life-saving and/or life-enhancing treatment for millions of Americans affected by rare cancers, ultimately increasing patient access to care through more treatment options that meet their unique needs, choices, and preferences."

Larry Holden, CEO of Global Liver Institute, said: “The ORPHAN Cures Act addresses a policy shift that unintentionally discouraged investment in therapies that could treat multiple rare diseases. This correction is vital, especially given that more than 100 rare liver diseases often share overlapping biological pathways, making research-driven drug repurposing a powerful and efficient approach for expanding treatment options. Scientific research consistently shows that targeting common mechanisms across diseases can accelerate therapy development and reduce

time to patient access. Including this language in the reconciliation process is more than a policy fix—it's a strategic investment in rare disease research that aligns with scientific best practices and comes at no additional cost. For patients who have waited far too long for even the hope of treatment, this is a meaningful step forward grounded in evidence-based policymaking.

Aki Smith, Executive Director of Hope for Stomach Cancer, emphasized the importance of including these provisions: “On behalf of the stomach cancer community, I urge Congress to retain the ORPHAN Cures Act language in reconciliation. Patients facing rare and aggressive cancers like stomach cancer often rely on orphan-designated therapies as their only remaining lifeline after exhausting standard treatment options. Without clear protections, they risk losing access to the very therapies that could prolong or save their lives. This is not about abstract policy—it’s about protecting patients who are already vulnerable and out of options. Congress must act now to preserve the intent of the Orphan Drug Act and ensure innovation continues to reach those who need it most.”

Pam Andrews, Executive Director of Firefly Fund, wrote: “Firefly Fund supports the inclusion of the ORPHAN Cures Act in reconciliation, and appreciates Congress prioritizing this solution to protect desperately needed innovation for children with rare diseases. 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. Policymakers have a responsibility to create an environment where life-changing and life-saving treatments can be developed, and where individuals with rare diseases and their families can have hope for a better tomorrow. The ORPHAN Cures Act is an important, critically needed step to incentivize new treatments.”

Lisa Schlager, Vice President of Public Policy at FORCE: Facing Our Risk of Cancer Empowered, noted: “We are thrilled to see the orphan drug IRA exclusion language included in the reconciliation package legislation. Ensuring that therapies for rare cancer and disease communities remain incentivized is crucial. We must continue encouraging innovation for rare patients and building on the successes we’ve seen as a result of the Orphan Drug Act.”

Kristen Wheeden, President, United Porphyrias Association, said: “With over 95% of rare diseases lacking an FDA-approved treatment, including the devastating types of porphyria we support, the ORPHAN Cures Act is essential. It restores critical incentives that drive research forward—especially for children and families who have waited too long for answers. At United Porphyrias Association, we know that innovation stalls when policy fails to protect it. This fix ensures that lifesaving progress continues for the 30 million Americans living with a rare disease.”

Stuart Miller, Director of Strategic Relationships at IgA Nephropathy Foundation, noted: "The IgA Nephropathy Foundation strongly encourages Congress to advance the provisions of the ORPHAN Cures Act included in the reconciliation recommendations. 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 Drug 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 Energy and Commerce Committee in prioritizing the needs of people with rare diseases, and look forward to working with policymakers to ensure that ORPHAN Cures becomes law."

Sarah Jones, Community Engagement with Eosinophilic & Rare Disease Cooperative (ERDC), wrote: "ERDC strongly supports the inclusion of the ORPHAN Cures Act in the reconciliation bill. As a patient- and care partner–led organization, we witness daily the urgent need for rare disease research and treatment access. For individuals with ANCA vasculitis, life expectancy without treatment is just 1 to 5 years. These treatments are not optional—they are the difference between life and death. The ORPHAN Cures Act is essential to ensuring continued innovation and access for those who need it most.

The Save Rare Treatments Task Force thanks the leadership of the House Energy and Commerce Committee for including The ORPHAN Cures Act in its budget reconciliation legislation and urges the Committee to advance these policies to protect research and development for new treatments for millions of Americans.

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