Save Rare Treatments

FOR IMMEDIATE RELEASE

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Patient-Led Task Force Celebrates the ORPHAN Cures Act Becoming Law

The Save Rare Treatments Task Force Thanks Congress for Passing Legislation to Protect Research Incentives for the Development of New Rare Disease Treatments

The Save Rare Treatments Task Force applauds Congress passing the ORPHAN Cures Act as part of H.R. 1, the One Big Beautiful Bill Act. The Optimizing Research Progress Hope And New (ORPHAN) Cures Act corrects unintended consequences of the Medicare Drug Price Negotiation Program that were reducing research and development for rare disease treatments. The Save Rare Treatments Task Force is grateful for Congress' action in enacting this critical legislative fix to restore long-standing incentives for the development of new treatments that will benefit Americans living with rare diseases.

While individual rare diseases impact 200,000 or fewer people in the U.S., the burden of rare disease is significant, with more than 30 million Americans living with an estimated 10,000 rare diseases and rare cancers. Only about 5 percent of people with a rare disease have an FDA-approved treatment option, leaving 28.5 million Americans waiting in hopes of innovation that will treat their disease.

Prior to the enactment of the ORPHAN Cures Act, incentives for research and development were weakened. The design of the Orphan Drug Exclusion in the Medicare Drug Price Negotiation Program was overly narrow, limiting protections only to drugs treating a singular rare disease and threatening decades of bipartisan incentives that drove research into secondary rare disease indications for rare disease drugs.

Evidence demonstrated a decline in research and development of new rare disease drugs. Research from the National Pharmaceutical Council showed a significant decrease in the percentage of second orphan designations, down 48 percent after the IRA's passage. This means that fewer investments were going toward expanded uses of FDA-approved drugs to treat more rare diseases with critical unmet needs. A tracker evaluating the impacts of the IRA on rare disease drug development showed that, since passage of the law, ten research programs have been discontinued and seven drugs have been discontinued.

The ORPHAN Cures Act restores healthy incentives for continued rare disease research and development. The legislation protects rare disease products exclusively approved for rare diseases from Medicare drug price negotiation and clarifies 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.

Below are statements of support from individual Task Force member organizations on the enactment of the ORPHAN Cures Act:

"The Alliance to Cure Cavernous Malformation celebrates the passage of the ORPHAN Cures Act. Cerebral cavernous malformations are abnormal blood vessels in the brain and spine and a leading cause of stroke and seizure in young adults. They can affect patients at any age, including children, and frequently runs in families. Currently, the only treatment is brain or spinal surgery. The ORPHAN Cures Act reduces barriers to rare disease drug development and offers hope to our families as we look for medical rather than surgical treatments." **– Connie Lee, CEO, Alliance to Cure Cavernous Malformation**

"I am incredibly grateful to Congress for protecting the 30 million Americans living with a rare disease—myself included—through the ORPHAN Cures Act. This legislation is lifesaving and protects the 42 years of rare disease drug discovery and treatment options already underway in the U.S. Thanks to Congress' actions to prioritize rare disease treatments, Spinal Muscular Atrophy (SMA) has gone from being fatal and the #1 genetic killer in infants to being a chronic disease with 3 safe, effective, and curative treatment options. It is incredibly comforting to know that the ORPHAN Cures Act will allow rare disease patients—with SMA and other diseases—to be afforded the same hopes and dreams as any other American, and that American innovation will continue to prosper." **– Arya Singh, public health researcher and rare disease advocate**

"I am the mother of two daughters with cystic fibrosis and the founder of The Bonnell Foundation: Living with cystic fibrosis (a national organization). We support the CF community and advocate for them. I know firsthand what's at stake. The ORPHAN Cures Act is critical to the future of rare disease treatment. The idea of losing access to lifesaving therapies is terrifying. We must not undermine the longstanding incentives that lead to the development of these treatments because without them, hope disappears. I urge you to stand up for the 30 million Americans living with a rare disease, and their families. If you don't know someone affected yet, you will." **– Laura Bonnell, CEO, The Bonnell Foundation**

"The passage of the ORPHAN Cures Act will ensure that the iterative nature of scientific progress isn't hindered, allowing for the development of rare disease treatments for patients suffering from conditions that have no available treatments. Promoting continued research will allow the life sciences sector to protect the pipeline of treatments for the millions of Americans living with rare diseases." **– Mike Guerra, President & CEO, California Life Sciences**

"Cancer*Care* celebrates Congress passing the ORPHAN Cures Act. The orphan drug designation has been critical 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 addresses a policy that would have undercut incentives to develop orphan drugs—including secondary indications for approved products—and ensures that 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***

"Cancer Support Community applauds Congress for passing the ORPHAN Cures Act as part of its reconciliation package. This legislation is critical for encouraging development of and patient access to life-saving and life-changing treatments for millions of Americans affected by rare cancers. People living with rare cancers deserve to have treatment options specifically designed for their disease and that meet their unique needs and preferences. The ORPHAN Cures Act ensures that improvements in innovation will continue to bring those treatments—and the hope they inspire—to millions of people with rare cancers." – **Sally Werner, CEO, Cancer Support Community**

"Passage of the ORPHAN Cures Act represents a major triumph for those with a rare cancer like cholangiocarcinoma, also known as bile duct cancer. We are extremely grateful to the United States Congress for recognizing and supporting the critical need for innovative drug development and treatment acceleration for rare cancers." **– Melinda Bachini, Chief Patient Officer, Cholangiocarcinoma Foundation**

"There is nothing worse for a patient and their family with a rare disease to be told there are No Treatment Options Left. Restoring incentives for rare disease drug development is a lifeline and brings hope where it is often scarce. The passage of the ORPHAN Cures Act restores a renewed sense of optimism to the chondrosarcoma community and the rare cancer field as a whole. We thank Congress for recognizing that lives are at stake and for making the right decision by passing the ORPHAN Cures Act." **– The Chondrosarcoma Foundation**

"ORPHAN Cures is an essential part of the reconciliation package and we thank Congress for passing it into law. Prior to ORPHAN Cures, the IRA only exempted drugs approved for a single rare disease. If a company found that the same therapy could help patients with a second rare disease, the drug risks becoming subject to price negotiations—often before companies have recovered their R&D costs. That's a huge disincentive to pursue additional research, even when the science is promising. And the damage is already evident. One analysis found a 48 percent decline in research into additional indications for orphan drugs since the IRA passed. That's not a statistic—that's children, families, and adults being told there's no hope. Their hope was lost not because the science failed, but because the incentive to pursue it was stripped away. ORPHAN Cures is critical to preserving the incentives which power hope." **– Pam Squires, Executive Director, Eosinophilic & Rare Disease Cooperative**

"FORCE and the hereditary cancer community applaud lawmakers for their support of the ORPHAN Cures Act. As the use of genetics and genomics in medicine grows, we will ultimately have smaller patient communities that may benefit from targeted—and potentially lifesaving—treatments. Generating a therapy for fewer than 200,000 people often isn't profitable. This is why incentives to develop therapies for not just one, but multiple rare cancer indications, are critical in bringing treatments to the patients who so desperately need them." **– Lisa Schlager, Vice President, Public Policy, FORCE: Facing Our Risk of Cancer Empowered**

"The ORPHAN Cures Act is a critical accelerator in the race against rare genetic pediatric neurodegenerative fatal diseases like Niemann Pick Type C. For Firefly Fund, passage of Orphan Cures means removing barriers and obstacles, empowering researchers and families, and fundamentally changing the landscape so we can deliver transformative treatments to the children who need them most, faster than ever before. This legislation provides families like ours with tremendous hope for the future, thanks to the United States Congress." **– Pam Crowley Andrews, Executive Director, Firefly Fund**

"The ORPHAN Cures Act offers a critical lifeline of hope to the rare disease community. There are over 10,000 rare diseases affecting patients throughout America, and this legislation sustains progress towards treatment for these individuals. By preserving incentives that support innovation for diseases with small patient populations, we move closer to life-saving therapies for those with rare liver diseases." **– Larry Holden, CEO, Global Liver Institute**

"The passage of the ORPHAN Cures Act represents a meaningful step toward equity in drug development for the rare disease community. For millions of patients still waiting for a treatment—and for every family who's ever been told there's 'nothing we can do'—this is more than policy; it's progress. As the daughter of a rare cancer survivor, I've seen how access to treatment can change the course of a life. We're deeply grateful to Congress for recognizing that hope should never be negotiable." **– Aki Smith, Founder & Executive Director, Hope for Stomach Cancer**

"The IgA Nephropathy Foundation appreciates Congress passing the ORPHAN Cures Act, ensuring that people with rare diseases like IgA nephropathy can have hope for new treatments designed specifically for their disease. As a person living with IgA nephropathy, I know firsthand the difficulty of uncertainty and the fear that comes from waiting for a treatment without knowing that it will be developed. Without the ORPHAN Cures Act, longstanding incentives for rare disease research and development would have continued to be undercut, meaning fewer people with rare diseases will have access to life-changing therapies. We appreciate Congress for its leadership in prioritizing the needs of people with rare diseases, and contributing to an environment that fosters both innovation and hope for the people that need it most." **– Stuart Miller, Director of Strategic Relationships, IgA Nephropathy Foundation**

"The Immune Deficiency Foundation (IDF) applauds the passage of the ORPHAN Cures Act, which will strengthen the development pipeline for therapies to treat every person affected by primary immunodeficiency (PI). PIs are a group of more than 550 rare, chronic, and life-threatening conditions in which part of the body's immune system is missing or does not function correctly. Many approved orphan drugs have applications across multiple rare diseases. Incentivizing further research into these therapies is both cost-effective and potentially lifesaving for patients who have few or no treatment alternatives. IDF strongly supports legislation that drives investment in orphan drug development so that individuals living with rare diseases have access to a full spectrum of effective treatments and a chance for a better quality of life."
– Jorey Berry, President & CEO, Immune Deficiency Foundation

"We are deeply grateful to Congress for passing the ORPHAN Cures Act, which ensures that potential treatments for LGMD2I/R9 and countless other rare diseases remain eligible for critical orphan drug development incentives. With this safeguard now in place, researchers and biotech companies can continue to pursue vital follow-on studies, bringing new hope for people affected by rare disorders. This is a testament to the power of collaboration and the relentless advocacy efforts of patients, families and organizations who are determined to find a brighter future for so many who are impacted by devastating rare conditions like the LGMDs." **– Kelly Brazzo, Co-Founder, President & CEO, CureLGMD2i**

"After years of dedicated advocacy, The Mended Hearts, Inc. is thrilled with the signing of the ORPHAN Cures Act into law. This legislation will facilitate the continued development of life-saving therapies for rare diseases, greatly benefiting our members and promising advancements in heart health. We extend our gratitude to those who prioritized and successfully enacted this important law." **– The Mended Hearts, Inc.**

"The ORPHAN Cures Act is critically important to the rare disease community. The lack of disease modifying treatments for 95 percent of those affected by a rare disorder is devastating. Accelerating the development and approval of treatments for rare diseases is crucial to those affected, their caregivers, and families. By providing incentives for pharmaceutical companies and streamlining regulatory pathways, the ORPHAN Cures Act encourages needed innovation and investment in research for conditions that affect small patient populations. We thank Congress for passing legislation that offers real hope for improved access to life-saving treatments and a better quality of life for individuals and families impacted by rare diseases." **– Leslie Baldwin, President & CEO, Michigan Rare Coalition, and an individual living with a rare disorder**

"The ORPHAN Cures Act is more than policy—it's hope. For families living with Neurofibromatosis and other rare diseases, this legislation protects innovation and accelerates treatments. Thank you, Congress, for upholding the belief that no one should be left without hope." **– Kim Bischoff, Executive Director, Neurofibromatosis Network**

"As a mother of two living with Primary Biliary Cholangitis—a rare, progressive disease with no cure—the ORPHAN Cures Act offers a vital sense of hope. This legislation is essential because it provides much-needed incentives for research and development of treatments and potential cures for rare diseases like mine. We thank Congress for passing this legislation, ensuring patients are no longer left behind. Every life affected by a rare disease deserves the promise of progress and the possibility of a future." **– Dr. Cecilia Duenas, Founder & CEO, PBC Research Foundation**

"The passage of the ORPHAN Cures Act is a monumental step forward for the rare disease community. For families like ours, this is more than policy—it is hope. Individuals living with Prader-Willi syndrome (PWS) and other rare diseases cannot afford delays in research or the loss of potential treatments due to harmful disincentives. We are grateful to Congress for listening to our voices and for passing legislation that protects innovation and prioritizes patients' futures." **– Dorothea Lantz, Director of Community Engagement, PWSA | USA**

"Rare Access Action Project (RAAP) is thrilled that Congress and the Administration recognized the importance of addressing the problems that were created by the Inflation Reduction Act by passing the ORPHAN Cures Act. By correcting provisions of the IRA and thus restoring incentives for innovation for needed rare disease treatments, we can ensure that lifesaving medicines will remain available for patients today and into the future. The inclusion of the ORPHAN Cures Act in H.R. 1 is an important win for rare disease patients." **– Michael Eging, Executive Director, Rare Access Action Project**

"Sarcoidosis is a rare disease that can impact any organ of the body and often impacts multiple organs at one time. We need more therapies to help manage this disease. Providing incentives for drug development in rare and chronic diseases like sarcoidosis are essential for creating paths to better therapies. Legislation like the ORPHAN Cures Act is essential for hope and a brighter future for those struggling with devastating chronic diseases." **– Tricha Shivas, Chief of Staff and Strategy, Foundation for Sarcoidosis Research**

"For porphyria patients, continued progress in research and drug development is not a luxury—it is a lifeline. We are grateful to Congress for recognizing this reality and for their commitment to including the ORPHAN Cures Act language in the budget reconciliation package. The recent passage of H.R. 1 will further incentivize innovative treatments and help ensure access to modern medicine for more than 30 million Americans living with rare diseases, including those with porphyria. We thank them for this renewed hope in our future." **– Kristen Wheeden, President, United Porphyrias Association**

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About Us: The Save Rare Treatment 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 is comprised of more than 40 organizations, 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 protect research and development of new treatments for people with rare diseases. Learn more about the Task Force at www.saveraretreatments.org.