Additional Support for the ORPHAN Cures Act:

"As a Spinal Muscular Atrophy (SMA) 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**

"Rare disease drug development is uniquely challenging, and a one-size-fits-all approach to policy can stymie innovation for the 30 million Americans living with a rare disease," **said Stacey Frisk, Executive Director of the Rare Disease Company Coalition.** "The ORPHAN Cures Act removes harmful barriers to innovation and opens the door for promising research, providing much-needed hope to the rare disease community. We thank Senators Barrasso and Heinrich for recognizing the needs of rare disease patients and championing this crucial bill."

"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 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**

"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**

"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**

"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**

"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**

“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**

“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 OCA is included in reconciliation.” **– Pam Squires, Program Development Officer, Eosinophilic & Rare Disease Cooperative**

“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 of Firefly Fund**