ORPHAN Cures Act: Protecting New Treatments for Patients with Rare Diseases

The ORPHAN Cures Act reaffirms Congress's strong, bipartisan commitment to protect the research and development of new treatments for patients with rare diseases. A rare disease is one that affects fewer than 200,000 people, and while that may seem small, the facts show rare diseases are far from rare.

More than **30 million Americans** have a rare disease.

More than **10,000 rare diseases** have been identified.

Children make up nearly 50% all rare disease cases.

Only **5% of rare diseases** have an approved treatment.

For more than 40 years, policymakers have recognized the unique challenges of developing treatments for rare diseases and put in place bipartisan policies to incentivize their R&D. These efforts have been a resounding success, leading to approval by the FDA of hundreds of treatments for rare diseases.

But future progress is at risk if Congress fails to act. The government price setting policies in the Inflation Reduction Act (IRA) are making it more difficult to develop new treatments for rare diseases.

- **The law upends incentives that encourage new drug development**. The IRA allows a single government agency to set prices for new medicines. While the law exempts medicines approved for one rare disease, many rare disease treatments are still at risk of government price setting. The law forces biotech companies to make difficult decisions, such as reconsidering rare disease research.
- **The IRA discourages critical post-approval R&D**. After a rare disease treatment is approved, research companies often pursue additional research post approval to learn if more patients could benefit. Between 2006 and 2012, more than 60% of medicines approved as orphan drugs later received one or more post-approvals. The IRA discourages this research by imposing government-set prices on medicines approved for multiple rare disease indications.
- **The IRA creates a perverse incentive to abandon pediatric research**. Research often starts in adults and is later expanded to include children. The IRA discourages this follow-on research because any later approval for children would subject the medicine to price controls.

The ORPHAN Cures Act (H.R. 946), introduced by Reps. Don Davis (D-NC) and John Joyce (R-NC), is a bipartisan solution that will protect new treatments for patients with rare diseases.

- **The bill strengthens long-standing incentives for rare disease drug development** by exempting from government price setting medicines approved for "one or more rare diseases or conditions."
- **Only treatments for rare diseases are protected by the ORPHAN Cures Act**. The bill only applies to treatments approved for one or more rare diseases or conditions – nothing more.
- **Protecting treatments for rare diseases will have almost no impact on Medicare**. This narrow fix will impact just 2% of what Medicare was projected to save through government price setting.
- **The bill maintains the number of drugs selected for government price setting**. This bill does not change how many medicines are eligible for government price setting and how they are selected.

The IRA was meant to protect treatments for rare diseases, but the law doesn't reflect how rare disease medicines are developed and threatens future progress. **The ORPHAN Cures Act reaffirms Congress's bipartisan commitment to rare disease patients and to developing the new treatments they need**.