The ORPHAN Cures Act is bipartisan legislation that would maintain existing incentives and boost research into new treatments for the 30 million Americans currently suffering from one of more than 7,000 rare diseases. The bill would achieve this by amending the Inflation Reduction Act’s harmful orphan drug exclusion and incentivizing critical follow-on investment into rare disease drug development.

Background: The Rare Disease Drug Market

A rare disease is one that affects fewer than 200,000 people in the United States. By definition, treatments for rare diseases — also known as “orphan drugs” — target narrow patient populations, making them particularly risky investments without special economic incentives.

- Key incentives stem from the 1983 Orphan Drug Act, which established a tax credit to help developers cover clinical trial costs and extended market exclusivity for FDA-approved orphan drugs.
- Even so, more than 90% of rare diseases have no FDA-approved treatment, underscoring the need to protect the incentives drug manufacturers rely on to research and develop medicines targeting rare conditions.

The Issue: A Harmful Provision of the Inflation Reduction Act

In the IRA, Congress recognized the need to preserve incentives for orphan drug development and excluded orphan drugs from Medicare price negotiations. However, this exemption only applies to drugs treating a single rare disease.

- Orphan drugs initially developed and approved for one condition often prove effective against other rare diseases following additional clinical testing.
- The IRA disincentivizes researchers and investors from pursuing costly follow-on research to find new orphan designations and approvals because, if their efforts prove successful, the drug would no longer be exempt from government price controls.
- The provision may leave millions of Americans without access to critical medicines.
- Incentives for orphan drug development should be structured to maximize the potential clinical benefit of each new medication, ensuring that patients with rare diseases have the broadest possible access to effective treatments.

The Solution: Pass the ORPHAN Cures Act

To protect the incentives necessary for drug manufacturers to research and develop treatments for rare diseases, Congress must pass the ORPHAN Cures Act.

- The ORPHAN Cures Act would amend the IRA to ensure orphan drugs treating one or more rare diseases or conditions are excluded from Medicare price negotiations.
  - The legislation clarifies that the countdown to eligibility for price negotiation would only begin when an orphan drug loses this exclusion.
- By passing the legislation, Congress can encourage follow-on investment into orphan drug development and preserve hope for millions of Americans living with a rare disease or condition.