THE ORPHAN CURES ACT

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.

The Optimizing Research Progress Hope And New (ORPHAN) Cures Act (H.R. 5539) was introduced in the U.S. House by Representatives by John Joyce, M.D. (R-PA-13) and Wiley Nickel (D-NC-13).