PATIENTS FOR AFFORDABLE DRUGS NOW™

ORPHAN Cures is **Unnecessary** for Innovation & Will **Drive Up** the Price of Drugs

Americans pay 3 - 8 times more than people in other wealthy nations pay for the exact same brand-name drugs. Consequently, 1 in 3 Americans report difficulty affording their prescriptions.

Passed in 2022, the Inflation Reduction Act (IRA) allows Medicare to negotiate prices with drug makers for the first time in history, ensuring a better deal for people with Medicare and taxpayers. The first round of negotiations secured an average list price reduction of 62% for 10 of the costliest drugs in Medicare - saving nine million patients an estimated $1.5 billion in 2026, while taxpayers will save $100 billion by 2031. Now, some in Congress want to roll back this progress. **The ORPHAN Cures Act (H.R. 946) would create a new loophole to benefit pharmaceutical corporations by excluding drugs with more than one orphan designation from negotiation** – keeping more drugs from being negotiated and prices artificially high for patients.

An orphan drug designation applies to a treatment for conditions affecting fewer than 200,000 people in the United States. However, drugs with multiple orphan indications often reach much larger patient populations and generate significant revenue in both public and private markets.

1 in 3
Americans report difficulty affording their prescriptions

$1.5B
savings for patients on first 10 drugs in 2026

62%
average list price reduction on the first 10 negotiated drugs

$100B
taxpayer savings by 2031 because of Medicare negotiation

We don't need the ORPHAN Cures Act to protect innovation.

All existing incentives for orphan drug development are maintained.
The IRA did not change or remove longstanding incentives to develop orphan drugs, such as tax credits for clinical trials and exemptions from user fees, which lower drugmakers' development costs.

Orphan drugs indicated for only a single rare disease are already excluded from negotiation.
If a drug is approved for only one orphan condition, it is not eligible for Medicare price negotiation.

Drugs with multiple indications, including orphan indications, are already highly profitable.
These drugs already expand beyond rare disease populations and bring in significant revenue, making additional exemptions unnecessary.

The highest expenditure drugs will be the products selected for negotiation.
Only a small percentage of drugs approved for more than one orphan disease gross over $200 million under Medicare each year, the threshold for negotiation eligibility. If a company is making $200 million per year from Medicare on a product, they have received their return on investment in the drug's development.

Roughly 50 percent of rare disease patients are children.
Medicare largely serves adults who are over the age of 65 or live with disabilities. With limited exceptions, children do not qualify for Medicare. Treatments for rare diseases with a majority pediatric population are therefore unlikely to be selected for negotiation.

Small biotech firms are excluded from negotiation until 2028.
Much rare disease drug development takes place within small biotech companies.