

BIG PHARMA **HIKES** **PRICES ON RARE DISEASE** **DRUGS** — AS MEDICARE PROTECTIONS ARE WEAKENED

1 IN 10

Americans live
with a rare disease

17X

Rare disease drugs are 17 times
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200

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In January, **pharmaceutical companies raised prices** on more than 200 drugs used to treat rare diseases, with **over 80% of those hikes exceeding inflation**.

These increases are not happening in a vacuum. They come as **Congress has weakened Medicare drug price negotiation for rare disease drugs**, giving pharmaceutical corporations and their allies new incentives to raise prices while facing fewer consequences.

RARE DISEASE DRUGS ARE AMONG THE **MOST EXPENSIVE**

Rare disease drugs are among the most expensive drugs on the market — in large part because of how they are treated under federal law.

In the U.S., a rare disease is defined as a condition affecting fewer than 200,000 patients. Yet while each condition is rare, an estimated **1 in 10 Americans lives with a rare disease**. Drugs approved to treat rare diseases — often referred to as orphan drugs — receive special market protections that limit competition and pricing oversight. As a result, the median cost of a rare

disease drug is more than 17 times higher than that of drugs that do not treat rare conditions.

However, many drugs labeled “orphan” are not limited to a single rare population. About **15% of orphan drugs have multiple rare disease approvals**. These drugs treat multiple rare conditions, generate billions in revenue, and face little to no competition — allowing manufacturers to maintain extraordinarily high prices year after year.

THE ORPHAN CURES ACT **MAKES THE PROBLEM WORSE**

Instead of closing gaps in the system, Congress widened them. The **ORPHAN Cures Act**, which passed as part of a 2025 reconciliation package, delivered an **\$8.8 billion giveaway to the pharmaceutical industry** by allowing blockbuster rare disease drugs that treat more than one orphan condition to avoid Medicare negotiation.

Without ORPHAN Cures, tens of thousands of patients on Medicare would have benefited from lower negotiated prices.

MEDICARE NEGOTIATION WORKS – BUT ORPHAN CURES UNDERMINES IT

In January 2026, CMS announced the next 15 top-selling drugs selected for round three of Medicare price negotiation. But some of the most expensive drugs were missing from the list.

Blockbuster cancer drugs Keytruda (\$12,031.36 / dose) and Opdivo (\$7,787 / dose) were widely expected to be selected, but were excluded because the ORPHAN Cures Act reset the negotiation clock until a drug receives its first non-orphan indication. That delay keeps prices high for patients — and profits flowing for drugmakers.

PRICE HIKES ON DRUGS PREVIOUSLY ELIGIBLE FOR MEDICARE NEGOTIATION

Pharmaceutical companies are already raising prices on drugs that would have been eligible for negotiation before the passage of ORPHAN Cures. Examples include:

FIRDAPSE (CATALYST)

Condition(s): Lambert-Eaton Myasthenic Syndrome (LEMS)

New price: \$63,684.77

2026 increase: \$4,717 (8.0%) per 240-tablet package

TIBSOVO (SERVIER)

Condition(s): Rare cancers (cholangiocarcinoma, AML)

New price: \$35,378.25

2026 increase: \$1,229 (3.6%) per 60-tablet package

BRUKINSA (BEIGENE)

Condition(s): Blood cancers (LBCL, CLL)

New price: \$16,673

2026 increase: \$929 (5.9%) per 60-tablet package

JUXTAPID (CHIESI USA)

Condition(s): homozygous familial hypercholesterolemia

New price: \$57,936.70

2026 increase: \$2,228 (4.0%) per 28-tablet package

TYVASO (UNITED THERAPEUTICS)

Condition(s): Pulmonary arterial hypertension

New price: \$26,155.15

2026 increase: \$1,457 (5.9%) per starter kit



These are not niche drugs. They are high-revenue products with limited competition — and are now shielded from Medicare negotiation.



For patients like Ginny, who lives with LEMS and relies on Medicare, that protection for drugmakers has real consequences. Her medication, Firdapse, would have been eligible for negotiation before the ORPHAN Cures Act. Now, it is not.

Congress removed one of the only tools available to lower the cost of the very drugs she relies on. **The ORPHAN Cures Act does not protect patients with rare diseases. It protects pharmaceutical profits.**

Congress must reject further industry efforts — including proposals like the EPIC and MINI Acts — that would carve out additional exemptions and further weaken Medicare's ability to negotiate lower drug prices.