Save Rare Treatments

The *Orphan Drug Act* spurred never-before-seen investments in rare disease research and development (R&D) resulting in a:

1,576%
increase in the number of FDA-approved orphan drugs from

38 TO 600+
therapies to treat more than

1,100
Indications

WHAT IS AN ORPHAN DRUG?

An orphan drug is a therapy used to treat a rare disease affecting **200,000 or fewer people**. This designation was established by the landmark *Orphan Drug Act* (*ODA*) of 1983, which has benefited millions of Americans living with rare diseases through incentives including:

- **ORPHAN DRUG TAX CREDIT**
- **FDA APPLICATION USER-FEE WAIVER**
- **ORPHAN DRUG EXCLUSIVITY**
- **FDA RESEARCH GRANTS**

THE NARROW ORPHAN DRUG EXCLUSION IN THE MEDICARE DRUG PRICE NEGOTIATION PROGRAM WILL HALT THIS PROGRESS

Under the Medicare Drug Price Negotiation Program's narrow Orphan Drug Exclusion provision, an orphan drug is only excluded from price negotiation if it is only approved for one rare disease or condition. Much of the rare disease development pipeline and the progress over the last 40 years has relied on identifying new applications for existing drugs that could address unmet needs for patients living with rare diseases.

THE POLICY DISINCENTIVIZES RESEARCH INTO EXISTING FDA-APPROVED ORPHAN DRUGS ON MULTIPLE RARE DISEASES:

1. Manufacturers take on **substantial risk** to research second indications due to genetic-based therapies' uniquely **limited ability** to target additional indications. However, this is a critical aspect of **expanding access** to treatments for rare disease patients.

2. Under the current policy, once an orphan therapy is designated by FDA for research on an additional rare disease, the drug is no longer excluded from negotiation, meaning companies absorb **even greater financial risk** to determine if a drug is effective in treating additional rare diseases.

3. If this remains, investing in an additional indication for a subsequent rare disease **may no longer be feasible** due to the newly associated **revenue penalties** that now result in **greater financial loss**.

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THE POLICY WILL REDUCE PATIENT ACCESS TO RARE DISEASE THERAPIES

A JAMA study showed that, of the 282 novel orphan drugs approved from 2003 to 2022, FDA approved 63 (23%) for at least 1 indication, including 29 (10%) for multiple indications.

92 (61%) of these additional therapies were for orphan conditions.

Source: https://jamanetwork.com/journals/jamanetworkopen/fullarticle/2808362

BASED ON A STUDY THAT ESTIMATED THE IMPACT OF THE ORPHAN DRUG EXCLUSION PROVISION ON ORPHAN DRUG THERAPIES, THIS PROVISION WILL:

Culminate in a reduction of **more than 40%** of FDA approvals from **34 to just 14** new orphan drugs from 2026 to 2035.

Reward companies that **delay market entry by up to 3 years** and focus on a non-orphan indication to avoid the penalties and losses now associated with an accelerated orphan approval.

Most directly impact **oncology therapies**, which make up **92% of 2nd orphan indications (7000+ therapies)**, putting the usual approach to expanding access to oncology therapies at risk.

Source: https://vitaltransformation.com/2023/05/iras-impact-on-the-us-biopharma-ecosystem/

SOLUTION: THE ORPHAN CURES ACT

Reps. John Joyce (R-PA-13) and Wiley Nickel (D-NC-13), and Sens. John Barrasso (R-WY) and Tom Carper (D-DE), have introduced targeted legislation to amend the Orphan Drug Exclusion so that it aligns with the incentives in the *ODA*. Other House cosponsors of the legislation (as of January 2023) include Reps. Donald Davis (D-NC-1), Kevin Hern (R-OK-1), Scott Peters (D-CA-50), Gus Bilirakis (R-FL-12), Josh Gottheimer (D-NJ-5), Lloyd Smucker (R-PA-11), and Mariannette Miller-Meeks (R-IA-1). The *Optimizing Research Progress Hope and New Cures Act (ORPHAN Cures Act)* will:

- **Improve the Orphan Drug Exclusion, ensuring that protections remain to incentivize research into multiple rare diseases.** This correction would allow orphan products to remain excluded from negotiation so long as their FDA approved uses are exclusively for rare diseases.
- **Clarify the timeline used to determine when an orphan drug may become eligible for negotiation.** This provision clarifies that the negotiation eligibility clock starts for an orphan product once it loses the exclusion due to approval to treat a non-rare disease.

For more information or to cosponsor H.R. 5539/S. 3131, please contact matt.tucker@mail.house.gov in Rep. Joyce's office, rachel.kline@mail.house.gov in Rep. Nickel's office, maddison_dillon@barrasso.senate.gov in Sen. Barrasso's office, or victoria_carle@carper.senate.gov in Sen. Carper's office.