

PATIENTS FOR AFFORDABLE DRUGS NOW™

February 6, 2025

Dear Member of Congress:

I write on behalf of Patients For Affordable Drugs Now to express our strong opposition to the ORPHAN Cures Act and the Ensuring Pathways to Innovative Cures Act. We urge you to oppose these bills, which would undermine Medicare negotiation as established in the Inflation Reduction Act (IRA), allow drug companies to extend monopolies, and drive up prices for patients – including for those with rare diseases like myself – which is exactly what the IRA aims to stop.

[Patients for Affordable Drugs Now](#) is an independent, bipartisan organization that focuses exclusively on policies to lower prescription drug prices and does not accept funding from any organizations that profit from the development or distribution of prescription drugs. We help educate and mobilize patients in support of legislation to ensure our system serves patients and consumers—not just those who profit from it. Since our founding seven years ago, we have collected over [36,000 stories](#) from patients across all 50 states struggling with high drug costs.

Americans pay four to eight times what people in other wealthy nations pay for the exact same brand-name drugs. Consequently, [three in ten Americans report](#) difficulty affording their medications. We support a system that ensures both the innovation we need and prices we can afford. The IRA strikes that balance.

Under the IRA, a drug approved for one orphan disease is exempt from negotiation – a measure that promotes innovation. However, if a drug company then decides to pursue additional indications for that medication, the drug can become eligible for negotiation depending on its price and utilization. **The ORPHAN Cures Act would undermine the balance achieved in the IRA by extending the negotiation exemption to drugs approved for more than one indication, allowing manufacturers to prolong their monopolies and inflate prices indefinitely.** Critically, drugs with additional indications generate higher revenue through expanded patient populations in both the public and private sectors. Drug companies don't need additional exemptions from negotiation to be profitable. And in reality, most true orphan drugs are unlikely to be selected for negotiation, as products with small patient populations are very unlikely to rank among the Medicare drugs with the highest gross expenditures. Importantly, all current incentives for orphan drug development are maintained, including tax credits for clinical trials and waived user fees. In addition, roughly 50 percent of patients with rare disease are children and are therefore unaffected by Part D Medicare negotiation.

We also urge you to oppose the Ensuring Pathways to Innovative Cures Act, which would extend the IRA's negotiation exemption period for small molecule drugs from nine to thirteen years. The claim that the IRA will stifle the development of small-molecule drugs is not supported by facts and is inconsistent with the pharmaceutical industry's long-held positions on the need for advantageous treatment for biologic drugs. To start, the only reason the U.S. has longer exclusivity periods for biologics is because drug manufacturers [lobbied aggressively](#) for them, insisting biologics are more expensive, risky and take longer to develop. In fact, most high-income countries provide small-molecule drugs and biologics with identical periods of

market exclusivity. In addition, since the enactment of the IRA, investment in small molecules has increased, not declined. **In the nine months following the passage of the IRA, big drug companies [acquired more small-molecule drugs](#) than in the nine months prior.** There is simply no evidence that drug development has slowed following the signing of the law.

The simple fact is that **healthy profit is guaranteed on safe and effective small-molecule drugs** because the IRA allows drug companies to set launch prices, and to increase those prices in subsequent years. This is the principal mechanism we use to reward risk and investment in drug development, and nothing in the IRA changed that core element of our system. It is impossible to see how any well-run business will not be able to make a healthy return under the current Medicare negotiation parameters when it can set the price, increase the price and not face negotiation for nine years. If manufacturers truly want parity between small molecules and biologics, both should be subject to negotiation after nine years—not thirteen.

The IRA carefully balances rewarding innovation while ensuring fair profits and promoting public health. The ORPHAN Cures Act and the Ensuring Pathways to Innovative Cures Act would tilt that balance in favor of greater profit at the expense of patients and taxpayers. We urge you to oppose these harmful bills.

Sincerely,

A handwritten signature in black ink, appearing to read "David Mitchell". The signature is fluid and cursive, with the first name "David" being larger and more prominent than the last name "Mitchell".

David Mitchell
President and Co-Founder
Patients for Affordable Drugs Now