



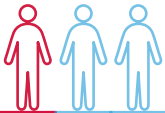
Big Drug Company Tactics Undermine Drug Pricing Reforms: The Case Against Orphan Drug Exemptions

To address ever-increasing prescription drug costs, federal and state policymakers have instituted reforms ranging from Medicare price negotiations to state Prescription Drug Affordability Boards (PDABs). But at every turn, big drug companies employ a barrage of tactics to undermine efforts to improve drug affordability in order to protect their outsized profits. That is exactly what is driving recent efforts to exempt certain drug classes, such as those for “orphan drugs,” from pricing reforms. While big drug companies try to argue that these exemptions are aimed at supporting people dealing with rare diseases, the end result is quite the opposite: these exemptions only serve to keep prices high at the expense of people who most need access to affordable prescription drugs.

The impact of high and rising drug prices — and the promise of new pricing reforms

Millions of Americans are increasingly concerned about the cost of everyday necessities like food, housing and health care. Prescription drugs are — as they have been for decades — a major source of these rising costs, both at the pharmacy counter and in the form of higher premiums and higher cost-sharing.¹ Almost one in every three adults report not taking their medications as prescribed due to cost² and an estimated 125,000 deaths a year happen because people do not take their drugs as prescribed.³

1 IN 3



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Drug pricing reforms are extremely popular: 7 in 10 voters support Medicare negotiation, and more than 90% of voters supporting government action to lower drug prices.

Just as these costs consume ever-increasing portions of family's budgets, the federal government and states also pay a significant and growing portion of high drug costs. Medicaid drug spending in states has increased 72%, from \$30 billion in 2017 to \$51 billion in 2023,⁴ and the nonpartisan Congressional Budget Office (CBO) estimates that by the end of 2025 spending on Medicare Part D drug benefits will reach \$137 billion.⁵

Effectively and robustly addressing high drug costs is critical for families and key to stopping big drug companies from price-gouging governments and taxpayers. Policy reforms like the Inflation Reduction Act (IRA) Medicare Drug Price Negotiation Program and the establishment of state PDABs with upper payment limits (UPLs) have the power to lower drug prices for the individuals who rely on them. When the new Medicare-negotiated prices on the first 10 drugs go into effect in 2026, Medicare beneficiaries are expected to save \$1.5 billion, in addition to the savings they are already getting through other IRA drug pricing reforms that cap out-of-pocket spending.⁶ Maryland and Colorado PDABs are set to release their first UPLs this fall, which is likely to show significant savings for their states as well.

These reforms are extremely popular: with 7 in 10 voters supporting Medicare negotiation, and more than 90% of voters supporting government action to lower drug prices.⁷ So, rather than trying to overturn or repeal these reforms in their entirety, big drug companies and their allies seem to have realized they can more easily succeed with a “death by a thousand cuts” strategy and advocate for excluding certain classes of drugs from such reforms. A recent example can be seen in H.R. 1, federal budget legislation that slashed health care coverage and included a special carve-out for drug companies that further limited what kinds of “orphan drugs” were exempt from Medicare Drug Price Negotiation.

For drug pricing reforms to be able to effectively lower drug costs for all our nation's families, all drug types must remain under consideration for reforms. If drug companies continue to be successful in moving the goal posts and narrowing which drugs are even eligible for pricing reforms, we will easily find ourselves in a reality where the only drugs eligible for lower costs are functionally handpicked by big drug companies.

Orphan drugs exclusions: expensive and unnecessary

“Orphan drug” is a classification under federal law for drugs that treat small populations of people with rare diseases or conditions, and they can provide a lifeline for people with very limited treatment options.⁸ But orphan drugs are also some of the most expensive drugs on the market, with some 25 times more expensive than non-orphan drugs. In 2017, the average annual cost for an orphan drug was \$186,758.⁹ Some orphan drugs can cost more than a million dollars a year.¹⁰

ORPHAN DRUG PRICE EXAMPLES

Zokinvy

(Eiger BioPharmaceuticals) treats Hutchinson-Gilford progeria syndrome.

- Annual list price of **about \$1.7 million** (2023)¹¹
- Approximately 400 to 450 children and young adults worldwide live with progeria.¹²

Trikafta

(Vertex Pharmaceuticals) treats cystic fibrosis.

- Annual list price **more than \$300,000** (2023)¹³
- Approximately 40,000 children and adults live with cystic fibrosis in the United States, and an estimated 105,000 people have been diagnosed with CF across 94 countries.¹⁴

Orphan drugs are becoming a larger, more expensive share of the market

These expensive drugs are also quickly becoming a larger share of the drug market. Food and Drug Administration (FDA) approvals of orphan drugs have risen rapidly in the past decades. Between 1998 and 2023, orphan drug approvals rose from only 10% of all drug approvals to 43% of new drugs.¹⁵ Meanwhile, the share of traditional drug approvals in that same time period declined from 65% to only 20%.¹⁶ This is due in large part to the fact that many orphan drugs can also be used to treat non-rare diseases.¹⁷ In fact, twenty percent of orphan drugs have approval for both a rare disease and a common disease, meaning the companies that make and distribute those drugs can make significant sales to the larger general public.¹⁸ And these drugs do well on the market: A 2021 study showed that of the top 200 selling branded drugs in the world that year, 73 were orphan drugs.¹⁹

Taxpayers already subsidize orphan drug research and development

People with rare diseases and their families need drug companies to invest in the development of new therapies that give new hope to those with limited or no treatment options. Drug companies leverage that fact when they argue that reforms to rein in high and rising drug prices would limit their ability to develop cures or treatments for rare diseases.²⁰

Yet for decades, drug companies have received significant incentives for investment in orphan drug research and development established through the Orphan Drug Act of 1983,²¹ including:

- Seven years of exclusivity to protect them from generic competition.²²
- Expedited access to the market through programs like Accelerated Approval Program, Breakthrough Therapy Designation, Fast Track Designation, and Priority Review Designation, which aim to help get new medications that might meet unmet medical need or that offer significant improvements to treatment options.²³
- Tax credits of 25% on qualified clinical trials, which make it more affordable for drug companies to develop orphan drugs and try to get them on the market.²⁴

Taken together, these incentives ensure that drug companies receive significant financial support from the federal government before an orphan drug even goes onto the market. Once they do, they often bring in record profits with gross profit margins of more than 80% in the rare disease industry.²⁵ One study published in 2023 notes that among the sole orphan drugs they assessed, the median drug was projected to earn **\$21.9 billion in revenue** before it could ever become eligible for Medicare price negotiation, negating the concerns made by drug companies on the need for exemptions from reforms.²⁶ And while drug companies who receive exemptions from pricing reforms for orphan drugs protect their profits, families who rely on these medications are left paying high prices that risk their health and financial security.

Exempting classes of drugs makes them more expensive and inaccessible

Drug companies charge exorbitantly high prices for their drugs even though they have benefited from significant financial incentives to research and develop them. And the people who rely on those drugs to treat chronic, rare health conditions — along with anyone who pays into the health care system — are paying the price. State and federal drug price reforms will only be able to provide rare disease communities with needed relief if those drugs, like those for more common diseases, are eligible for lower prices.

Policymakers should oppose drug companies' continued efforts to circumvent these important reforms in order to maintain the predatory, profiteering behaviors that necessitated in the first place the creation of solutions like the Medicare Drug Negotiation Program and the setting of upper payment limits by PDAB authorities.

Drug companies' quest to get carve-outs for specific or unique classifications of drugs is akin to calling for full repeal of the reforms themselves, and lawmakers should view each of these attempts with appropriate skepticism.

Endnotes

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