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Cost of Exempting Sole Orphan Drugs From Medicare Negotiation

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IMPORTANCE The Inflation Reduction Act (IRA) requires Medicare to negotiate prices for some high-spending drugs but exempts drugs approved solely for the treatment of a single rare disease.

OBJECTIVE To estimate Medicare spending and global revenues for drugs that might have been exempt from negotiation from 2012 to 2021.

DESIGN, SETTING, AND PARTICIPANTS This cross-sectional study analyzed drugs that met the IRA threshold for price negotiation (Medicare spending >$200 million/y) in any year from 2012 to 2021 and had an Orphan Drug Act designation. We stratified drugs into 4 mutually exclusive categories: approved for a single rare disease (sole orphan), approved for multiple rare diseases (multiorphan), initially approved for a rare disease and subsequently approved for a nonrare disease (orphan first), and initially approved for a nonrare disease and subsequently approved for a rare disease (non–orphan first).

OUTCOMES The primary outcomes were the number of sole orphan drugs, estimated Medicare spending on those drugs from 2012 to 2021, and global revenue since launch.

RESULTS Among 282 drugs, 95 (34%) were approved to treat at least 1 rare disease, including 25 sole orphan drugs (26%), 20 multiorphan drugs (21%), 13 orphan first drugs (14%), and 37 non–orphan first drugs (39%). From 2012 to 2021, Medicare spending on sole orphan drugs increased from $3.4 billion to $10.0 billion. Each year, a median (IQR) of $2.5 ($1.9-$2.6) billion in Medicare spending would have been excluded from price negotiation because of the sole orphan exemption. The cumulative global revenue of the median (IQR) sole orphan drug was $11 ($6.6-$19.2) billion.

CONCLUSIONS AND RELEVANCE The sole orphan exemption will exclude billions of dollars of Medicare drug spending from price negotiation. The high level of global revenues achieved by these drugs, however, suggests that special exemption is unnecessary for them to achieve financial success. Congress could consider removing the sole orphan exemption to obtain additional savings for patients and taxpayers and to eliminate any potential disincentive for developing additional indications for these drugs.

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Supplemental content

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The Inflation Reduction Act (IRA) requires Medicare to negotiate lower prices for certain drugs with more than $200 million per year in Medicare spending. The first 10 drugs were selected on August 29, 2023, with negotiated prices to take effect in 2026.[1] The IRA exempts several categories of drugs from negotiation, including drugs that treat a single rare disease. The law adopts the definition of a rare disease from the Orphan Drug Act, which provides incentives to develop drugs treating conditions affecting fewer than 200 000 individuals in the US and drugs with "no reasonable expectation" that revenues will be sufficient to offset development costs.[2,3] The original purpose of the Orphan Drug Act, and presumably the new IRA exemption, was to incentivize manufacturers to develop treatments for rare diseases that may not be profitable enough for companies to otherwise pursue.

To be eligible for the IRA sole orphan exemption, a drug may have been granted only a single Orphan Drug Act designation by the US Food and Drug Administration (FDA) and must be approved exclusively for indications within that designation. The distinction between a "designation" and an "indication" is critical. An Orphan Drug Act designation specifies a rare disease or condition (eg, *EGFR*-mutated non–small cell lung cancer). The FDA can grant such designations at any time, including before a drug has been submitted for approval. An Orphan Drug Act indication refers to a specific labeled use of a drug in treating a designated disease (eg, first-line treatment of metastatic *EGFR*-mutated non–small cell lung cancer).

Congress limited the scope of the sole orphan exemption to ensure that the many top-selling drugs that are used to treat both common and rare diseases remain eligible for Medicare

negotiation. Drugs with more than 1 Orphan Drug Act designation or with nonorphan indications do not qualify for exemption. By 2020, nearly half of drugs approved by the FDA,[4] and most of the highest-spending drugs in Medicare, had at least 1 Orphan Drug Act designation. Among drugs with both rare and nonrare indications, the nonrare indications account for most of the drugs' sales.[5,6] Drugs with Orphan Drug Act designations have similar revenues and lower development costs compared with drugs without Orphan Drug Act designations.[7,8]

We sought to understand the number of drugs that will qualify for the IRA exemption from price negotiation, the effect of this exemption on Medicare spending, and the revenue potential of these drugs. We performed a cross-sectional analysis of drugs that would have been eligible for the sole orphan exemption had the IRA been in effect starting in 2012. We tabulated Medicare spending and global revenue for these drugs.

Key Points

Question What are the costs of the exemption from Medicare price negotiation under the Inflation Reduction Act for drugs approved solely for the treatment of a single rare disease?

Findings This cross-sectional study identified 25 "sole orphan" drugs qualifying for exemption from Medicare price negotiation. Medicare spending on these drugs increased from $3.4 billion in 2012 to $10.0 billion in 2021; the sole orphan exemption would have prevented Medicare from negotiating prices on drugs with $1.1 to $3.0 billion in Medicare spending in each year.

Meaning The results of this study suggest that exempting sole orphan drugs from Medicare price negotiation will cost taxpayers billions of dollars per year; such savings could be used to control Medicare premium increases or provide other benefits for patients.

Methods

We used US Centers for Medicare and Medicaid Services (CMS) Medicare Part B and Part D Drug Dashboard data to identify drugs with annual Medicare spending exceeding $200 million (the threshold for negotiation under the IRA) in any year from 2012 to 2021, the years for which data are available.[9] Consistent with the IRA, we used gross Part D spending before manufacturer rebates and other discounts. All values were inflation adjusted to 2022 dollars using the consumer price index for all urban consumers (CPI-U).[10] As specified in CMS guidance, we combined spending for drugs made by the same manufacturer that shared an active moiety (small molecule drugs) or active ingredient (biologics).[11] Plasma-derived products, which we identified using the FDA Plasma Product website, were excluded because they are categorically exempt from negotiation.[12]

Drugs With Orphan Designations

We identified Orphan Drug Act designations and indications for drugs with high Medicare spending using the FDA's Orphan Drug Product Designation Database through March 20, 2023 (eFigure 1 in Supplement 1).[13] For drugs with at least 1 Orphan Drug Act designation, we used Drugs@FDA to identify nonorphan indications (ie, the treatment of a nonrare disease).[14] Per CMS guidance, we excluded Orphan Drug Act designations withdrawn by manufacturers.[11] For designations that had not yet resulted in approved indications as of March 2023, we assumed drugmakers would withdraw the designations if necessary to qualify for the sole orphan exemption.

Building on previous classification schemes,[15,16] we categorized drugs using 4 mutually exclusive and exhaustive categories based on each drug's labeled indications in each year (1) sole orphan: drugs approved solely for indications within a single Orphan Drug Act designation; (2) multiorphan: drugs approved for multiple Orphan Drug Act–designated indications and no non-Orphan Drug Act indications; (3) orphan first: drugs initially approved for an Orphan Drug Act–designated indication and subsequently approved to treat 1 or more non-Orphan Drug Act indications; and (4) non-orphan first: drugs initially approved for a non-Orphan Drug Act indication and subsequently approved for 1 or more Orphan Drug Act indications.

This analysis focused on 2 groups of drugs. The first group consisted of sole orphan drugs that, except for the sole orphan exemption, would have been eligible for Medicare price negotiation had the IRA been in effect from 2012 to 2021. We used this group to estimate Medicare spending excluded from negotiation and to assess what level of global revenues these drugs have historically been able to obtain. The second group consisted of multiorphan drugs, which would not qualify for the sole orphan exemption. This group is important because industry analysts have raised concerns that the sole orphan exemption may disincentivize manufacturers from exploring additional uses of orphan-designated drugs if doing so would subject the drugs to price negotiation.

Medicare Spending on Orphan-Designated Drugs

For each year in the study period, we determined which sole orphan drugs would have been eligible for negotiated prices in that year if the IRA price negotiation provision had been in effect "but for" the sole orphan exemption. We then tabulated annual Medicare spending on each drug as reported by CMS. We determined which drugs met all Medicare price negotiation eligibility criteria (other than the sole orphan exemption) by applying, in addition to the $200 million Medicare spending requirement, the IRA requirement that at least 7 years have passed since a drug's initial FDA approval (11 years for biologics), and that the drug faces no generic or biosimilar competition. We identified approval dates using Drugs@FDA.[14] We determined when generic or biosimilar versions were first marketed using manufacturer-reported data to the Medicaid Drug Rebate Program.[17,18] The IRA also contains a temporary provision exempting a narrow group of "small biotech" drugs, but none of the sole orphan drugs in our cohort would have qualified for this exemption.

Manufacturer Revenues From Sole Orphan Drugs

Drugs designated under the Orphan Drug Act are sometimes perceived as having limited commercial prospects, necessitating special incentives to encourage their development. To

assess the revenue potential of sole orphan drugs otherwise eligible for price negotiation, we obtained quarterly corporate securities filings for the manufacturers of each drug from its launch through the second quarter of 2023.

For drugs that had not been marketed long enough to face negotiation as of 2023, we additionally included revenue projections from 2023 through the year of earliest possible eligibility for negotiation. Forecasts were obtained from Visible Alpha, a third-party vendor that aggregates revenue forecasts from Wall Street equity research analysts.[19,20] Though revenue forecasts are inherently uncertain, they are widely used by the pharmaceutical industry and financial markets, providing valuable insight into the revenue expectations of the key decision-makers for investment in drug development.

We excluded drugs that were approved by the FDA before 2002 (due to lack of readily available historical revenue data), those that faced generic competition before they would have been eligible for price negotiation, and those manufactured by private firms that do not publicly report revenue data. We stratified revenue earned before vs after drugs could face negotiated prices (eg, at least 9 years after approval for small molecules, 13 years for biologics).

All analyses were conducted in Excel (version 16; Microsoft). Institutional review board approval was not sought because this study did not involve research with human participants. This study followed the Strengthening the Reporting of Observational Studies in Epidemiology (STROBE) reporting guideline.

Results

Between 2012 and 2021, 282 drugs had at least 1 year of Medicare spending exceeding $200 million, after excluding 8 plasma-derived products. Among these, 95 products (34%) had at least 1 Orphan Drug Act indication.

Drugs With Orphan Designations

Among the 95 Orphan Drug Act–designated drugs, 25 were sole orphan drugs (26%), 20 multiorphan drugs (21%), 13 orphan first drugs (14%), and 37 non-orphan first drugs (39%) as of March 2023 (**Table 1**, **Table 2**; eFigure 2 in Supplement 1). Among the 25 sole orphan drugs, 8 had at least 1 additional Orphan Drug Act designation that had not yet been associated with an FDA-approved indication (eTable 1 in Supplement 1). For example, teduglutide was approved in 2012 for treating parenteral-dependent short bowel syndrome; the drug was subsequently granted an Orphan Drug Act designation in 2020 for prevention of graft-vs-host disease, although this indication had not yet been FDA-approved as of March 2023.

Of the 20 multiorphan drugs, 11 were approved for only 2 Orphan Drug Act–designated conditions (Table 2; eTable 2 in Supplement 1). For example, pomalidomide was approved in 2003 for multiple myeloma. Subsequently, the drug manufacturer received an orphan designation for Kaposi sarcoma in 2018, and the FDA approved pomalidomide for this second indication in 2020. The remaining 9 multiorphan drugs were approved for more than 2 Orphan Drug Act–designated conditions. Among the 13 orphan first drugs, 10 drugs (77%) were approved for a nonorphan indication within 4 years of initial approval (eTable 3 in Supplement 1).

Medicare Spending

From 2012 to 2021, total Medicare Part B and Part D spending on the 95 high-spending Orphan Drug Act–designated drugs in this cohort was $517 billion, which represented 25% of Medicare's $2.1 trillion in prescription drug spending during this period. This spending on Orphan Drug Act–designated drugs included $77 billion (15%) on sole orphan drugs, $108 billion (21%) on multiorphan drugs, $75 billion (15%) on orphan first drugs, and $257 billion (50%) on non-orphan first drugs. Annual spending on these drugs increased from $25 billion in 2012 (19% of Medicare drug spending) to $72 billion in 2021 (26%; **Figure 1**).

The median (range) sole orphan drug had peak annual Medicare expenditures of $567 ($243-$1805) million, lower than median multiorphan ($746 million), orphan-first ($709 million), and non-orphan first ($714 million) drugs (eTable 4 in Supplement 1).

Had the IRA been in effect without the sole orphan exemption from 2012 to 2021, 12 of the sole orphan drugs would have been eligible for negotiated prices at some point during this time (Table 1). Medicare spending on these drugs in the years they would have been eligible for negotiated prices totaled $22.3 billion and ranged from $1.1 to $3.0 billion per year during the study period (median, $2.5 billion; IQR, $1.9-$2.6 billion). One of these sole orphan drugs (tetrabenazine) faced generic competition before being on the market long enough to qualify for price negotiation. Six of these drugs would have become eligible for negotiation and then subsequently ineligible owing to generic competition. The other 5 drugs did not face generic competition as of March 2023.

Of the remaining 13 drugs that were not yet eligible for negotiations as of 2021, 3 (dalfampridine, droxidopa, pirfenidone) faced generic competition as of 2023 and so therefore will not become eligible in the future. The remaining 10 drugs could first become eligible for Medicare price negotiation between 2024 and 2034 if the sole orphan exemption were removed.

Sole Orphan Drug Revenues

We obtained revenue data for 16 of the 25 sole orphan drugs. Four drugs were excluded because they were approved before 2002 (interferon beta-1a, glatiramer, lidocaine, and bosentan); 4 drugs were excluded because generic entry exempted them from price negotiation (tetrabenazine, dalfampridine, pirfenidone, and droxidopa); and 1 drug was excluded because its manufacturer did not publicly report its revenues (cenegermin). As of the second quarter of 2023, the median (IQR) sole orphan drug had earned global revenue of $11 ($6.6-$19.2) billion.

Of the 16 drugs, 8 (50%) would have been otherwise eligible for price negotiation as of 2023. For these drugs, median revenue from launch through the earliest onset of price negotiation ranged from $3.0 billion to $15.3 billion (median, $8.1 billion; **Figure 2**A). Total revenue for these drugs from launch through the second quarter of 2023 ranged from $4.0 billion to $24.9 billion (median, $13.7 billion).

Table 1. Drugs Approved to Treat a Single Orphan-Designated Condition

Brand name (manufacturer)	Generic name	Orphan designation	FDA approval	Years with Medicare spending >$200 million	Year of generic/ biosimilar entry	Earliest year for negotiated prices
Drugs otherwise eligible for Medicare price negotiation as of 2023						
Copaxone (Teva)	Glatiramer acetate	Multiple sclerosis (relapsing-remitting)[a]	1987	2012-NA[b]	2015	NA[c]
Avonex (Biogen)	Interferon beta-1a	Multiple sclerosis[a]	1991	2012-NA[b]	NA	NA[c]
Lidoderm (Teikoku Pharma)	Lidocaine	Postherpetic neuralgia	1999	2012-2014	2013	NA[c]
Tracleer (Johnson & Johnson)	Bosentan	Pulmonary arterial hypertension	2001	2012-2018	2019	NA[c]
Tyvaso (United Therapeutics)	Treprostinil	Pulmonary arterial hypertension[a]	2002	2013-NA[b]	2019	2016
Xenazine (Prestwick Pharmaceuticals)	Tetrabenazine	Huntington disease[a]	1997	2014-2015	2015	NA[d]
Tasigna (Novartis)	Nilotinib	Chronic myelogenous leukemia	2007	2014-NA[b]	NA	2017
Suboxone (Indivior)	Buprenorphine, naloxone	Opiate addiction	2002	2015-2019	2018	2018
Xyrem (Jazz Pharmaceuticals)	Sodium oxybate	Narcolepsy	2002	2015-NA[b]	N/A	2018
Letairis (Gilead Sciences)	Ambrisentan	Pulmonary arterial hypertension	2007	2014-2019	2019	2017
Gattex (Takeda)	Teduglutide	Short bowel syndrome[a]	2010	2017-NA[b]	NA	2022[c]
Kyprolis (Amgen)	Carfilzomib	Multiple myeloma[a]	2012	2015-NA[b]	NA	2022[c]
Opsumit (Johnson & Johnson)	Macitentan	Pulmonary arterial hypertension[a]	2013	2015-NA[b]	NA	2023[c]
Drugs potentially eligible for Medicare price negotiation in 2024 or later						
Ampyra (Acorda)	Dalfampridine	Multiple sclerosis	2010	2015-2018	2018	NA[d]
Esbriet (Genentech)	Pirfenidone	Idiopathic pulmonary fibrosis[a]	2014	2016-NA[b]	2022	NA[d]
Krystexxa (Horizon Therapeutics)	Pegloticase	Hyperuricemia with severe gout	2010	2021-NA[b]	NA	2024
Northera (Lundbeck)	Droxidopa	Neurogenic orthostatic hypotension	2014	2017-2020	2021	NA[d]
Ninlaro (Takeda)	Ixazomib citrate	Multiple myeloma	2015	2017-NA[b]	NA	2025
Tagrisso (AstraZeneca)	Osimertinib	EGFR$^+$ non-small cell lung cancer	2015	2017-NA[b]	NA	2025
Uptravi (Johnson & Johnson)	Selexipag	Pulmonary arterial hypertension	2015	2017-NA[b]	NA	2025
Darzalex (Johnson & Johnson)	Daratumumab	Multiple myeloma	2015	2017-NA[b]	NA	2029
Trikafta (Vertex)	Elexacaftor, tezacaftor, ivacaftor	Cystic fibrosis	2019	2020-NA[b]	NA	2029
Vyndaqel (Pfizer)	Tafamidis	Transthyretin amyloid cardiomyopathy	2019	2020-NA[b]	NA	2029
Oxervate (Dompe Farmaceutici)	Cenegermin	Neurotrophic keratitis	2018	2020-NA[b]	NA	2032
Tepezza (Horizon Therapeutics)	Teprotumumab	Thyroid eye disease	2020	2021-NA[b]	NA	2034

Abbreviations: EGFR, epidermal growth factor receptor; FDA, US Food and Drug Administration; NA, not applicable.

[a] Has second orphan designation with no approved indications.

[b] The spending for this drug continued to be more than $200 million in the most recent year for which data were available (2021).

[c] Exact date of earliest year for negotiated prices is unknown because Medicare spending data are not available prior to 2012.

[d] Faced generic competition prior to becoming eligible for negotiation.

Among the 8 drugs that were not yet marketed long enough to face Medicare negotiation as of 2023, median historical revenues from launch to the second quarter of 2023 ranged from $3.5 billion to $26.2 billion (median, $7.7 billion; Figure 2B). Including projected revenues for these drugs from quarter 3 of 2023 through their earliest possible year a Medicare negotiated price might be put in effect in the future, total revenue from launch ranged from $3.9 billion to $71.8 billion (median, $21.9 billion).

Discussion

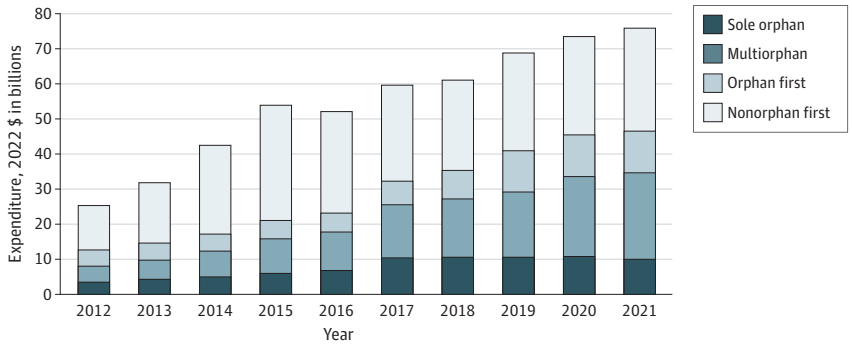
From 2012 to 2021, 25 drugs met the definition of a sole orphan drug under the IRA. Had the IRA been in effect during this period, the sole orphan exemption would have prevented Medicare from negotiating prices on otherwise-eligible drugs with a total $1.1 to $3.0 billion in Medicare spending each year. Medicare spending on sole orphan drugs has been

Table 2. Secondary Orphan Drug Act Designations Among High-Spend Multiorphan Drugs

Brand name (manufacturer)	Generic name	First Orphan Drug Act designation	Second Orphan Drug Act designation
Adempas (Bayer)	Riociguat	Chronic thromboembolic hypertension	Pulmonary arterial hypertension
Bendeka/Treanda (Teva)	Bendamustine	Chronic lymphocytic leukemia	FL, SLL, LL, SMZL, MALT, NMZL[a]
Calquence (AstraZeneca)	Acalabrutinib	Mantle cell lymphoma	Chronic lymphocytic leukemia
Exjade (Novartis)	Deferasirox	Dependent anemia iron overload	α-Thalassemia iron overload
Nplate (Amgen)	Romiplostim	Immune thrombocytopenic purpura	Radiation exposure
Ofev (Boehringer Ingelheim)	Nintedanib	Idiopathic pulmonary fibrosis	Systemic sclerosis
Pomalyst (Bristol-Myers Squibb)	Pomalidomide	Multiple myeloma	Kaposi sarcoma
Reblozyl (Bristol-Myers Squibb)	Luspatercept	Beta-thalassemia	Myelodysplastic syndrome
Sprycel (Bristol-Myers Squibb)	Dasatinib	Chronic myelogenous leukemia	Acute lymphoblastic leukemia
Velcade (Takeda)	Bortezomib	Multiple myeloma	Mantle cell lymphoma
Venclexta (AbbVie)	Venetoclax	Chronic lymphocytic leukemia	Acute myeloid leukemia

[a] Single designation covering follicular lymphoma (FL), small lymphocytic lymphoma (SLL), lymphoplasmacytic lymphoma (LL), splenic marginal zone lymphoma (SMZL), extranodal marginal zone B-cell lymphoma of mucosa-associated lymphoma tissue (MALT), and nodal marginal zone lymphoma (NMZL).

Figure 1. 2012 to 2021 Medicare Expenditures on High-Spend Orphan Drugs



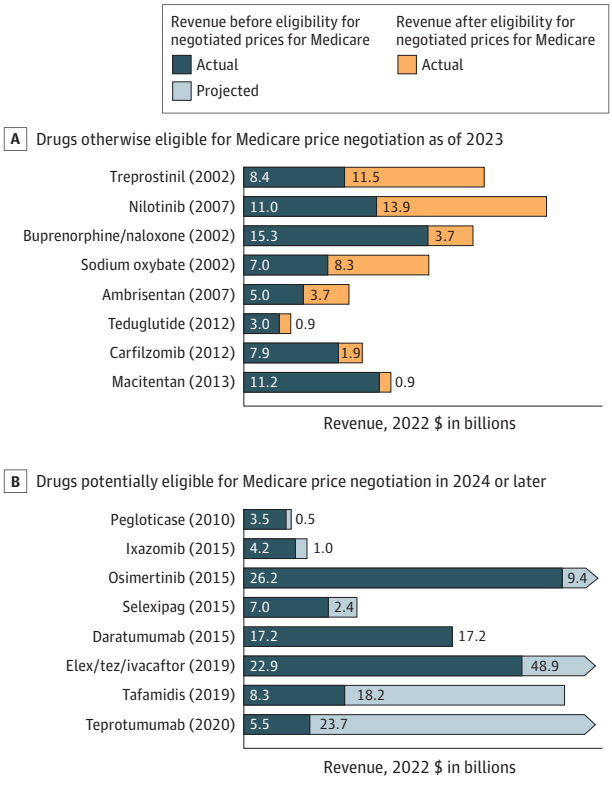
increasing over time, from $3.4 billion in 2012 to $10.0 billion in 2021.

Existing incentives in the Orphan Drug Act of 1983 seek to promote the development of drugs that may otherwise not be brought to market because of financial concerns.[21] Several studies,[7,22-25] however, have found that drugs approved for rare diseases earn similar revenues to drugs that treat more common conditions, raising questions about whether such incentives are necessary. By virtue of the $200 million per year in Medicare spending requirement, it is likely that any drug eligible for the sole orphan exemption is among the most financially successful of all drugs treating a single rare disease. These results suggest that exempting sole orphan drugs from Medicare negotiation will offer generous benefits for a small handful of products that have already achieved meaningful financial success. The median sole orphan drug in this cohort, for example, was projected to earn revenues of $21.9 billion in the years before even becoming eligible for negotiated prices on purchases by Medicare. This far exceeds the estimates of average new drug development costs from the literature.[26-28]

Some industry groups have also raised concerns that the sole orphan exemption could disincentive firms from repurposing sole orphan drugs for the treatment of other conditions.[29,30] Before the IRA, drug developers might pursue additional indications if incremental revenues were expected to exceed associated development costs. Following passage of the IRA, for drugs expected to achieve high levels of Medicare spending, it is possible that under some circumstances increased sales from additional indications will not offset revenues that are lost if the approval of those indications results in the drug being subject to Medicare negotiation.

Pomalidomide, for example, was initially approved to treat multiple myeloma, which is newly diagnosed in approximately 107 per million individuals in the US each year.[31] The second FDA approval of the drug was in a much rarer disease, Kaposi sarcoma (2 per million individuals in the US).[32] In such an instance, however, manufacturers could still conduct clinical trials for additional patient populations while foregoing FDA approval and thereby retaining the sole orphan exemption. Other parties could also seek to fill the gap. Indeed, in the case

Figure 2. Sole Orphan Drug Revenues



of pomalidomide, the Kaposi sarcoma trial was sponsored and conducted by the National Cancer Institute.[33] Though this approach does not guarantee insurance reimbursement in all cases, it would be an improvement for both patients and manufacturers vs the alternative of not generating additional evidence.[34]

As Medicare price negotiation gets underway, several pharmaceutical companies and industry organizations have filed lawsuits challenging the constitutionality of the IRA.[35] In Congress, the industry is lobbying to narrow or repeal the law. At the same time, the Biden administration has proposed loosening the selection criteria to increase the number of drugs for which the cost to Medicare can be negotiated.[36] Whether any of these efforts will result in changes to the implementation of the IRA is unclear.

Should Congress decide to revisit the law, these results suggest that eliminating the sole orphan exemption would generate substantial taxpayer and patient savings. Subjecting the most financially successful sole orphan drugs to the same negotiation as other drugs with high levels of Medicare spending is unlikely to render these drugs unprofitable. Moreover, eliminating the exemption would reduce any potential disincentive for repurposing these rare disease drugs for other, even rarer diseases.

Alternatively, Congress could consider limiting the exemption to circumstances in which an otherwise eligible drug is not likely to become profitable, as required by the economic profitability test in the original Orphan Drug Act.[37] Congress could also follow the suggestion of the sponsor of the Orphan Drug Act, Congressman Henry Waxman, who suggested Orphan Drug Act benefits could end once a drug achieved a specified revenue level.[38]

Some have advocated expanding the sole orphan exemption by exempting all drugs with any orphan designation. This would be a costly and inefficient approach, undermining the core purpose of the law.[29] We found that such an expansion would exempt a quarter of all Medicare prescription drug spending from negotiation because many blockbuster drugs are used to treat both rare and common diseases. Such an expansion would also create an opportunity for companies to avoid negotiation on blockbuster products by pursuing low-cost, low-value Orphan Drug Act designations or subdividing existing patient populations, a behavior firms have historically engaged in to retain Orphan Drug Act incentives after a disease no longer qualifies due to increased prevalence.[39]

Limitations

This retrospective analysis may not be representative of drugs that qualify for Medicare price negotiation in the future. A growing number of drugs target rare diseases, and it is possible that the spending on sole orphan drugs might change in future years. Some of the sole orphan drugs that would be exempt from price negotiation as of March 2023 might become eligible for negotiation in the future if approved for additional indications. Finally, we did not address how the sole orphan exemption might interact with other policy changes in the IRA outside of price negotiation, such as inflation rebates or the Part D benefit redesign.

Conclusions

This cross-sectional study found that the sole orphan exemption will exclude billions of dollars of Medicare drug spending from price negotiation. The high level of global revenues achieved by these drugs, however, suggests that special exemption is unnecessary for them to achieve financial success. Congress could consider removing the sole orphan exemption to obtain additional savings for patients and taxpayers and to eliminate any potential disincentive for developing additional indications for these drugs.

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