PORTAL
Program On Regulation, Therapeutics, And Law

Division of Pharmacoepidemiology and Pharmacoeconomics
Brigham and Women's Hospital • Harvard Medical School
41 Avenue Louis Pasteur, Suite 0B17, Boston, MA 02115
portalresearch.org • portal@bwh.harvard.edu

August 17, 2026

The Honorable Ron Wyden
Ranking Member
US Senate Committee on Finance
219 Dirksen Senate Office Building
Washington, DC 20510-6200
Attn: Committee Minority Staff

RE: Request for Information: Commonsense Policy Options to Lower Drug Prices for Patients

Dear Ranking Member Wyden,

As members of the Program On Regulation, Therapeutics, And Law (PORTAL), we appreciate the opportunity to provide input on Senate Democrats' proposals to address the high cost of prescription drugs in the United States.

Based in the Division of Pharmacoepidemiology and Pharmacoeconomics at Brigham and Women's Hospital and Harvard Medical School, PORTAL is one of the largest academic research centers in the United States dedicated to investigating the regulation, use, evidence, and cost of prescription drugs. Our research program is independent of the pharmaceutical industry.

We commend Senate Democrats for their continued commitment to prescription drug policy reform. **In the comments that follow, we outline the areas where our research group has generated relevant evidence and offer recommendations informed by that work.** We do not address every proposal in the RFI, and the absence of comment should not be interpreted as opposition.

We appreciate the opportunity to share these comments and welcome further discussion on ways to improve prescription drug affordability and access. Please do not hesitate to contact us with any questions at PORTAL@bwh.harvard.edu.

Sincerely,

Matthew J. Martin, MA
Kelly E. Anderson, PhD, MPP
Aaron S. Kesselheim, MD, JD, MPH

SECTION I: Lowering Drug Prices

I. Bolstering Medicare's Ability to Negotiate Lower Drug Prices

We appreciate Senate Democrats' commitment to strengthening and expanding Medicare's ability to lower drug prices through the Medicare Drug Price Negotiation Program. The program has already been remarkably successful, and that success has grown over time. The most recently negotiated prices for the initial price applicability year (IPAY) 2027 drugs were on average 44% lower than what Medicare currently pays for these 15 drugs.[1] This represents even greater savings than for the 10 IPAY 2026 drugs, with an average savings of 22%.[2] Savings from negotiation will likely continue to increase in the coming years, as the program now includes drugs reimbursed under Medicare Part B. Simultaneously, the program has been consistently upheld by Federal courts amid legal challenges by pharmaceutical companies. We believe that further changes to the negotiation program have the potential to improve on this success.

Factoring International Pricing into Negotiation

Congress should authorize CMS to consider international drug prices as a factor in negotiation and require participating manufacturers to submit international pricing data. The US pays substantially more for prescription drugs than other countries,[3] and applying comparator prices to US sales could yield substantial savings. For example, international prices for top-selling cancer drugs run 65% to 81% below US prices, and applying most-favored-nation pricing to already-negotiated cancer drugs would have reduced 2024 Medicare spending by an additional $3.2 to $4.7 billion.[4] Although the current negotiation framework has been effective, negotiated maximum fair prices for selected drugs remain above prices in other high-income countries.[5] International prices are therefore a valuable factor for CMS to consider.

If international prices are incorporated into the ceiling price, the second approach described in the RFI is preferable, under which the ceiling is lowered by a defined percentage when the US price substantially exceeds prices in the reference basket. This would provide predictability and achieve the aim of making international prices a core part of CMS's process without directly limiting the negotiations based on another country's value judgements.[6] Treating international prices as one factor among several would also align with practice in peer countries. In a review of 19 comparator countries' health technology assessment processes, 15 were found to use some form of international reference pricing, and most use it to supplement domestic value assessment and negotiation rather than as the determinative factor.[7]

---

[1] Rome BN, Kesselheim AS. The Growing Success of Medicare Drug-Price Negotiation. *N Engl J Med*. 2026;394(9):835-838.
[2] Rome BN, Kesselheim AS, Feldman WB. Medicare's First Round of Drug-Price Negotiation — Measuring Success. *N Engl J Med*. 2024;391(20):1865-1868.
[3] Mulcahy AW, Schwam D, Lovejoy SL. International Prescription Drug Price Comparisons: Estimates Using 2022 Data. *Rand Health Q*. 2024;11(3):5.
[4] Hwang TJ, Tibau A, Kesselheim AS, Vokinger KN. Implications of Medicare Negotiation and Most-Favored-Nation Pricing for Cancer Medicine Costs. *JAMA Health Forum*. 2026;7(5):e260509.
[5] Wouters OJ, Sullivan SD, Cousin EM, Gabriel N, Papanicolas I, Hernandez I. Drug Prices Negotiated by Medicare vs US Net Prices and Prices in Other Countries. *JAMA*. 2025;333(1):85-87.
[6] Rand LZ, Kesselheim AS. An International Review of Health Technology Assessment Approaches to Prescription Drugs and Their Ethical Principles. *J Law Med Ethics*. 2020;48(3):583-594.
[7] Rand LZ, Kesselheim AS. International Reference Pricing for Prescription Drugs: A Landscape Analysis. *J Manag Care Spec Pharm*. 2021;27(9):1309-1313.

Regarding the selection of comparator countries, the basket should reflect both GDP *and* pharmaceutical market size. The countries listed in H.R. 3 would be appropriate, as would the broader G-7-based basket proposed for CMS's GENEROUS Model in Medicaid.[8] Focusing on larger markets also limits manufacturers' ability to game the basket by delaying launches in or withdrawing from smaller or lower-income countries.[9] Rather than fixing the list in statute, though, Congress should set a minimum pharmaceutical market size and direct the Secretary to select countries through rulemaking, allowing the basket to adapt as countries change in their suitability as comparators.

Legislation should also address the limitations of available international pricing data. Because many countries negotiate confidential rebates below publicly disclosed prices, list prices can overstate what those countries pay. For example, among anticancer drugs launched in Germany between 2002 and 2017, negotiated prices averaged 24.5% below launch prices.[10] These net pricing data are essential for international reference pricing to be effective, yet manufacturers may argue that country-level disclosure of net prices would breach confidentiality agreements**. For this reason, manufacturers should be required to submit an average or median net price across the basket rather than country-specific prices.** This would inform negotiation without raising confidentiality concerns or placing disproportionate weight on any one country's price. **CMS should further be directed to aggregate pricing data across dosage forms, strengths, and routes of administration**, preventing manufacturers from evading reference pricing through strategic launches of new formulations and dosage forms in reference countries.[11]

More broadly, we note that the appeal of international prices reflects, in part, the absence of domestic infrastructure for assessing the comparative clinical value and cost-effectiveness of prescription drugs. Prices in peer countries are the output of formal health technology assessment processes that compare new drugs against existing alternatives and align price with added benefit. The US has no comparable capacity. **Establishing an independent federal entity to assess the value of newly approved drugs at the time of approval, including both clinical and economic considerations alongside robust opportunity stakeholder input, would provide meaningful information about a drug's clinical benefit and whether its price is aligned with that benefit.** This information could be leveraged in Medicare negotiations and by other payers to achieve greater savings while ensuring that our health care dollars are directed towards the therapies that most benefit patients.[12]

Negotiating Lower Prices on More Drugs

We support eliminating the annual drug selection maximum, enabling CMS to negotiate above the existing limit of 20 drugs per year. This would increase the savings potential of the program, provided CMS has the resources to ensure that negotiation of every selected drug remains robust. Any increase should be paired with additional appropriations for the negotiation program.

---

[8] CMS. GENEROUS (GENErating cost Reductions fOr U.S. Medicaid) Model. Updated July 1, 2026.
[9] Maini L, Pammolli F. Reference Pricing as a Deterrent to Entry: Evidence from the European Pharmaceutical Market. *Am Econ J: Microecon*. 2023;15(2):345-383.
[10] Lauenroth VD, Kesselheim AS, Sarpatwari A, Stern AD. Lessons from the Impact of Price Regulation on the Pricing of Anticancer Drugs in Germany. *Health Aff (Millwood)*. 2020;39(7):1185-1193.
[11] Galizzi MM, Ghislandi S, Miraldo M. Effects of Reference Pricing in Pharmaceutical Markets: A Review. *Pharmacoeconomics*. 2011;29(1):17-33.
[12] Ollendorf DA, Rand LZ, Blonda A, Mooney H, Phillips M, Basu A. Health Technology Assessment, Again: A Transparent, Evidence-Based Approach for CMS Drug Price Negotiations. *Health Affairs*. 2025;44(8):898-905.

But given the manifold current constraints on drug qualifying for the program, eliminating the cap of 20 drugs per year will very soon cease to be relevant in the drug selection process. **Far more impactful than eliminating the cap would be:**

(1) **Initiating the process for negotiation shortly after drug launch.** Shifting negotiations to within a few years after launch should be a realistic goal.

(2) **Allowing more drugs to qualify for negotiation.** This could include, for example, shifting drug selection from qualifying based on gross Medicare spending of $200 million per year in Medicare to net spending that accounts for rebates. Rebates may already substantially lower spending in competitive therapeutic areas relative to those where rebates are less common, such as oncology and the protected classes. Selecting drugs on net rather than gross spending would have increased savings from the first negotiation cycle by an estimated 45%.[13] It could also include disposing of unnecessary exclusions, such as those for drugs with Orphan Drug Act designations and plasma-derived products.

We discuss both ideas in more detail below.

Delivering Lower Negotiated Drug Prices to Patients Faster

We support accelerating the timeline for Medicare drug price negotiation. Ideally, Congress would authorize CMS to negotiate prices at or near the time of launch. Short of that, any reduction in the current waiting periods would deliver additional savings to patients and the Medicare program. Earlier negotiation would also maximize the use of CMS resources, as the resulting maximum fair prices will be in effect for longer prior to generic or biosimilar competition.

Each year of delayed negotiation is a year in which Medicare pays a price set unilaterally by the manufacturer using a nonpublic process that can be based on factors unrelated to the clinical benefits of the drug. Rebates negotiated by Part D plans reduce net spending during this period, but vary considerably across plans and therapeutic areas, and more often reflect a plan's negotiating position than a drug's clinical value.[14]

Negotiation at or near launch is the standard approach in peer countries.[15] Germany, for example, allows manufacturers to set launch prices freely for one year, during which a formal benefit assessment is conducted, after which price is negotiated with representatives of the statutory health insurers.[16] Adopting a similar approach would secure a negotiated price at the outset and would distribute costs more equitably, since patients who need expensive brand-name drugs shortly after they are launched currently bear disproportionately high prices relative to patients who begin treatment years later.

At a minimum, small-molecule drugs and biologics should be subject to the same waiting period. Industry has argued that the current 4-year differential penalizes small-molecule development and

[13] Reitsma MB, Dusetzina SB, Ballreich JM, Trujillo AJ, Mello MM. Examining Opportunities to Increase Savings From Medicare Price Negotiations. *JAMA Intern Med*. 2023;183(6):581-588.
[14] GAO. Medicare Part D: CMS Should Monitor Effects of Rebates on Plan Formularies and Beneficiary Spending. GAO-23-105270. Published September 5, 2023.
[15] Syversen ID, Schulman K, Kesselheim AS, Feldman WB. A Comparative Analysis of International Drug Price Negotiation Frameworks: An Interview Study of Key Stakeholders. *Milbank Quarterly*. 2024;102(4):1004-1031.
[16] Lauenroth VD, Kesselheim AS, Sarpatwari A, Stern AD. Lessons from the Impact of Price Regulation on the Pricing of Anticancer Drugs in Germany. *Health Aff (Millwood)*. 2020;39(7):1185-1193.

should be resolved by extending the small-molecule period to 13 years. We agree that the difference is unjustified, but the evidence rather supports setting the waiting period at 9 years. Equalizing at 13 years would effectively end negotiation for many small-molecule drugs, since generic competition typically begins 12 to 17 years after FDA approval. Prior analysis from our team found that extending the small-molecule waiting period would affect eligibility for 23 drugs with combined gross Medicare spending of $24.1 billion in the first year each would otherwise be eligible.[17] It would also extend additional protection to biologics when there is little evidence that these therapies warrant different treatment: development times and costs are comparable across the two categories, biologics have higher clinical trial success rates and substantially more patent protection, and biologics generate greater global revenue by year 9 than small-molecule drugs generate by year 13.[18,19]

Boosting the Biosimilar Market

We appreciate Senate Democrats' attention to biosimilar competition and its intersection with Medicare price negotiation. Since its early years, the biosimilar market has been characterized by slow uptake,[20] minor spending reductions,[21] and inconsistent patient savings,[22] though there have been signs of progress.[23] These trends support Senate Democrats' interest in improving biosimilar availability and competition in the biosimilar market.

Biosimilars have thusfar entered the US market upwards of 20 years—or more—after approval of the reference product, compared with average 13 years of post-approval market exclusivity for small-molecule drugs.[24] Thus, even substantial acceleration of the Medicare negotiation timeline would still leave a considerable market period prior to biosimilar launch for many products. **There are numerous barriers to timely biosimilar entry, including the complex web of patents surrounding biologics, current restrictions on biosimilar substitution, and formulary practices that reward high list prices.**

Biologics are associated with a median of 14 patents per product compared with 3 for small-molecule drugs, and the strength of many of these patents, particularly those covering secondary features such as methods of treatment, is questionable.[25] Administrative review at the US Patent and Trademark Office's Patent Trial and Appeal Board (PTAB) has been an effective avenue for challenging these patents: between 2012 and 2021, biosimilar manufacturers used these procedures to invalidate 14 patents on reference biologics, and originator revenues declined afterward, indicating improvements in market competition.[26] Other studies have demonstrated the value of administrative review pathways to

[17] Cai C, Kesselheim AS, Rome BN. Costs of Extending the Small Molecule Exemption Period in Medicare Drug Price Negotiation. *Health Affairs Forefront*. Published online May 8, 2025.
[18] Vogel M, Feldman WB, Cowan Z, et al. Revenue Differences Between Top-Selling Small-Molecule Drugs and Biologics in Medicare. *JAMA Health Forum*. 2025;6(10):e254720.
[19] Wouters OJ, Vogel M, Feldman WB, Beall RF, Kesselheim AS, Tu SS. Differential Legal Protections for Biologics vs Small-Molecule Drugs in the US. *JAMA*. 2024;332(24):2101.
[20] Rome BN, Bhaskar A, Kesselheim AS. Use, Spending, and Prices of Adalimumab Following Biosimilar Competition. *JAMA Health Forum*. 2024;5(12):e243964.
[21] Hong D, Kesselheim AS, Sarpatwari A, Rome BN. Biosimilar Uptake In The US: Patient And Prescriber Factors. *Health Affairs*. 2024;43(8):1159-1164.
[22] Feng K, Russo M, Maini L, Kesselheim AS, Rome BN. Patient Out-of-Pocket Costs for Biologic Drugs After Biosimilar Competition. *JAMA Health Forum*. 2024;5(3):e235429.
[23] Riegler JS, Kesselheim AS, Rome BN. Out-of-Pocket Spending for Biologic Drugs After Biosimilar Competition for Medicare Patients. *JAMA Netw Open*. 2026;9(1):e2554235.
[24] Wouters OJ, Vogel M, Feldman WB, Beall RF, Kesselheim AS, Tu SS. Differential Legal Protections for Biologics vs Small-Molecule Drugs in the US. *JAMA*. 2024;332(24):2101.
[25] *Ibid.*
[26] Raymakers AJN, Van De Wiele VL, Kesselheim AS, Tu SS. Changes in Biologic Drug Revenues After Administrative Patent Challenges. *Health Affairs*. 2025;44(3):274-279.

promoting biosimilar competition.[27] However, recent increases in discretionary denials at the PTAB have narrowed biosimilar makers' access to these administrative pathways.[28] **Congress should work to ensure that biosimilar developers can continue to use the PTAB to review and, if necessary, invalidate erroneously granted patents that delay competition and patient access to lower-cost therapies.**

Current patent litigation timelines further compound this problem. Under the Biologics Price Competition and Innovation Act, biosimilar manufacturers cannot initiate patent litigation until after submitting a biologics license application to FDA, which follows completion of extensive and costly clinical testing, while generic manufacturers can begin far earlier and launch soon after key patents expire. Permitting biosimilar developers to initiate litigation at the start of phase 3 testing, once the product and manufacturing process are largely finalized, could reduce entry delays by nearly two years.[29]

FDA interchangeability requirements and state substitution laws create a second barrier. Unless a biosimilar maker seeks an interchangeable designation, pharmacies cannot substitute the product as they would a generic, despite substantial evidence of clinical equivalence: across 31 cancer biosimilar studies, biosimilar trials on average enrolled more patients than the reference products' own pivotal trials and were more likely to be randomized and double-blind, and every biosimilar examined was indistinguishable from its reference product on surrogate efficacy.[30] Among five national medicines regulators, FDA also remains the only one to maintain a separate interchangeability designation, which sets a higher approval standard that can be a barrier to substitution.[31] **We support FDA's recent proposals to revise interchangeability testing requirements as well as introduced legislation that would eliminate the designation, thus ensuring that biosimilars are not treated more restrictively than generics.**

Formulary and rebate practices present a third barrier. Pharmacy benefit managers have treated biologics and biosimilars as competing brand-name products rather than interchangeable therapies, enabling originators to protect formulary position by raising list prices and offering larger rebates that lower-priced biosimilars cannot match. For example, in the first year after adalimumab (Humira) biosimilars became available, net spending fell nearly 50% while biosimilars represented less than 2% of prescriptions, indicating that savings came from originator rebates rather than competition.[32] By January 2024, nearly all Part D plans covered Humira, only half covered any biosimilar, and fewer than 2% preferred one.[33] Several biosimilar makers have even launched products at both a high and a low list price, anticipating that some plans would prefer the higher-priced version for its larger rebate. Because cost-sharing such as coinsurance is typically calculated on list price, this preference raises patient out-of-pocket costs.

---

[27] Van De Wiele VL, Kesselheim AS, Tu SS. Biologic patent challenges under the America Invents Act. *Nat Biotechnol*. 2024;42(3):374-377.
[28] Tu SS, Rai A, Kesselheim AS. Recent Changes in Discretionary Denials of Drug Patent Challenges. *Health Aff Sch*. 2025;3(11):qxaf215.
[29] Tu SS, Goode R, Turner M, Van De Wiele V. Accelerating biosimilar market access: the case for allowing earlier standing. *J Law Biosci*. 2025;12(1):lsae030.
[30] Bloomfield D, D'Andrea E, Nagar S, Kesselheim A. Characteristics of Clinical Trials Evaluating Biosimilars in the Treatment of Cancer: A Systematic Review and Meta-analysis. *JAMA Oncol*. 2022;8(4):537-545.
[31] Knox RP, Desai V, Sarpatwari A. Biosimilar Approval Pathways: Comparing the Roles of Five Medicines Regulators. *J Law Biosci*. 2024;11(2):lsae020.
[32] Rome BN, Bhaskar A, Kesselheim AS. Use, Spending, and Prices of Adalimumab Following Biosimilar Competitionv. *JAMA Health Forum*. 2024;5(12):e243964.
[33] Klebanoff MJ, Li P, Lin JK, Doshi JA. Formulary Coverage of Brand-Name Adalimumab and Biosimilars Across Medicare Part D Plans. *JAMA*. 2024;332(1):74.

Various policies could address these PBM distortions. Congress could prohibit confidential rebates on originator biologics once a biosimilar becomes available.[34] Star Rating or plan transparency measures rewarding selection of low list price biosimilars would further align plan and patient interests. CMS could also apply maximum allowable cost schedules across a reference biologic and its biosimilars in Part D, as it does for generics, though this would only be effective alongside the substitution reforms described above. For clinician-administered drugs covered under Part B, CMS could establish bundled payment rates for clinician-administered biologics and their biosimilars in place of separate rates for each.

Finally, **we encourage greater scrutiny of comarketing arrangements through which pharmacy benefit managers sell private label biosimilars**, a practice with no analogue in the generic market and one that raises vertical integration concerns. Because three pharmacy benefit managers account for approximately 80% of US prescriptions, a biosimilar developer without such an arrangement may have limited assurance that its product will generate sales sufficient to recoup development costs, which is itself a disincentive to market entry. Members of Congress have already asked federal regulators to examine these agreements for anticompetitive conduct, and we encourage further scrutiny.

Eliminating Blockbuster Bailouts

The Inflation Reduction Act included an exclusion for drugs approved for a single rare disease. **But the evidence does not support excluding high-revenue rare disease therapies from negotiation, even using a higher threshold.** Among the 100 highest-revenue prescription drugs in 2022, 19 were approved exclusively for rare disease indications, accounting for $45.1 billion in spending, of which Medicare bore $23.6 billion.[35] Drugs initially approved for rare conditions earn five-year net sales that are fiscally comparable to those of non-rare drugs,[36] and drugs approved for a single rare disease that would otherwise have been eligible for negotiation would have earned a median of $11 billion in cumulative global revenue before first becoming eligible.[37] These products also already benefit from extended FDA market exclusivity, research tax credits, and user fee waivers.[38] These findings suggest that existing Medicare spending thresholds intended to ensure that Medicare negotiates only high-revenue drugs is sufficient to provide reasonable incentives to pursue rare disease therapies.

This rare disease drug exclusion was expanded even further with the One Big Beautiful Bill Act of 2025, which amended the exemption to cover drugs treating multiple rare diseases and also included a provision delaying negotiation for rare disease drugs later approved for non-rare conditions. We estimated that these expanded carve-outs will prevent or delay negotiation in the short-term for nearly two dozen drugs accounting for $18 billion in Medicare spending.[39]

---

[34] Rome BN, Bhaskar A, Kesselheim A. Biosimilar Interchangeability and Substitution in the US: What Comes Next? *Am J Manag Care*. 2026;32(4):198-201.
[35] Mooney H, Kesselheim AS, Rome BN. US Spending on High-Revenue Rare Disease Drugs in 2022. *Am J Manag Care*. 2025;31(11):e329. doi:10.37765/ajmc.2025.89823
[36] Tu SS, Nagar S, Kesselheim AS, Lu Z, Rome BN. Five-Year Sales for Newly Marketed Prescription Drugs With and Without Initial Orphan Drug Act Designation. *JAMA*. 2023;329(18):1607. doi:10.1001/jama.2023.3079
[37] Vogel M, Zhao O, Feldman WB, Chandra A, Kesselheim AS, Rome BN. Cost of Exempting Sole Orphan Drugs From Medicare Negotiation. *JAMA Intern Med*. 2024;184(1):63. doi:10.1001/jamainternmed.2023.6293
[38] Sarpatwari A, Kesselheim AS. Reforming the Orphan Drug Act for the 21st Century. *N Engl J Med*. 2019;381(2):106-108.
[39] Rome BN, Mooney H, Kesselheim AS. Prescription Drugs Affected by an Expanded Rare Disease Carve-Out from Medicare Price Negotiation. *J Gen Intern Med*. 2026.

The exclusion's cumulative effect on future cycles is likely to be larger. The number of rare disease approvals has grown steadily over time,[40] meaning that the pool of drugs available for selection will narrow without changes to the existing exclusion, potentially limiting CMS's ability to select even the required number of drugs each year. This would have a direct impact on the proposal to convert the annual selection maximum into a minimum.

We support replacing the rare disease drug exclusion with a spending threshold along the lines of the No Big Blockbuster Bailouts Act. A single threshold is a simpler mechanism than the current exclusion's structure, and it would avoid penalizing manufacturers for seeking non-rare indications, since eligibility would be based solely on Medicare spending rather than on which indications a manufacturer chooses to pursue.

II. Enhancing Manufacturer Accountability for Price Gouging

Extending Part B Penalties to Medicare Advantage

The Inflation Reduction Act included language authorizing CMS to collect rebates from drug manufacturers when changes in the average sales price of single source Part B products exceed inflation. CMS implemented this provision specifically for the traditional Medicare program and excluded units of the products administered to Medicare Advantage enrollees. **The current text of the law authorizes CMS to include Medicare Advantage units, but legislation could further strengthen this provision by requiring CMS to collect rebates for the Medicare Advantage program.**[41] With over half of Medicare beneficiaries selecting Medicare Advantage coverage, CMS may be missing major savings associated with the Part B penalties.

Extending Rebate Penalties to the Commercial Market

There are several additional opportunities for CMS to expand the scope of the inflation rebates. First, within the Medicare program, legislation could remove the exemption for units administered at 340B facilities. Second, legislation could remove the lower penalty rate for prescription drugs experiencing a shortage. Legislation could also expand the scope of the inflation rebates, for example to the commercial market. A key challenge with this approach is that it would require additional data collection on units of each eligible drug administered to commercial enrollees.

Congress should also extend negotiated Medicare prices to the commercial market, as it originally intended to do in the legislation that eventually became the IRA.

III. Potential New Payment Models for Prescription Drugs

Subscription Models

Subscription models have been investigated as a promising solution to improve patient access to highly effective therapies of public health importance, such as direct-acting antivirals for the treatment of hepatitis C (HCV). Subscription programs that rely on government-based drug procurement have

[40] Miller KL, Lanthier M. Orphan Drug Label Expansions: Analysis of Subsequent Rare and Common Indication Approvals. *Health Aff (Millwood)*. 2024;43(1):18-26.
[41] Linke Young C. The inflation rebate for Medicare Part B-covered drugs should apply to Medicare Advantage. *Brookings Institution*. Published May 14, 2025.

generally succeeded in improving patient access in states like Louisiana and abroad,[42,43,44] but such models have not been piloted nationwide in the US. Accordingly, **any federal subscription program would need to be properly calibrated to balance patient access with the need to constrain prices through negotiation.**

Part of what makes antiviral therapies for HCV more promising candidates for subscription models over therapies like GLP-1s used for diabetes and weight loss in obese patients is that the former are curative, whereas the latter require chronic use to maintain their therapeutic effect. This means that the covered population for a GLP-1 subscription program would likely grow over time rather than decline, and manufacturers would likely price this growth into their program bids, translating into a higher upfront cost. The eligible population for GLP-1s is also substantially larger than that for HCV or most other conditions for which a subscription might be considered.[45]

Any subscription model should be structured to ensure the federal government negotiates the most favorable price. In assessing manufacturer bids to the program, HHS should be required to consider comparative and cost-effectiveness data, federal contributions to the development of the products, prior US spending, prices offered to other domestic and international purchasers, and the availability of generics or biosimilars of the drug and its therapeutic alternatives, among other factors. Prior US spending is a particularly important consideration for products like GLP-1s, on which manufacturers have already generated substantial revenue, meaning concerns about recouping development costs should be minimal.

At a minimum, prices negotiated through a subscription model should not exceed the net price currently paid per patient, since manufacturers receive guaranteed payment and expanded coverage in exchange for agreeing to a lower price**.** In Medicare, the subscription price should not exceed a drug's negotiated maximum fair price if one has been established. Congress would need to specify how a subscription interacts with negotiation eligibility, including whether a drug supplied under a subscription remains a selected drug and how the maximum fair price would be effectuated for products already paid for through a lump sum. Contracts should also be negotiated on a shorter time horizon of 2-3 years in rapidly evolving markets so that terms and prices can adapt accordingly.

On the structure of the subscription model itself, **we would recommend a single national subscription over multiple payer-specific models,** which would maximize the volume commitment offered to manufacturers and thus the government's leverage in negotiation**.** This could be structured such that public sector plans (e.g., Medicare, Medicaid) are required to participate, whereas commercial plans and employers could buy into the model through plan assessments. We support member-weighted assessments as a funding mechanism, with the important qualification that assessments be weighted by enrollment rather than by use of the qualifying drug. A utilization-based assessment would allow a plan to reduce its financial contribution by restricting access, undermining the model's intent.

---

[42] Auty SG, Shafer PR, Griffith KN. Medicaid Subscription-Based Payment Models and Implications for Access to Hepatitis C Medications. *JAMA Health Forum*. 2021;2(8):e212291.
[43] Moon S, Erickson E. Universal Medicine Access through Lump-Sum Remuneration — Australia's Approach to Hepatitis C. *N Engl J Med*. 2019;380(7):607-610.
[44] NHS England. Antimicrobial Products Subscription Model: guidance on commercial arrangements. Published May 8, 2024.
[45] Wong ND, Karthikeyan H, Fan W. US Population Eligibility and Estimated Impact of Tirzepatide Treatment on Obesity Prevalence and Cardiovascular Disease Events. *Cardiovasc Drugs Ther*. 2025;39(4):837-847

All plans participating in the model should be required to commit to defined patient access standards, such as the elimination of prior authorization for drugs purchased through the subscription. Subscriptions should similarly cover all FDA-approved indications for a selected therapy, as limiting them to specific indications would be both administratively challenging and counter to the aim of the subscription to improve patient access.

Addressing Launch Prices

Launch prices remain high for many new therapies, with median annual launch prices rising from approximately $2,100 in 2008 to $180,000 in 2021, and $300,000 in 2023.[46] More worryingly, these prices are set with little transparency into the relationship between the drug's price and its clinical benefit. For example, among the 50 top-selling brand-name drugs in Medicare in 2020, 55% were assessed as having low added therapeutic benefit by HTA bodies in Canada, France, and Germany, yet accounted for over $19 billion in annual net Medicare spending.[47] In oncology, specifically, analyses in both the US and Europe have found no consistent association between price and clinical benefit.[48,49]

One effective way to address excessive launch prices for new drugs would be to establish a domestic health technology assessment agency that would assess whether a new drug's launch price is justified by its added clinical benefit. Manufacturers should be required to submit newly approved products for review by this independent federal entity as a condition of participation in Medicare, Medicaid, and other federal health programs. The resulting analysis could then be made public so that payers could leverage the resulting analysis in subsequent manufacturer negotiations. This procedure would also necessitate manufacturers to publicly justify the price of their products and would incentivize evidence generation to demonstrate a drug's clinical benefit over alternatives. Congress could also require manufacturers to disclose launch prices and the basis on which they were set, since only about one-third of new drugs approved between 2022 and 2024 had prices proactively disclosed by their manufacturers, and disclosure was least common among the largest firms.[50]

SECTION II: Enhancing Prescription Drug Affordability

I. Lowering Out-of-Pocket Costs

Out-of-Pocket Caps

Drug-specific out-of-pocket caps have been implemented by more than 20 states (most commonly for insulin), as well as by the Medicare program. In the commercial market, insulin out-of-pocket caps have not appeared in population-level studies to meaningfully increase insulin use, in part because many enrollees were already paying amounts below cap thresholds.[51,52] Estimates of the impact of manufacturer-initiated inhaler out-of-pocket caps found that only 37.5% of individuals prescribed

[46] Rome BN, Egilman AC, Kesselheim AS. Trends in Prescription Drug Launch Prices, 2008-2021. *JAMA*. 2022;327(21):2145.
[47] Egilman AC, Rome BN, Kesselheim AS. Added Therapeutic Benefit of Top-Selling Brand-name Drugs in Medicare. *JAMA*. 2023;329(15):1283.
[48] Vokinger KN, Hwang TJ, Daniore P, et al. Analysis of Launch and Postapproval Cancer Drug Pricing, Clinical Benefit, and Policy Implications in the US and Europe. *JAMA Oncol*. 2021;7(9):e212026.
[49] Vokinger KN, Hwang TJ, Grischott T, et al. Prices and Clinical Benefit of Cancer Drugs in the USA and Europe: A Cost–Benefit Analysis. *Lancet Oncol*. 2020;21(5):664-670.
[50] Reid B, Cohen JT, Lin PJ, Neumann PJ. Communication of launch prices by drug companies, 2022-2024. *Am J Manag Care*. 2025;31(12):e351-e352.
[51] Anderson KE, Chaiyakunapruk N, Gutierrez EJ, et al. State Out-of-Pocket Caps on Insulin Costs: No Significant Increase in Claims or Utilization. *Health Affairs*. 2024;43(8):1137-1146. doi:10.1377/hlthaff.2024.00118
[52] Garabedian LF, Zhang F, Costa R, Argetsinger S, Ross-Degnan D, Wharam JF. Association of State Insulin Out-of-Pocket Caps With Insulin Cost-Sharing and Use Among Commercially Insured Patients With Diabetes: A Pre-Post Study With a Control Group. *Ann Intern Med*. 2024;177(4):439-448.

eligible inhalers would be expected to save money, and that savings among that group would be concentrated among a small number of patients with high costs.[53] By contrast, for Medicare enrollees, the insulin out-of-pocket cap has increased insulin use, particularly in the beginning of the year, when patients may have previously struggled with affordability due to the deductibles.[54,55] **Taken together, these findings suggest a need for careful policy design that not only considers the level of rebates for eligible products, but also the current out-of-pocket cost amounts relative to proposed cap amounts.**

When considering broader caps, evaluations of Medicare's $2,000 cap provide evidence on how patients may respond. The $2,000 cap has thus far been associated with minimal changes in the use of low-cost products, but substantial increases in the use of high-cost products.[56] In research (currently under review at a peer-reviewed journal), we have shown that for cancer therapies the increases in spending for high-cost products occur for both low-value and high-value therapies, raising concerns that caps designed like the one in the IRA may increase overall spending without meaningfully improving the value of care available to patients.

Distortions in Coinsurance & Deductibles

We support establishing rules that PBMs and plans would base patient coinsurance and cost-sharing in the deductible phase on a drug's net rather than list price, and we agree that such a policy should apply broadly rather than to a subset of drugs. In fact, we recommend this approach to expanding drug-specific out-of-pocket caps, given the potential to improve affordability for larger groups of patients across therapeutic areas.

We have previously found that patient out-of-pocket costs track more closely with list than with net prices, with commercially insured patients' out-of-pocket costs for a cohort of 79 brand-name drugs increasing each year the manufacturer raised list prices.[57] Meanwhile, patient exposure to list prices has increased as more plans move toward coinsurance- and deductible-based benefit designs. For example, the proportion of Medicare Advantage enrollees facing coinsurance instead of copayments for brand-name drugs rose from 0.8% in 2019 to 27.7% in 2025, while standalone Part D plans saw an increase from 21.9% to 84.0%.[58]

[53] Mein SA, Kludze AK, Feldman WB, Tale AP, Stevens JP, Wadhera RK. Out-of-Pocket Savings Under the Inhaler Price Cap. *JAMA Intern Med*. 2025;185(3):339.
[54] Myerson R, Kim NH, Romley J, et al. Insulin Costs and Use by Medicare Beneficiaries After the Inflation Reduction Act Out-of-Pocket Cap. *JAMA*. 2026;336(1):25.
[55] Hong D, Cantos K, Morgenstern D, et al. Out-of-Pocket Spending for Insulin by Medicare Beneficiaries After Monthly Caps. *JAMA Intern Med*. 2026;186(6):723-730.
[56] Cai CL, Anderson KE, Kesselheim AS, Rome BN. Changes in Medication Use After Medicare Part D Annual Out-of-Pocket Spending Caps. *JAMA Health Forum*. 2026;7(6):e261545.
[57] Rome BN, Feldman WB, Desai RJ, Kesselheim AS. Correlation Between Changes in Brand-Name Drug Prices and Patient Out-of-Pocket Costs. *JAMA Netw Open*. 2021;4(5):e218816.
[58] Cai CL, Bhaskar A, Kesselheim AS, Rome BN. Changes in Medicare Part D Plan Designs After the Inflation Reduction Act. *JAMA Intern Med*. 2025;185(10):1266.

SECTION III: Bolstering Biopharmaceutical Innovation in the US

I. Enhancing Investments in Drug Research & Development

Basic Research

As we have explained in prior testimony to Congress, the discovery of new therapeutic interventions typically begins with foundational research, followed by translational studies and proof-of-concept testing in laboratory settings and in patients.[59] **Countless reviews and studies show the central role that NIH plays in advancing such therapeutic discovery**.[60,61,62,63] Much of the NIH's funding focuses on early discovery and development stages when private investment is least available because of the high level of risk, including research on disease mechanisms, identification of modifiable biochemical pathways, isolation of druggable targets, and development of systems for *in vitro* testing of candidate compounds.

NIH's investment ranges from relatively small sums at early stages to substantial funding at later ones, and often both.[64] All FDA-approved gene and cell therapies can be traced back to academic and research institutions or spin-offs from research at those institutions.[65] For example, the federal government was a major catalyst for new sickle cell disease gene therapies, and later-stage clinical trials to study those products additionally received NIH funding, with some studies using NIH facilities as trial sites.[66,67] In the case of CAR-T treatments useful in various forms of cancer, key discoveries related to tisagenlecleucel (Kymriah) arose from scientists at the University of Pennsylvania, who entered into licensing agreements with Novartis.[68] Public funding made meaningful financial contributions to the development of axicabtagene ciloleucel (Yescarta), for treatment of B-cell lymphoma, through grants provided by NCI to universities and academic medical centers. By our estimates, taxpayers spent over $100 million on early- and late-stage development of axicabtagene ciloleucel, including refinement of its manufacturing process and early-phase clinical testing.

Virtually all drugs can be linked in some way to public funding early in their development,[69] and roughly 25% of new drugs and 42% of biologics approved between 2008 and 2017 had late-stage reliance on public funding or could be traced to companies spun off from publicly supported

[59] Sampat BN, Lichtenberg FR. What Are the Respective Roles of the Public and Private Sectors in Pharmaceutical Innovation? *Health Aff (Millwood)*. 2011;30(2):332-339. doi:10.1377/hlthaff.2009.0917
[60] Stevens AJ, Jensen JJ, Wyller K, Kilgore PC, Chatterjee S, Rohrbaugh ML. The Role of Public-Sector Research in the Discovery of Drugs and Vaccines. *N Engl J Med*. 2011;364(6):535-541. doi:10.1056/NEJMsa1008268
[61] Barenie RE, Avorn J, Tessema FA, Kesselheim AS. Public Funding for Transformative Drugs: The Case of Sofosbuvir. *Drug Discovery Today*. 2021;26(1):273-281.
[62] Tessema FA, Barenie RE, Avorn J, Kesselheim AS. Federal Funding For Discovery And Development Of Costly HIV Drugs Was Far More Than Previously Estimated. *Health Aff (Millwood)*. 2023;42(5):642-649.
[63] Barenie RE, Darrow J, Avorn J, Kesselheim AS. Discovery and Development of Pregabalin (Lyrica): The Role of Public Funding. *Neurology*. 2021;97(17):e1653-e1660.
[64] Lalani HS, Nagar S, Sarpatwari A, et al. US Public Investment in Development of mRNA Covid-19 Vaccines: Retrospective Cohort Study. *BMJ*. 2023;380:e073747.
[65] Newham M, Vokinger KN. Adverse effects of acquisitions in the pharmaceutical industry. *Nat Med*. 2022;28(7):1342-1344.
[66] Tessema FA, Sarpatwari A, Rand LZ, Kesselheim AS. High-Priced Sickle Cell Gene Therapies Threaten to Exacerbate US Health Disparities and Establish New Pricing Precedents for Molecular Medicine. *J Law Med Ethics*. 2022;50(2):380-384.
[67] Doudna J, Marson A. Federal funding for basic research led to the gene-editing revolution. Don't cut it. *Vox.* Published April 22, 2017.
[68] Vokinger KN, Avorn J, Kesselheim AS. Sources of Innovation in Gene Therapies — Approaches to Achieving Affordable Prices. *N Engl J Med*. 2023;388(4):292-295.
[69] Ledley FD, Cleary EG. NIH funding for patents that contribute to market exclusivity of drugs approved 2010-2019 and the public interest protections of Bayh-Dole. *PLOS One*. 2023;18(7):e0288447.

research.[70,71] Drugs with links to public funding were also more likely to show indicators of therapeutic importance, such as qualifying for expedited regulatory approval. NIH support leads to new medical innovation while also supporting the US economy; according to one advocacy organization, the approximately $37 billion awarded to researchers in FY2025 supported nearly 400,000 jobs and over $94 billion in new economic activity nationwide, or $2.57 for every $1 invested.[72]

Despite this record, recent reductions in federal research funding have done substantial damage. About one in eight drugs approved since 2000 could have been at risk had current cuts been in place at the time, given the extensive connections between medical advances and research funded by grants that would have been eliminated under a sharply reduced NIH budget.[73] These reductions will affect innovation across all fields of medicine, and the losses will extend well beyond the short term as future scientists are driven out of the US or into other fields. There is also no way that private investment can make up the shortfall. **Congress should act to restore the normal flow of these funds before the US scientific establishment and the prospect of a new generation of innovative treatments is irreparably damaged.** Federal funding of research through the NIH has proven an effective way of generating breakthrough drugs, and additional funding would be extremely valuable in the push to identify treatments for unmet medical needs.

Beyond restoring NIH funding, we see three areas where policy could both support innovation and ensure that the public shares in the returns on its investment.

Contracts transferring government-funded innovations to a private company should provide for reasonable royalties and reasonable pricing. At present, these contracts rarely provide for royalties returned to the government for reinvestment in research and development or for lowering drug prices, and they never restrict the price manufacturers may charge for taxpayer-funded discoveries. Congress should examine how a reasonable pricing clause associated with government-funded work could be structured.[74] Such a provision could return funding to the research enterprise and could ensure that products whose development was largely underwritten by taxpayers are priced at levels patients can afford and commensurate with clinical value. One approach would require that the ultimate price of a product not exceed a value-based price that reflects the product's ability to improve patient outcomes relative to comparable interventions, as determined by an independent body of the kind we describe in our comments above.

Tax provisions supporting research should be tightened and, in cases in which the resulting products become highly profitable, partially recouped. Changes to the research and experimentation tax credit could more narrowly define qualifying research activities and raise documentation requirements, including greater specificity about the nature of claimed costs. Senate Democrats could also consider a "payback" provision for drugs that benefited from tax preferences during development and subsequently became blockbuster products earning over $1 billion in annual sales. One field of medicine that has seen unexpected blockbusters in recent years has been rare disease drugs. The Orphan Drug Act was enacted to encourage investment in treatments that would otherwise be unprofitable, so a

[70] Nayak RK, Avorn J, Kesselheim AS. Public sector financial support for late stage discovery of new drugs in the United States: cohort study. *BMJ*. 2019;367:l5766.
[71] Nayak RK, Lee CC, Avorn J, Kesselheim AS. Public-Sector Contributions to Novel Biologic Drugs. *JAMA Intern Med*. 2021;181(11):1522-1525.
[72] United for Medical Research. NIH's Role in Sustaining the U.S. Economy: 2026 Update. Published March 10, 2026.
[73] Azoulay P, Clancy M, Li D, Sampat BN. What if NIH had been 40% smaller? *Science*. 2025;389(6767):1303-1305.
[74] Gabriele SME, Martin MJ, Kesselheim AS, Tu SS. The strength and importance of government-funded patents for approved drugs. *Nat Biotechnol*. 2025;43(7):1050-1052.

'highly profitable' rare disease drug is difficult to reconcile with the statute's purpose.[75] Thus, Orphan Drug Act tax credits could be made repayable for extremely profitable rare disease products, and Congress could consider narrowing the definition of qualifying diseases to its original form: rare diseases for which the manufacturer is not expected to be able to make a profit. This definition would include, for example, infectious diseases prevalent in resource-poor settings around the world.

Policies that support biomedical research or that foster idea development, such as open-source drug development, may be more powerful ways of producing more transformative drugs in the future. Other solutions that have been proposed include public-private partnerships, such as the NIH's Accelerated Medicines Partnership, an effort to combine companies and academic partners in processing mountainous data on biomarker drug candidates. The NIH National Center for Advancing Translational Sciences (NCATS) represents another attempt to harness NIH-funded research to more directly serve the goal of new drug development by the pharmaceutical industry. However, this assumption by the federal budget of the high-risk, high-cost aspects of basic research that may lead to widely-used new therapies has raised concern as to whether this could lead to "socialization of risk and privatization of profit" in drug development.[76] Therefore, it is also important to think about ways to ensure that the public can gain from its investment, such as returning a small share of the revenues to the public infrastructure, perhaps by being directed into more NIH-funded research.

[75] Sarpatwari A, Kesselheim AS. Reforming the Orphan Drug Act for the 21st Century. *N Engl J Med*. 2019;381(2):106-108
[76] Avorn J, Kesselheim AS. The NIH Translational Research Center Might Trade Public Risk for Private Reward. *Nat Med*. 2011;17(10):1176-1176.