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PUBLIC FUNDING OF R&D AND FAIR DRUG PRICES ARE ESSENTIAL TO SUPPORT INNOVATION IN BIOTECHNOLOGY

Testimony of:

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Chairman Cassidy, Ranking Member Sanders, and Members of the Committee:

My name is Aaron Kesselheim. I am an internal medicine physician, lawyer, and a Professor of Medicine at Harvard Medical School, in the Division of Pharmacoepidemiology and Pharmacoeconomics of the Department of Medicine at Brigham and Women's Hospital in Boston, one of the main Harvard teaching hospitals. Within the Division, I lead the Program On Regulation, Therapeutics, And Law (PORTAL), an interdisciplinary research center that studies the intersections between prescription drug affordability and use, laws and regulations related to medications, and the development and cost of drugs. PORTAL is one of the largest non-industry-funded research centers in the country that focuses on pharmaceutical use, law, and economics. In 2020, I was elected to the National Academy of Medicine.

The topic of today's hearing is how Congress can help support the future of biotechnology innovation. One of the primary ways is ensuring that essential biotechnology products are discovered and developed to treat medical conditions, a process that is driven by public research funding in the US through the National Institutes of Health (NIH) and other federal agencies. But in the last nine months, the government has moved to cut NIH funding by 40%.

Another way Congress can support the transformative patient impact of biotechnology innovation is by helping make such innovation available to the patients who need it. This means ensuring that new therapies are sold at reasonable prices, since high costs can limit patient access. The Inflation Reduction Act (IRA) initiated a process of drug price negotiation for Medicare, but further steps should be taken to ensure that negotiation occurs in a timely fashion and that high-expenditure drugs are not exempted. I strongly urge this Committee to incentivize public investment in biotechnology discovery while promoting fair prices.

I. Research Supported by the National Institutes of Health Is a Main Engine of Biotechnology Innovation[1]

The discovery of new therapeutic interventions typically begins with foundational research, followed by translational studies and proof-of-concept testing in laboratory settings and patients. Countless reviews and studies show the central role that NIH plays in advancing such therapeutic discovery.[2] Much of the NIH's funding focuses on early drug discovery and development stages when private investment is least available due to the high level of risk. These early stages include

[1] This testimony derives in part from testimony that I gave to the House of Representatives on April 8, 2025 and on May 10, 2023. See, e.g., Kesselheim AS. National Institutes of Health funding, drug price negotiation, and biosimilars: three factors essential for patients and pharmaceutical innovation. Hearing before the House Subcommittee on Health of the Committee on Ways and Means (Rep. Buchanan, Chairman). 8 April 2025. United States Congressional Record. Available on-line at: https://waysandmeans.house.gov/wp-content/uploads/2025/04/Kesselheim-Testimony-1.pdf

[2] See, e.g., 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 Feb 10;364(6):535-41; Sampat BN, Lichtenberg FR. What are the respective roles of the public and private sectors in pharmaceutical innovation? Health Aff (Millwood). 2011 Feb;30(2):332-9.

researching disease mechanisms, identifying modifiable biochemical pathways, isolating druggable targets, and developing systems for *in vitro* testing of potential drug candidates.[3] The NIH's track record in supporting basic and translational research that underlies therapeutic innovation is particularly strong as it relates to biotechnology, the topic of the hearing today. Over the past decade, biotechnology products supported by NIH—which include biologic drugs, vaccines, and cell and gene therapies—have been growing as a share of new drug approvals and, in some cases, have shifted the paradigm for treating disease.

The NIH's investment in biotechnology products can range from relatively small sums invested at early development stages to far more substantial investment at later stages. For example, my colleagues and I recently completed a study on the cancer drug ibrutinib (Imbruvica), the first-in-class BTK inhibitor, which originated in a small biotech company, Pharmacyclics. In this case, federally-supported researchers discovered the genetic sequence of BTK and were involved with early testing of ibrutinib.[4] In part due to public support for early-stage research, ibrutinib cost as little as about $10-12 million to develop through preclinical and Phase I testing, far less than many other pharmaceuticals.[5] It was not until ibrutinib's commercial and clinical potential became clear in early-stage trials that its developers invested hundreds of millions in the drug's late-stage trials. On the other end of the financial spectrum, during the COVID-19 pandemic, government funding supported advancements in lipid nanoparticles, mRNA technology, and the SARS-CoV-2 spike protein structure to help understand the virus and provide a guaranteed market for the vaccines, which played a vital role in the rapid development of vaccines that protected millions from COVID-19 complications, with a total investment of at least $31.9 billion.[6]

In the case of CAR-T treatments that have proven useful in various forms of cancer, substantial public funding helped support their discovery, with large companies entering later in the development process. The CAR-T treatment brexucabtagene autoleucel (Tecartus) was co-developed by the National Cancer Institute, Tel Aviv Sourask Medical Center and Cabaret Biotech, the latter being a research spin-off of Israel's Weizmann Institute.[7] Even for gene therapies without direct origins in academic research settings like idecabtagene vicleucel (Abecma), the first CAR-T cell therapy for multiple myeloma, the underlying technology directed towards aspects of T cell-based products that target B-cell maturation antigen derived from work done at the NIH.[8] For these cellular-based therapies, licensing agreements with private companies allowed for important subsequent development. Key discoveries related to tisagenlecleucel

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[3] See, e.g,. Barenie RE, Tessema FA, Avorn J, Kesselheim AS. Public funding for transformative drugs: the case of sofosbuvir. Drug Discovery Today 2021;26(1):273-281 (finding $60.9 million in NIH funding linked to the development of sofosbuvir [Sovaldi] for chronic hepatitis C virus infection, including key work on virus cell culture systems).

[4] Bendicksen L, King LP, Scheffer Cliff ER, Kesselheim AS. Discovering a Transformative Cancer Drug: The Case of Ibrutinib. Drug Discovery Today 2025 (in press).

[5] Id.

[6] Lalani HS, Nagar S, Sarpatwari A, Barenie RE, Avorn J, Rome BN, Kesselheim AS. US Public investment in the development of mRNA COVID-19 vaccines: retrospective cohort study. BMJ 2023;380:e073747.

[7] Vokinger KN, Avorn J, Kesselheim AS**.** Sources of innovation in gene therapies—approaches to achieving affordable prices. New England Journal of Medicine 2023;388(4):292-295

[8] Id.

(Kymriah) arose from scientists at the University of Pennsylvania, who entered into licensing agreements with Novartis.[9] 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.[10] According to our estimates, taxpayers spent over $100 million on early- and late-stage development of axicabtagene ciloleucel, including the refinement of its manufacturing process and early-phase clinical testing.

In addition to cell therapies, all FDA-approved gene therapies can be traced back to academic and research institutions or spin-offs from research efforts at those institutions.[11] For example, the the gene therapy voretigene neparvovec (Luxturna) for inherited blindness was developed by researchers at the Children's Hospital of Philadelphia, University of Pennsylvania, Cornell University and University of Florida.[12] Spin-offs from these publicly-funded research institutions also played a major role.[13] The federal government was a major catalyst for the new sickle cell disease (SCD) gene therapies.[14] Viral vector technology used in SCD gene therapy was developed in part at the NIH.[15] CRISPR technology, a therapeutic treatment modality for SCD gene therapy, was invented at academic institutions with extensive NIH support.[16] Ongoing clinical trials to study the effects of SCD gene therapies additionally received funding from the NIH, with some studies using NIH facilities as trial sites.[17]

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[9] Id.

[10] US Senate Health, Education, Labor, and Pensions Committee Majority Staff Report. Public Investment, Private Greed. June 12, 2023.

[11] Vokinger KN, Avorn J, Kesselheim AS. Sources of innovation in gene therapies—approaches to achieving affordable prices. New England Journal of Medicine 2023;388(4):292-295

[12] Id.

[13] Id.

[14] 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. Journal of Law, Medicine & Ethics 2022;50(2):380-384

[15] Id. National Institutes of Health. NIH researchers create new viral vector for improved gene therapy in sickle cell disease. U.S. Department of Health & Human Services; 2019. Available from: https://www.nih.gov/news-events/news-releases/nih-researchers-create-new-viral-vector-improved-gene-therapy-sickle-cell-disease; Uchida N, Hsieh MM, Raines L, Haro-Mora JJ, Demirci S, Bonifacino AC, et al. Development of a forward-oriented therapeutic lentiviral vector for hemoglobin disorders. Nature Communication 2019;10(1):4479.

[16] Doudna J, Marson A. Federal funding for basic research led to the gene-editing revolution. Don't cut it. Vox. 2017. Available from: https://www.vox.com/the-big-idea/2017/4/22/15392912/genes-science-march-nih-funding-basic-research-doudna; Sherkow JS. CRISPR Patent Landscape: Past, Present, and Future. CRISPR Journal 2018;1:5-9.

[17] Bluebird bio. Longterm Follow-up of Subjects with Hemoglobinopathies Treated with Ex Vivo Gene Therapy. Clinicaltrials.gov. U.S. National Library of Medicine; 2020. Available from: https://clinicaltrials.gov/ct2/show/NCT02633943?cond=sickle+cell+gene+therapy&draw=3&rank=9; Bluebird bio. A Study Evaluating the Safety and Efficacy of the LentiGlobin BB305 Drug Product in Severe Sickle Cell Disease. Clinicaltrials.gov. U.S. National Library of Medicine;2020. Available from: https://clinicaltrials.gov/ct2/show/NCT02140554?cond=sickle+cell+gene+therapy&draw=3&rank=10; Thompson AA, Walters MC, Kwiatkowski J, Rasko JEJ, Ribeil J-A, Hongeng S, et al. Gene Therapy in Patients with Transfusion-Dependent β-Thalassemia. New England Journal of Medicine 2018;378(16):1479-1493.

A few studies have assessed drug development histories and public-sector research in the development of biologics and other biotechnology products. In a study published in JAMA Internal Medicine, we found that about two-fifths (42%) of new biologic drugs approved between 2008 and 2017 had late-stage reliance on public funding or could be traced to companies that were spun off from publicly-supported research.[18] Biologics with links to public funding were also more likely to have indicators of therapeutic importance, such as qualifying for expedited regulatory approval. This is consistent with the growing recognition that government, academic, and non-profit funding plays a substantial late-stage role in new drug discovery and development. We also found several examples in which public support was directly used to finance the clinical testing of a drug. Many of these examples were for drugs treating rare diseases or with biosecurity implications (e.g., anthrax antitoxin) for which there may not be financial incentives from the private market.[19]

Despite this track record, the current Presidential administration has already threatened NIH funding and is poised to do substantially more damage. This year, the administration has targeted universities, blocking funds from scientists and other researchers doing groundbreaking work.[20] According to a review by the Brookings Institution, the health budget for 2026 includes only $27.5 billion for NIH, about a 40% from the 2025 appropriation of $48 billion.[21] Although the final budget should depend on Congressional action, about one-in-eight drugs approved since 2000 could have been at risk had those cuts been in place at the time given the "extensive connections between medical advances and research that was funded by grants that would have been cut if the NIH budget was sharply reduced."[22]

The cuts will affect innovation in all fields of medicine, including breast cancer[23] and HIV.[24] The destruction of funding will lead to losses in opportunities for scientific discovery not only in the short-term, but for generations to come as future scientists are driven out of the US or into other fields entirely.[25] There is also no way that private investment can make up for such shortfall. As my colleague Jerry Avorn put it, "The amount available to universities from pharma is smaller;

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[18] Nayak R, Lee CC, Avorn J, Kesselheim AS. Public-sector contributions to novel biologic drugs. JAMA Internal Medicine 2021;181(11):1522-1525.

[19] Lupkin S. How Operation Warp Speed's Big Vaccine Contracts Could Stay Secret. National Public Radio. September 29, 2020. Available at: https://www.npr.org/sections/health-shots/2020/09/29/917899357/how-operation-warp-speeds-big-vaccine-contracts-could-stay-secret.

[20] Johnson CY, Douglas-Gabriel D, Brasch B. Trump slashed university funding. Here are 6 key drugs that relied on it. Washington Post. Oct 7, 2025.

[21] Frank RG. The 2026 Health and Health Care Budget. June 27, 2025. Available at: https://www.brookings.edu/articles/the-2026-health-and-health-care-budget/

[22] Azoulay P, Clancy M, Li D, Sampat BN. What if NIH had been 40% smaller? Science. 2025;389(6767):1303-1305.

[23] Coffey S. Concerns rise over NIH budget cuts impact on breast cancer research, innovation. Yahoo.com News. October 24, 2025.

[24] Ryan B. Republicans seek deep cuts to HIV prevention and treatment funding.. NBC News. March 26, 2025 (one expert projected that one cut to HIV programs "would raise the number of new infections by 12% by 2030.")

[25] Witze A. 75% of US scientists who answered Nature poll consider leaving. Nature March 27, 2025, available at: https://www.nature.com/articles/d41586-025-00938-y (poll of over 1600 scientists find that many are looking for work in Europe and Canada).

despite the industry claim that it is the wellspring of pharmaceutical innovation, most major companies spend a far smaller share of revenues on innovative research than on promotion and marketing, stock buyback programs, shareholder dividends, and executive compensation."[26] NIH support leads to new medical innovation while it also has supported the US economy; according to one advocacy organization, "the $36.94 billion awarded to researchers in the 50 U.S. states and the District of Columbia in FY2024 supported 407,782 jobs and $94.58 billion in new economic activity nationwide — or $2.56 for every $1 invested."[27]

The US government has for decades played a fundamental role in the discovery and development of important new drugs and vaccines through the NIH and other sources of public funding. In the face of meaningful budget cuts initiated by the Trump administration and its appointees at the NIH and other agencies, Congress must do whatever it can to re-establish the normal flow of these funds before the US scientific establishment and the prospect of a new generation of innovative treatments is irreparably damaged.

II. Biotechnology Innovation Must Be Available to Patients at Fair Prices

Impactful biotechnology innovation also depends on the therapeutic products being available to patients at fair prices that adequately recognize the private investment in those products. Congress must ensure that US patients and the health care system do not pay excessive prices for biotechnology products that are out of proportion to the value they offer patients and do not account for the substantial public investment that can accompany these products' development. In the US, we allow manufacturers to price their products at whatever level they want, which leads biotechnology companies to establish high prices that can limit patient access and strain the budgets of payers, including government insurance programs like Medicare and Medicaid. For example, US health care spending on biologic drug products is rising. In the 5-year period 2019-2024, in retail settings, spending on biologics rose 80% versus only 14% for small-molecule drugs.[28] Medicare Part B spending on biologics more than tripled from 2008 to 2021, with biologics representing 79% of Medicare Part B prescription drug spending in 2021. Cellular and genetic therapies are also priced at exceedingly high levels, sometimes surpassing a million dollars per treatment. For example, at the published price of $2.2 million for an SCD gene therapy,

[26] Avorn J. Corporate support cannot make up for threats to the NIH budget. STAT News First Opinion. Oct 22, 2025. Avialable at: https://www.statnews.com/2025/10/22/nih-budget-cuts-pharmaceutical-industry-research/ ("Greater reliance on corporate largesse can never be a satisfactory alternative to a healthy and adequately budgeted source of peer-reviewed public support.")

[27] United for Medical Research. UMR Releases Annual NIH Economic Impact Report: 2025 Update. March 11, 2025. Available at: https://www.unitedformedicalresearch.org/statements/umr-releases-annual-nih-economic-impact-report-2025-update/

[28] IQVIA. Biosimilars in the United States 2023-2027. Available from: https://www.iqvia.com/insights/the-iqvia-institute/reports-and-publications/reports/biosimilars-in-the-united-states-2023-2027.

we estimated a 5-year budgetary impact for state Medicaid programs of $837.5 million, with one product alone reaching nearly one-fifth of overall state spending on SCD.[29]

The growing challenge of paying for biotechnology products has raised questions about the fair pricing of these products, particularly for those that received substantial public-sector support in research and development. For example, in 2015, Kite's anticipated base price for the CAR-T axicabtagene ciloleucel was $150,000; however, in 2017, Gilead Sciences acquired Kite for $11.9 billion. When the product received FDA approval, Gilead set an initial launch price of $373,000 and then, in subsequent years, increased this price by more than $50,000.[30] At launch, the median price for a year of treatment with a new FDA-approved product increased from $2,115 in 2008 to about $300,000 in 2023.[31] As a result of high prices, about one-third report being unable to afford their medications.[32] Rising drug costs are passed on to consumers either as out-of-pocket costs or through higher premiums, which makes insurance less affordable and available. Medicaid programs, for example, have had to respond to expanding prescription drug costs by cutting coverage for other services and limiting access to medications.[33]

In this context, the Inflation Reduction Act of 2022 offered important relief for the health care system by allowing Medicare to directly negotiate prices for certain drugs, just as it negotiates prices with other entities that provide goods or services to the Medicare program, such as hospitals, doctors, and clinical laboratories.[34] Negotiation is based on a number of measurable factors, such as whether the drug represents an important therapeutic advance or fulfills an unmet medical need, whether it has recouped its research and development costs, and any federal funding that contributed to its discovery.[35] Biologic drugs are only eligible to be sold at negotiated prices once they have been on the market for at least 13 years, during which time manufacturers have been able to earn tens of billions of dollars in US and global sales. Among the first ten drugs negotiated by the Centers for Medicare and Medicaid Services under the IRA in

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[29] Harvey JP, Raymakers AJN, Rand LZ, Goshua G, Kesselheim AS, Pandya A. Modeling the budgetary impacts of sickle cell disease gene therapies on state Medicaid programs. J Gen Intern Med. 2025 Sep 17.

[30] US Senate Health, Education, Labor, and Pensions Committee Majority Staff Report. Public Investment, Private Greed. June 12, 2023.

[31] Rome BN, Egilman A, Kesselheim AS. Trends in prescription drug launch prices, 2008-2021. JAMA 2022;327(21):2145-2147**;** Beasley D. Prices for new US drugs rose 35% in 2023, more than the previous year. Reuters. February 23, 2024.

[32] I-MAK. Understanding Americans' top concerns on drug pricing: corporate greed. Oct 2025. Available at: https://reports.i-mak.org/drug-pricing-concerns ("Among the 71% of adults who reported taking prescription medications in the past year, one in three (31%) did not fill at least one prescription due to cost.")

[33] Galewitz P. States cut Medicaid drug benefits to save money. Kaiser Health News July 24 2012. Available at: https://khn.org/news/medicaid-cuts-sidebar/

[34] This testimony derives in part from testimony that I previously gave to the Energy and Commerce Committee on September 20, 2023. See The Inflation Reduction Act of 2022: reducing excessive spending and Supporting patient access to brand-name drugs while promoting meaningful innovation. Hearing before the House Subcommittee on Oversight and Investigations of the Committee on Energy and Commerce (Rep. Griffith, Chairman). 20 September 2023. United States Congressional Record. Available on-line at: https://energycommerce.house.gov/events/oversight-and-investigations-subcommittee-hearing-1

[35] Hwang TJ, Kesselheim AS, Rome BN. New reforms to prescription drug pricing in the US: opportunities and challenges. JAMA 2022;328(11):1041-1042.

2024, negotiation led to discounts ranging from 38% to 79% off list prices. Notably, the final prices were still higher than the prices paid for these same drugs in comparable high-income countries around the world.[36] One review examining drugs expected to be negotiated in 2026-2028 found that they accounted for $67.4 billion in Medicare spending and treated conditions such as diabetes, cancer, and cardiovascular disease, suggesting savings and out-of-pocket cost reductions may be achievable for common chronic conditions through negotiation.[37]

While the IRA was an important step toward curbing excessive drug prices, additional Congressional action is needed to ensure that biotechnology innovations are accessible to the patients who need them. This can be achieved in a few ways. First, Congress should fix the "biologic bonus," the period of 4 years of extra delay in negotiation that applies to biologic drugs beyond the standard 9-year delay for small-molecule drugs. Biologic drugs do not need a delay in qualifying for negotiation. In a recent study published in JAMA[38] examining 599 new therapeutic agents approved by the FDA from 2009 to 2023, of which 159 (27%) were biologics and 440 (73%) were small-molecule drugs, we found that median development times were nearly identical for biologics (12.6 years) and small-molecule drugs (12.7 years). In addition, biologics had *higher* clinical trial success rates at every phase of development. Median development costs were not statistically different, and biologics were protected by a median of 14 patents compared with 3 patents for small-molecule drugs. The median time to follow-on competition was 20.3 years for biologics compared with 12.6 years for small-molecule drugs. Finally, the median annual cost of treatment was $92,000 for biologics and $33,000 for small-molecule drugs. Indeed, biologics had higher median revenues than small-molecule drugs in each year after FDA approval. In another recent study, focusing on top-selling drugs in Medicare, we found that biologics, on average, earned $7.3 billion more in cumulative revenue than small-molecule drugs during their first 13 years on the market,[39] showing how the IRA overly rewards the development of biologics relative to small-molecule drugs. These special legal and regulatory protections for biologics in the US are not justified by differences in development costs or risks, and Congress should therefore align the negotiation timeline for biologics with that of small-molecule drugs.

Second, while the IRA currently limits drug price negotiation to Medicare, these negotiated prices should be extended to benefit all US patients, including those with private insurance. This was initially intended to be included in the IRA before the bill was limited to Medicare by the Senate Parliamentarian.

Third, Congress should ensure that there is direct competition for biotechnology products in a reasonable time frame by preventing manufacturers from amassing large thickets of patents that

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[36] Rome BN, Kesselheim AS, Feldman WB. Medicare's first round of drug-price negotiation – measuring success. New England Journal of Medicine 2024;391(20):1865-1868.

[37] Dickson S, Hernandez I. Drugs likely subject to Medicare negotiation, 2026–2028. J Manag Care Spec Pharm. 2023;29(3):229–235.

[38] 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):2101-2108.

[39] Vogel M, Feldman WB, Cowan Z, Rome BN, Chandra A, Kesselheim AS, Wouters OJ. Revenue Differences Between Top-Selling Small-Molecule Drugs and Biologics in Medicare. JAMA Health Forum 2025;6(10):e254720.

delay the entry of biosimilars or other products that lead to price reductions. In a review of top-selling biologic drugs, we found that they were protected by a median of 8 patents at the time of approval (IQR: 7-20), and the median biologic patent thicket reached a peak density of 41 active patents (IQR: 18-58) at 13 years after approval, of which 76% came from post-approval patents.[40] The biosimilar version of etanercept (Enbrel) launched in Europe 13.1 years earlier than the expected US entry; its European patent thicket was over 4-times less dense.[41] Such competition can be impactful in lowering prices. We found that in the first year after US entry of biosimilar versions of adalimumab, there was a nearly 50% decrease in adalimumab net spending and prices.[42] Strategies for inhibiting excessive patent thickets include revisiting a bipartisan bill in Congress that would have permitted patentees to enforce against generic and biosimilar manufacturers only one patent per group connected by terminal disclaimers,[43] or resurrecting a recently abandoned US Patent and Trademark Office (USPTO) proposed rule that would have established that if any claim in a patent is found invalid, all patents linked to it via terminal disclaimer would become unenforceable.[44] Congress should also pass legislation to prevent the USPTO from undermining the work of the Patent Trial and Appeals Board, which has been useful in reducing biologic drug patent thickets,[45] but has been threatened by recent proposed changes by the new administrators in charge of the USPTO.

It's particularly important for Congress to act because recent administrative announcements on drug pricing are unlikely to result in meaningful reductions in the prices patients actually pay. First, the administration has announced private deals with some pharmaceutical manufacturers in which the manufacturers promised to introduce future drugs at prices for Medicaid only that are comparable to those in other countries. However, most new drugs are launched first in the US before they have comparable prices in other countries. In addition, Medicaid already gets prices for many brand-name drugs that can approach price levels in other countries because of guaranteed rebates, best price match guarantees, and rebates for price increases over inflation. In these deals, the Trump administration also announced that it would be setting up a website to help manufacturers facilitate direct-to-consumer sales of their brand-name drugs at reduced prices, which generally involves patients paying the manufacturers directly outside of their insurance companies. However, most patients cannot afford to pay out-of-pocket for brand-name drugs that often cost thousands of dollars per month, even at the discounted prices offered

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[40] Horrow C, Gabriele SME, Tu SS, Sarpatwari A, Kesselheim AS. Patent portfolios protecting 10 top-selling prescription drugs. JAMA Internal Medicine 2024;184(7):810-817.

[41] I-MAK. Overpatented, overpriced. Sept 2022. Available at: https://www.i-mak.org/wp-content/uploads/2023/01/Overpatented-Overpriced-2023-01-24.pdf

[42] Rome BN, Bhaskar A, Kesselheim AS. Use, spending, and prices of adalimumab following biosimilar competition. JAMA Health Forum 2024;5(12):e243964.

[43] Peter Welch Press Release. Welch, Braun, and Klobuchar Introduce Bipartisan Legislation to Streamline Drug Patent Litigation, Lower Cost of Prescription Drugs. https://www.welch.senate.gov/welch-braun-and-klobuchar-introduce-bipartisan-legislation-to-streamline-drug-patent-litigation-lower-cost-of-prescription-drugs/.

[44] Patent and Trademark Office. Terminal Disclaimer Practice to Obviate Nonstatutory Double Patenting. Notice of Proposed Rulemaking. https://public-inspection.federalregister.gov/2024-10166.pdf.Accessed June 22, 2024).

[45] 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.

through these websites. The overall limited impact of these deals has been made clear as manufacturers' stock prices have tended to remain stable or even increase coincident with these announcements.

In addition, Congress should fix its recent move to pass legislation as part of the One Big Beautiful Bill Act (OBBBA) that expanded the rare disease exclusion in the IRA. The IRA had made drugs approved exclusively for a single rare disease exempt from negotiation. But the OBBBA expanded this "sole orphan" exclusion to cover drugs FDA-approved for more than one rare disease indication, and it delayed the start of the 7- or 11-year negotiation period for drugs first approved for a rare condition until the date they later receive approval for a non-rare condition. These steps will unnecessarily limit the number of drugs eligible for Medicare price negotiation. In a previous analysis, we found that among the nearly 300 drugs with over $200 million in Medicare sales from 2012-2021, 20 drugs were multi-orphan drugs and 13 were orphan-first drugs that would be affected by the proposed expanded carve-outs; these drugs collectively accounted for $183 billion in Medicare drug spending.[46] Under OBBBA, the blockbuster cancer drugs pembrolizumab (Keytruda) and nivolumab (Opdivo) will be exempt from negotiation for an additional year, and in 2023 the Medicare spent over $7 billion on these two drugs alone.[47] The Congressional Budget Office recently concluded that this change could cost the health care system $8.8 billion over the next decade. Instead, Congress should pass the recently-introduced No Big Blockbuster Bailouts Act (NOBBBA), which allows rare disease-designated drugs that account for $400 million per year or more in Medicare sales to be eligible for negotiation.[48] We should not worry about this bill taking away incentives for investment in rare disease biotechnology products, because when weighing the prospect of earning over $400 million in Medicare against the possibility that under the IRA, Medicare will seek to negotiate a fair price for the drug that would be higher than in other settings around the world *and* that would take effect about a decade after FDA approval, a rational biotech company would still seek to pursue this investment.

Finally, making biotechnology innovation available to patients also requires that patients have adequate insurance coverage, but the government has moved in recent months to reduce enrollment in Medicaid and is currently poised to allow insurance marketplace premiums to soar to levels beyond what many patients can pay. In addition to seeking fair prices for biotechnology products, making affordable insurance available to as many people as possible can help ensure that innovative biotechnology products reach the patients who need them.

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[46] Vogel M, Zhao O, Feldman WB, Chandra A, Kesselheim AS, Rome BN. Cost of exempting sole orphan drugs from Medicare negotiation. JAMA Internal Medicine 2024;184(1):63-69.

[47] Mooney H, Kesselheim AS, Rome BN. Congress should remove the rare disease carve-out from Medicare drug price negotiation, not expand it. *Health Affairs Forefront*. June 30, 2025. Available on-line at: https://www.healthaffairs.org/content/forefront/congress-should-remove-rare-disease-carve-out-medicare-drug-price-negotiation-not

[48] See bill at: https://www.welch.senate.gov/wp-content/uploads/2025/10/Bill-Text-No-Big-Blockbuster-Bailouts-Act-Welch-20251021.pdf.