IN THE UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF MARYLAND

ASTRAZENECA PHARMACEUTICALS LP, et al., Plaintiffs, vs. ROBERT F. KENNEDY, JR., in his official capacity as Secretary of the U.S. Department of Health and Human Services, et al., Defendants.	§	Civil Action No. 1:25-cv-04212-MJM

BRIEF OF AMICI BAUSCH HEALTH COMPANIES INC., BRISTOL MYERS SQUIBB COMPANY, ELI LILLY AND COMPANY, JOHNSON & JOHNSON, SANOFI-AVENTIS U.S. LLC, AND BIOTECHNOLOGY INNOVATION ORGANIZATION IN SUPPORT OF PLAINTIFFS' MOTION FOR SUMMARY JUDGMENT

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INTEREST OF AMICI CURIAE

Amici Bausch Health Companies Inc., Bristol Myers Squibb Company, Eli Lilly and Company, Johnson & Johnson, and Sanofi-Aventis U.S. LLC are among the leading biopharmaceutical research companies in the world. The Biotechnology Innovation Organization is the principal trade association representing the biotechnology industry. As innovators, amici invest billions of dollars to develop innovative products that improve and save people's lives. Amici believe that the guidance implementing the Inflation Reduction Act of 2022 sharply impacts innovators' ability to bring new lifesaving drugs to market, with harmful effects on public health.

INTRODUCTION

Pharmaceutical innovators invest billions of dollars every year to develop safe and effective medications that save people's lives. But success is far from guaranteed: Only 0.02% of therapies in development are ever approved to enter the market, and only a third of those will recoup their development costs.[1] As a result, innovators have long depended on free-market sales and exclusivity rights over their products to regain the capital necessary to reinvest in future breakthrough treatments.

The Inflation Reduction Act of 2022 (IRA) dramatically altered this settled understanding of fundamental market realities. In seeking to lower the cost of Medicare, Congress instructed the Centers for Medicare & Medicaid Services (CMS) to identify a limited number of top-spend medications that have been approved and marketed for a given number of years and to then

[1] *See* Sandra Kraljevic et al., *Accelerating Drug Discovery*, 5 Eur. Molecular Biology Org. Reps., no. 9, 837 (2004), https://tinyurl.com/525p87tp; John A. Vernon & Joseph H. Golec, *Pharmaceutical Price Regulation: Public Perceptions, Economic Realities, and Empirical Evidence* 7 (2008), https://tinyurl.com/2k3hfyw5; U.S. Food & Drug Admin., *The FDA's Drug Review Process: Ensuring Drugs Are Safe and Effective* (Nov. 24, 2017), https://tinyurl.com/32xnaus2.

"negotiate" with the manufacturers of those medications to steeply discount the prices that manufacturers may charge for them. But the IRA's dubiously named "Drug Price Negotiation Program" (DPNP) allows for "negotiation" in name only. After CMS identifies a drug, it gets to name its price. The manufacturer, in turn, must accept the price and provide access to the drug at that price no matter how unreasonable, or else either face crushing penalties or withdraw all its medicines from the Medicare and Medicaid programs entirely. Those are choices no manufacturer can afford to make. So the "negotiation" between CMS and manufacturers exists only "in the Vito Corleone sense—an offer one can't refuse."[2]

In this forced-sale regime, Congress limited the agency's capacious grant of statutory authority by prescribing *which* and *how many* medications CMS can select for "negotiation." Congress defined "qualifying single source drugs"—what CMS must rank when selecting top-spend medications—as including only drugs that had been approved by the Food and Drug Administration (FDA) and marketed for at least seven years.[3] Congress's choice to exclude newly approved medications from price "negotiation" under the DPNP's forced-sale regime shows that it wanted innovators to recoup at least some meaningful portion of their multi-billion-dollar investments in new and life-saving drugs.

CMS's implementing guidance contravenes the statute's few but important limitations on the DPNP. Most importantly, the guidance redefines "qualifying single source drug" to include all of a manufacturer's medications with the same "active moiety" (i.e., the part responsible for

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[2] Daniel Hemel, *A Complete Breakdown of the Good, the Bad, and the Ugly in the Inflation Reduction* Act, Slate (Aug. 10, 2022), https://tinyurl.com/3zttxhat.

The IRA's Drug Price Negotiation Program is itself being challenged as unconstitutional in other litigation. This lawsuit challenges only CMS's guidance.

[3] In addition to small-molecule drugs, the DPNP also covers biologics. *See* 42 U.S.C. § 1320f-1(e)(1)(A). As relevant here, the DPNP treats small-molecule drugs and biologics in essentially the same way.

the physiological or pharmacological action)—even though that term is nowhere in the DPNP.[4] CMS claims it can subject even *newly approved products* to the DPNP, so long as those products share the active moiety of an earlier product that had been approved for long enough to be "negotiation-eligible." As a result, the guidance effectively does away with the ineligibility period for many new medications and results in many more medications being subject to the DPNP's regime than the text of the statute allows.

By subjecting *brand-new* products to the DPNP's forced-sale regime, CMS's guidance disincentivizes manufacturers from innovating and developing new medications and lifesaving treatments—with devastating consequences for public health. Amici urge this Court to grant the Plaintiffs' motion for summary judgment, declare CMS's guidance unlawful, and set the guidance aside.

ARGUMENT

I. CMS Exceeded Its Authority By Expanding The Number and Types Of Medicines That Congress Made Eligible For The DPNP.

When Congress authorized CMS to select certain top-spend medications for the DPNP's forced-sale regime, it restricted which and how many medications CMS could select. That choice reflects a compromise that Congress struck when it passed the IRA. "Passing a law often requires compromise, where even the most firm public demands bend to competing interests." *N.L.R.B. v. SW Gen., Inc.*, 580 U.S. 288, 306 (2017). And "agencies must respect and give effect to these sorts of compromises," which reflect Congress's best attempt to deal with "groups with marked but divergent interests." *Ragsdale v. Wolverine World Wide, Inc.*, 535 U.S. 81, 93-94 (2002). CMS has failed to do so here. Its guidance broadens the DPNP beyond recognition and disregards the few statutory guardrails Congress put in place.

[4] *See* 21 C.F.R. § 314.3(b) (defining "active moiety").

The IRA directs CMS to rank Medicare's top-spend, "negotiation-eligible drugs" and select a certain number for price "negotiation." 42 U.S.C. § 1320f-1(a), (b)(1)(A), (d)(1). But instead of giving the agency boundless discretion to do so, Congress prescribed specific criteria for identifying those medications—that is, the top 50 highest-spend "qualifying single source drugs." *Id.* § 1320f-1(e)(1). Congress defined "qualifying single source drug" as a drug that (1) "is *approved* [by the FDA] … and is marketed pursuant to such approval"; (2) "for which, as of the selected drug publication date … at least 7 years will have elapsed since the date of such approval"; and (3) that is "not the listed drug for any drug that is approved and marketed" as a generic. *Id.* § 1320f-1(e)(1)(A) (emphasis added). This language indicates that Congress tied the eligibility of the medications to their particular applications for FDA "approval," such that a drug is eligible for the DPNP only if it has already been marketed for at least seven years under its specific new drug application ("NDA"). *See* Mot. for Summ. J. ("MSJ") at 14-17.

Those limitations reflect Congress's attempt to balance the "competing interests" at issue, *SW General, Inc.*, 580 U.S. at 306—such as manufacturers' need to recoup their investments, which they could then reinvest into researching future medicines, and the government's desire to lower the cost of Medicare. Especially because the IRA stacks the decks so decisively against manufacturers, it is critical for this Court to ensure that CMS complies with the few statutory limitations that Congress prescribed. But CMS's implementing guidance strays far away from this plain text, redefining "qualifying single source drug" to include all drugs "with the same active moiety and the same holder of" an NDA, "inclusive of products that are marketed pursuant to different" applications.[5] Put more simply, the guidance claims CMS can aggregate

[5] *See* CMS, *Medicare Drug Price Negotiation Program: Final Guidance, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2027 and Manufacturer Effectuation of the Maximum Fair Price in 2026 and 2027 for Initial Price Applicability Year 2026*, at 167-68 (Oct. 2, 2024), https://tinyurl.com/52a6e8c7 ("Final Guidance").

all of a manufacturer's products that have the same active moiety into one fictional "super drug," regardless of whether each distinct product may have only recently obtained FDA approval under its own regulatory application.[6] So a newly approved drug may be deemed eligible for the DPNP *well before* its seven-year period has expired—or, indeed, immediately upon approval—so long as it has the same active moiety as another marketed drug from the same manufacturer that the FDA approved at least seven years ago. *See* MSJ at 13, 19. That is true even if the new drug treats a different condition, is administered differently, or has a different active ingredient (e.g., a more stable prodrug or salt of the original compound).

Take, for example, a hypothetical manufacturer that discovered a particular molecule ("Molecule A"), which it hoped would be effective in fighting skin cancer. After extensive trial-and-error, the manufacturer developed the medication—call it Product AB®—which FDA approved in 2014 and quickly became a standard treatment for melanoma. Now imagine that scientists also believed that "Molecule A" could yield other benefits. After years of further testing, the manufacturer discovered that its product also results in weight loss. In 2024, FDA approved the anti-obesity indication as Product ABD®. Because the manufacturer marketed Product AB®—the melanoma medication—for more than a decade, *that* medication might be a "qualifying single source drug" and thus eligible for the DPNP. But the same should not be true for Product ABD®, which FDA *approved* recently under a distinct application to treat a different medical condition. Similarly, imagine that after years of research the manufacturer discovered a much more stable form of the original drug that produces many fewer side effects. In 2025,

[6] *Id.* at 168-69. To be sure, CMS treats so-called "fixed combination drugs," 21 C.F.R. § 300.50, slightly differently. *See* CMS, *Medicare Drug Price Negotiation Program: Final Guidance, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2028 and Manufacturer Effectuation of the Maximum Fair Price in 2026, 2027, and 2028*, at 167 (Sep. 30, 2025), https://tinyurl.com/2exc6t88.

FDA approved that different active ingredient for treatment of melanoma and obesity as Product ABDE®. That recent product with its new active ingredient approved under a distinct application should not be eligible for the DPNP under the statute.

Yet under its guidance, CMS could unilaterally force Products ABD® and ABDE® to be sold at slashed prices from the very moment they enter the market—simply because they have the same "active moiety" as Product AB®. Never mind the years and hundreds of millions of dollars expended to develop and rigorously test Products ABD® and ABDE®. CMS's aggregation of distinct, separately approved medications makes it virtually impossible for innovators to recoup their investments and to continue to innovate, reinvest and develop new products.

Further, CMS's interpretation cannot be squared with other federal laws, which similarly recognize that distinct products should be treated separately in order to incentivize innovation. For example, the Hatch-Waxman Act, Pub. L. No. 98-417, 98 Stat. 1585 (1984), and the Biologics Price Competition and Innovation Act (BPCIA), Pub. L. No. 111-148, § 7001, 124 Stat. 119, 804 (2010), authorize an abbreviated path to FDA approval for generics and biosimilars so long as these products are tied to the innovator's *distinct* NDAs or Biologics License Applications (BLAs). *See* 21 U.S.C. § 355(j)(2)(A)(ii), (iv); 42 U.S.C. § 262(k)-(l). By operating on a product-by-product basis, each law reflects Congress's longstanding commitment to *both* making safe and effective treatments more accessible to patients *and* fostering innovation. CMS's guidance and its aggregation of different, separately approved products accordingly breaks with the balance struck by Congress's successful Hatch-Waxman and BPCIA regimes.

* * *

In issuing guidance that substantially broadens the sweep of the IRA, CMS has failed to act "within its statutory authority." *Loper Bright Enters. v. Raimondo*, 603 U.S. 369, 412 (2024). CMS has taken it upon itself to target medications beyond those that Congress intended and compel their sale at prices that are dramatically slashed, often by more than 50 percent.[7] Thus, CMS's (re)definition of "qualifying single source drug" contradicts the IRA's text and the negotiated compromises underlying that legislation. And where, as here, the agency has failed to "respect and give effect to these sorts of compromises," it is this Court's job to vindicate Congress's intent and reject the agency's unauthorized expansion of the statute. *Ragsdale*, 535 U.S. at 94.

II. CMS's Guidance Stifles Innovation And Harms The Public Health.

CMS's guidance discourages the development of new products and harms the public health. By defining "qualifying single source drug" in a manner that bundles different products, regardless of when they are approved to enter the market, CMS has paved the road for having fewer medications that improve and save people's lives. The guidance's distorting effect on innovation is too significant to be cast aside.

A. Innovating new indications and compositions improves patients' lives.

To fully appreciate the guidance's threat on innovation, consider first the countless ways drug manufacturers continue to innovate even after a drug is initially approved.

Indications. An "indication" is a medical condition that a drug is used to treat or prevent. For example, a drug indication of insulin is Type 2 diabetes. Often, as a result of extensive research and clinical testing, one drug will have more than one indication, meaning that it can be used to treat more than just one condition. For example, the FDA has approved tirzepatide

[7] *See* CMS, *Medicare Drug Price Negotiation Program: Negotiated Prices for Initial Price Applicability Year 2026*, at 2 (Aug. 2024), https://tinyurl.yfj2wjn9.

medications to lower blood glucose for patients with Type 2 diabetes *and* to treat obesity and obstructive sleep apnea.

Before or after the FDA approves a drug for one indication, that drug's manufacturer often begins post-approval research of additional indications. That is for good reason: Post-approval research and development (R&D) for new indications is "vital to addressing unmet needs for patients."[8] For example, "a medicine approved to treat asthma in adults may be studied post-approval for safety and efficacy in children."[9] Similarly, the manufacturer of a medicine that treats a rare disease may find that the medicine is "relevant to multiple diseases."[10] The benefits of this post-approval innovation are real. One recent study concluded that 63% of medicines first approved as orphan drugs—that is, drugs approved to treat a single rare disease or condition—"were awarded at least one post-approval indication."[11]

Drug compositions, presentations, and delivery mechanisms. A drug's pharmaceutical composition, presentation, and delivery mechanism relate to both how (and how often) the drug is administered (e.g., capsule or intravenous injection) and its physical features. These key characteristics of a medication matter greatly to patients. Unsurprisingly, patients prefer—and are more likely to take—medicines that are easy to consume or use.

There are countless real-life examples of these innovation-related benefits. Take for example Gilead Sciences, Inc.'s long-acting antiviral medication for HIV prevention, lenacapavir, which requires only twice-a-year dosing.[12] Prior to lenacapavir, antiviral

[8] Partnership for Health Analytic Research, *Implications of the Inflation Reduction Act Price Setting Provisions on Post-approval Indications for Small Molecule Medicines* 4, 12 (2023), https://tinyurl.com/mr2yzuft.

[9] *Id.* at 3.

[10] *Id.* at 3-4.

[11] *Id.* at 2.

[12] Julia Paik, *Lenacapavir*: *First Approval*, 82 Drugs 1499 (2022), https://tinyurl.com/3unhrj7b.

medications for HIV prevention required more frequent administration.[13] Another example is Neurelis's diazepam nasal spray, which treats acute repetitive seizures. The nasal spray served as an easier-to-administer alternative to diazepam rectal gel.[14] Similarly, Arecor Therapeutics is developing a new version of insulin that accelerates the drug's absorption and thus requires smaller amounts for each injection than previous insulin products.[15] Finally, consider Mitsubishi Tanabe Pharma America, Inc.'s medication, edaravone, which treats patients with amyotrophic lateral sclerosis (ALS or Lou Gehrig's disease), a motor neuron disease. In 2017, FDA approved edaravone for daily intravenous (IV) infusion in ALS patients in medical settings.[16] But subsequent R&D resulted in FDA approval of an oral version of edaravone, allowing patients to receive treatment in their own homes.[17]

Each of these new products—all of which required separate FDA approvals under their own applications—ensures that patients will take and benefit from the drug. These innovations transform people's lives.

Innovating new indications, as well as the compositions, presentations, and delivery mechanisms of new products. Market demand and unmet needs spur market participants to search for innovative solutions. Pharmacological innovation—including searching for new indications and developing new and improved versions of existing drugs—is no exception. For

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[13] Giovanni Di Perri, *Pharmacological Outlook of Lenacapavir: A Novel First-in-Class Long-Acting HIV-1 Capsid Inhibitor*, La Infezioni in Medicina, 495, 498 (2023), https://tinyurl.com/mryaf8nn.

[14] R. Edward Hogan et al., *Bioavailability and Safety of Diazepam Intranasal Solution Compared to Oral and Rectal Diazepam in Healthy Volunteers*, Epilepsia (2020), https://tinyurl.com/4mx6hken.

[15] Arecor Therapeutics plc, *AT278 Ultra-Concentrated Ultra-Rapid Acting Insulin Demonstrates Superiority in Phase 1 Clinical Trial in Overweight and Obese People with Type 2 Diabetes* 1 (May 20, 2024), https://tinyurl.com/2p8k2mjf.

[16] FDA News Release, *FDA Approves Drug to Treat ALS* (May 5, 2017), https://tinyurl.com/bde9p87b.

[17] Mitsubishi Tanabe Pharma America, Inc., *Mitsubishi Tanabe Pharma America Presents 48-Week Results from Global Phase 3 Safety Clinical Study of RADICAVA ORS® (edaravone), an Oral Treatment for ALS* (June 1, 2022), https://tinyurl.com/49neccx8.

example, when a disease or condition lacks an adequate treatment, innovators either develop new medications or search for new indications for existing drugs.[18] This "[d]evelopment of and regulatory approval of new uses of already-approved drugs and biologics is an important source of innovation by biopharmaceutical firms."[19] Likewise, where the drug presentation or delivery mechanism is burdensome and patient adherence to the treatment is accordingly low, innovators seek to develop a new composition, presentation, or delivery mechanism of the drug.[20]

Drug manufacturers are constantly innovating to find new, better ways to improve patients' health. But pharmaceutical innovation is not easy—or cheap. It requires a great deal of scientific knowledge, time, and money. In searching for innovative solutions, manufacturers make critical decisions early in the drug development process, and those decisions dictate the path to approval. In the development stage, for example, manufacturers determine the drug composition, presentation, and delivery mechanism they intend to pursue and test in subsequent clinical trials.[21] Those decisions matter greatly because FDA's ultimate approval of the medication is generally limited to the indication and version of the drug tested during its development. Any subsequent indication or new version must undergo its own approval, which can require companies to restart the entire R&D process—all the way from the initial research to the testing in animals and then humans.[22]

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[18] *See, e.g.*, JP Hughes et al., *Principles of Early Drug Discovery*, 162 Brit. J. Pharmacology 1239 (2011), https://tinyurl.com/5n6b8cyz; Joseph A. DiMasi, *Innovating by Developing New Uses of Already-Approved Drugs: Trends in the Marketing Approval of Supplemental Indications*, 35 Clinical Therapeutics 808, 809 (June 2013), https://tinyurl.com/5yhr7s4w.

[19] DiMasi, *supra* n. 18.

[20] Anjali D. Deshmukh, *Redefining Innovation for Pharmaceutical Regulation*, 104 B.U. L. Rev. 577, 583 (Mar. 2024); *see also* Shanta Afrin & Vikas Gupta, *Pharmaceutical Formulation*, StatPearls (2023), https://tinyurl.com/26r2er25.

[21] Gail A. Van Norman, *Drugs, Devices, and the FDA: Part 1*, 1 JACC: Basic to Translational Science no. 3, 172 (Apr. 2016), https://tinyurl.com/4893zahc.

[22] *Id*. at 172, 175.

Drug development typically takes ten to 15 years and costs over two billion dollars on average.[23] For example, J&J has invested $90 billion in medical innovation through continuous R&D since 2016, including more than $14.6 billion in 2025.[24] Similarly, Lilly invested more than $10 billion for *each* new FDA-approved molecular entity it brought to market from 2006 to 2014.[25] And every year, Lilly re-invests 25% of its revenue into R&D of future medical breakthroughs, including more than $13 billion in 2025 alone and more than $10 billion in 2024. Also in 2025, Sanofi invested more than €7.8 billion in R&D (18.0% of net sales), up from approximately €7.4 billion in 2024. Bristol Myers Squibb Company invested more than $10 billion in R&D in 2025. Bausch Health Companies invested $629 million.

Innovation is complex and expensive, and significant trial and error is involved.[26] There is, after all, no guarantee whatsoever that manufacturers will succeed. And risk must be incentivized, not discouraged. Among other things, manufacturers must balance competing considerations throughout the development process, including whether to trade-off some of the drug's efficacy with its safety (and, if so, how much), while simultaneously accounting for the cost and feasibility of production. Moreover, drugs that secure FDA approval represent only a minute fraction of the therapies developed and put into preclinical and clinical testing. Recall that a mere 0.02% of drugs that go into preclinical testing end up receiving FDA approval for

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[23] BIO, *The U.S. Bioscience Industry: A Power Engine for State Economies* 18 (2025), https://tinyurl.com/4f8t827e; *see also* Duxin Sun et al., *Why 90% of Clinical Drug Development Fails and How to Improve It*, 12 Acta Pharmaceutica Sinica B 7, 3050 (July 2022), https://tinyurl.com/zxj4y28p.

[24] Johnson & Johnson, *U.S. Pricing Transparency Report* 2 (2024), https://tinyurl.com/3p52hs4u.

[25] A. Schuhmacher et al., *Changing R&D Models in Research-Based Pharmaceutical Companies*, 14 J. Transl. Med. 105 (2016), https://tinyurl.com/53rkbh9a.

[26] Pauric Bannigan et al., *Machine Learning Directed Drug Formulation Development*, 175 Advanced Drug Delivery Revs. 12 (2021); *see also* Zeqing Bao et al., *Revolutionizing Drug Formulation Development: The Increasing Impact of Machine Learning*, 202 Advanced Drug Delivery Reviews 2 (2023) ("However, the design and development of advanced pharmaceutical products is a complex process that requires significant time, resources, and expertise. This complexity arises from numerous factors, including the need to consider various parameters related to the drug, excipients, and manufacturing conditions within a high-dimensional design space.").

therapeutic use—and only one in three of that minute percentage will ever recoup its development costs.[27]

New indications and improved products are win-wins for innovators and patients alike.[28] Commercial success means that innovators can recoup the return on their investments, reinvest profits into additional R&D, and celebrate the societal benefits of their discoveries. It also means better and improved lives for patients and, in some cases, the difference between life and death. Indeed, a new indication gives hope to millions of patients suffering from otherwise untreated diseases or conditions, and a new drug composition, presentation, or delivery mechanism can offer more effective and safer medication, as well as a treatment plan that patients are more likely to follow. Put simply: When manufacturers can adequately recoup their investments, innovation and better patient care invariably follow.

B. CMS's guidance disrupts much-needed innovation.

As explained, medications that achieve commercial success after extensive R&D enable the next generation of innovation. By the same token, if a product becomes eligible for price "negotiation" prematurely or even immediately upon approval, a manufacturer is even less likely to recoup its development costs for the product and is accordingly less able to reinvest in future innovations.[29] This is what likely would happen to the hypothetical manufacturer discussed

[27] *See* Vernon, *supra* note 1 at 7; Kraljevic, *supra* note 1 at 837.

[28] *See* Andrew Powaleny, *3 Things to Know About the Importance of Post-Approval Research and Development*, PhRMA (Dec. 6, 2021), https://tinyurl.com/4xhcnube ("Many of these advances that occur following initial FDA approval have resulted in increased survival rates, improved patient outcomes and enhanced quality of life for patients with cancer, autoimmune diseases and rare diseases, among others.").

[29] Allison Hickman, *When Eating the Rich Has Consequences: The Potential Long-Term Effects of the Inflation Reduction Act's Drug Price Negotiation Program*, 11 Emory Corporate Governance and Accountability Review Perspectives 14, 17 (2024), https://tinyurl.com/yxzd7zuh ("The question … is how to conduct necessary drug testing trials when they may not make returns on developmental costs because of the future drastic increase in revenue by the implementation of the DPNP. A potential answer, unfortunately, might be to limit research and development … funding for niche medication.").

above: The manufacturer might not be able to recoup the R&D investments that led to the development of its products. CMS's guidance, in other words, upends the incentives that make pharmaceutical innovations possible.

As discussed above, CMS defines "qualifying single source drug" to include all of the manufacturer's approved products with the same active moiety. This means that new and completely distinct products will become eligible for DPNP's forced-sale regime at the same time as their already approved counterpart. That practical effect of CMS's guidance will lead to reduced investment in subsequent generations of drug development. Innovation is largely possible where manufacturers have sufficient time on the free market—unencumbered by forced sales—to financially justify their expenditures and arrive at a place where they are able to reinvest in new R&D.[30] By depriving innovators of this much-needed time, CMS's guidance will cause fewer drugs to enter the market, denying patients and their caretakers access to innovative products. Rare and untreated conditions will remain just that. And even as to those conditions for which an approved treatment is already available, patients might have no choice but to rely on versions of existing drugs that are hard to administer or to use—resulting in less patient adherence to lifesaving treatment plans.

Consider STELARA®, a Janssen Biotech, Inc. biological medication. FDA initially approved STELARA® to treat psoriasis in 2009 (BLA 125261). Further development of STELARA® resulted in multiple FDA approvals, via supplemental BLAs, for additional indications, including psoriatic arthritis, psoriasis in patients 12 years and older, and psoriasis and psoriatic arthritis in patients six years and older. These versions of STELARA® come in vials or prefilled syringes, allowing patients to receive treatment at home. In 2016, STELARA®

[30] Tomas J. Philipson et al., *The Impact of Price Setting at 9 Years on Small Molecule Innovation Under the Inflation Reduction Act*, U. of Chi., at 7 (Oct. 2023), https://tinyurl.com/y8z79hjc.

received an additional FDA approval—pursuant to a separate BLA (BLA 761044)—to treat Crohn's disease and, three years later, to treat ulcerative colitis. This version of STELARA® is either injected in patients subcutaneously (i.e., through the skin) or administered via an IV. In total, Janssen invested two decades and hundreds of millions of dollars into R&D of STELARA® and conducted more than 100 clinical trials to identify the safest and most effective uses of the drug's active ingredient. In 2024, CMS selected STELARA® for inclusion in the DPNP. Because the later-approved indications of STELARA® share the same active moiety as the original version of the biological medication (despite having a different BLA), they too were included in CMS's selection. That means that STELARA® products approved after 2009 became subject to the DPNP well in advance of their 11-year ineligibility period expiry. For example, the STELARA® product that treats ulcerative colitis (approved in 2019 after substantial investment), became subject to the IRA's forced-sale regime in 2024, rather than 2030.

This kind of aggregation of different treatments is not only unfair but also will have lasting effects on pharmacological innovation and patient access. One study estimates, for example, that the DPNP will "reduce overall annual cancer R&D spending by about $18.1 billion, or 31.8%."[31] In another study, researchers concluded that the IRA's reduction of innovation of small-molecule drugs will result in a loss of 116 million life years due to missed opportunities for health improvement.[32] And these IRA-specific studies did not even account for CMS's guidance and the attempt to sweep more medications prematurely into the DPNP.

[31] Tomas J. Philipson et al., *Policy Brief: The Impact of Recent White House Proposals on Cancer Research*, U. of Chi., at 1 (June 2022), https://tinyurl.com/nufwucj8.

[32] The study concluded that the absence of small molecule innovation resulting from the IRA will result in 188 fewer small molecule treatments, including 79 fewer new small molecule drugs and 109 fewer post-approval indications for these drugs. *See* Philipson, *supra* note 30, at 3.

These impacts are already being felt. One recent study estimated that following the passage of the IRA, the "average monthly number of industry-sponsored trials on post-approval drugs decreased by 38.4%."[33] Another study suggested that there has been an "overall decline in industry-funded post-approval clinical trials in oncology drugs" following the IRA's passage.[34] And there is evidence that the DPNP already has caused manufacturers to "shelve promising new medical treatments."[35] Alnylam Pharmaceuticals announced that it would not start clinical trials for a rare genetic eye disease treatment, "as the company 'continues to evaluate the impact of the [IRA].'"[36] Likewise, Lilly has publicly stated that the IRA caused it to deprioritize three drugs in development, and would have undermined Lilly's research on existing small-molecule drugs if the IRA had come into effect earlier.[37] Novartis and Genentech also have warned that the IRA's DPNP has negatively impacted investment and research into cancer treatments.[38]

This is just the beginning. By expanding the definition of "qualifying single source drug" and bundling different products regardless of their date of FDA approval, CMS's guidance has made things worse for innovators and patients across the country.

CONCLUSION

This Court should grant the Plaintiffs' motion for summary judgment, declare CMS's guidance unlawful, and set the guidance aside.

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[33] Hanke Zheng et al., *The Inflation Reduction Act and Drug Development: Potential Early Signals of Impact on Post-Approval Clinical Trials*, 59 Therapeutic Innovation & Regulatory Science 781, 781 (2025), https://tinyurl.com/mryc2pkr.

[34] Hanke Zheng et al., *Early Impact of the Inflation Reduction Act on Small Molecule vs. Biologic Post-Approval Oncology Trials* at 4, Health Affairs Scholar (2025), https://tinyurl.com/yrpv42ah.

[35] Brad Watts & Katie Mahoney, *Why We're Suing HHS and CMS to Challenge Illegal Price Controls*, U.S. Chamber of Commerce (July 12, 2023), https://tinyurl.com/4nw64v9w.

[36] Jonathan Saltzman, *Alnylam Decides to 'Pause' Drug Trial, Citing New Federal Pricing Law*, Boston Globe (Oct. 27, 2022), https://tinyurl.com/3eucw3e9.

[37] Jessica Merrill, *Lilly Sidelined Three Drugs Due to IRA, CEO Rick Says*, Pink Sheet Citeline Regulatory (June 14, 2023), https://tinyurl.com/3scrudf2.

[38] *See* Watts, *supra* note 35.

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