IN THE UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF COLUMBIA

PHARMACEUTICAL RESEARCH AND MANUFACTURERS OF AMERICA, Plaintiff, v. UNITED STATES DEPARTMENT OF HEALTH AND HUMAN SERVICES 200 Independence Avenue SW Washington, DC 20201, SYLVIA MATHEWS BURWELL, in her official capacity as Secretary of Health and Human Services Office of the Secretary 200 Independence Avenue SW Washington, DC 20201, HEALTH RESOURCES AND SERVICES ADMINISTRATION 5600 Fishers Lane Rockville, MD 20857, and MARY K. WAKEFIELD, in her official capacity as Administrator of the Health Resources and Services Administration 5600 Fishers Lane Rockville, MD 20857, Defendants.	Civil Action No. _____

COMPLAINT FOR DECLARATORY AND INJUNCTIVE RELIEF

INTRODUCTION

1. Plaintiff Pharmaceutical Research and Manufacturers of America ("PhRMA") seeks declaratory and injunctive relief to enjoin a second attempt by the U.S. Department of Health and Human Services ("HHS") to re-issue a flawed rule construing and implementing 42 U.S.C. § 256b(e). The statutory provision at issue exempts "orphan drugs" from a drug price-control program called the 340B Program. In May 2014, this Court vacated HHS's first rulemaking purporting to construe § 256b(e), holding that HHS had "acted beyond the bounds of its statutory authority" because Congress did "not confer any rulemaking authority upon HHS" to regulate the scope of the orphan drug exclusion. *PhRMA v. HHS*, No. 13-1501, 2014 WL 2171089, at *8 (D.D.C. May 23, 2014) (quotation marks omitted). HHS's second attempted rulemaking contains the same substance as the first rule, adopts the same flawed interpretation of § 256b(e), and should similarly be invalidated as inconsistent with the statute.

2. The statutory orphan drug exclusion exempts from 340B price controls "a drug designated by the Secretary [of HHS] . . . for a rare disease or condition"—referred to as an "orphan-designated drug" or "orphan drug." 42 U.S.C. § 256b(e). In July 2013, following formal notice-and-comment rulemaking procedures, HHS issued a regulation purporting to "interpret" this statutory orphan drug exclusion. *See* 78 Fed. Reg. 44016 (July 23, 2013) ("Final Rule" or "Vacated Final Rule"). But in reality, the regulation narrowed and rewrote § 256b(e) in violation of Congress's clearly expressed intent. Contrary to the statute's plain language, the Final Rule exempted from 340B pricing only those orphan-designated drugs that are "*used* for the rare condition or disease for which that orphan drug was designated." *Id.* at 44027 (emphasis added). The Final Rule also required healthcare providers participating in the 340B Program—referred to as "covered entities"—to "maintain and provide auditable records" demonstrating

their compliance with the regulation. *Id.* at 44028. HHS concluded in the Final Rule that a "regulation is necessary to implement these changes" to the 340B Program. *Id.* at 44017.

3. This Court vacated the Final Rule on the ground that HHS lacks authority to issue legislative rules construing § 256b(e). *PhRMA*, 2014 WL 2171089, at *12. The Court also reasoned that it was "inclined to think" that the Final Rule could not survive as an interpretive rule either, explaining that the rule "*has a 'legal effect'* on the parties so regulated *because the interpretation* of 'covered outpatient drug,' as well as the compliance procedures impose obligations on covered entities and manufacturers alike." *Id.* at *13 (emphasis added). The Court invited HHS to further defend its Final Rule as an interpretive rule, but HHS declined.

4. Two months after this Court's decision, HHS promulgated a new rule setting forth in substance the same statutory interpretation as the Vacated Final Rule. *See* HHS, Interpretive Rule: Implementation of the Exclusion of Orphan Drugs for Certain Covered Entities Under the 340B Program (July 23, 2014), *available at* http://www.hrsa.gov/opa/programrequirements/interpretiverule/interpretiverule.pdf ("July 2014 Rule") (Ex. A); *see also* 79 Fed. Reg. 42801 (July 23, 2014) (announcing publication of the July 2014 Rule). The new rule reiterates verbatim the Vacated Final Rule's use-based limit on the scope of § 256b(e) and imposes the same tracking requirements on covered entities. *See* July 2014 Rule at 6.

5. The July 2014 Rule violates the plain language of the statutory orphan drug exclusion. The statutory text as written by Congress provides:

> EXCLUSION OF ORPHAN DRUGS FOR CERTAIN COVERED ENTITIES—For covered entities described in subparagraph (M) (other than a children's hospital described in subparagraph (M)), (N), or (O) of [42 U.S.C. § 256b(a)(4)], the term "covered outpatient drug" shall not include *a drug designated by the Secretary under section 526 of the Federal Food, Drug, and Cosmetic Act for a rare disease or condition*.

42 U.S.C. § 256b(e) (emphasis added). This specific, clear language is self-executing and requires no interpretation or implementation. If the Secretary designates "a drug" as an orphan drug, the drug falls within the exclusion when sold to a newly covered entity; how the drug is *used* is irrelevant.

6. The July 2014 Rule alters the scope of the orphan drug exclusion by grafting a use-based limit onto the language that Congress enacted. The statutory text as rewritten by HHS would provide:

> EXCLUSION OF ORPHAN DRUGS FOR CERTAIN COVERED ENTITIES—For covered entities described in subparagraph (M) (other than a children's hospital described in subparagraph (M)), (N), or (O) of [42 U.S.C. § 256b(a)(4)], the term "covered outpatient drug" shall not include a drug designated by the Secretary under section 526 of the Federal Food, Drug, and Cosmetic Act for a rare disease or condition *and used to treat such rare disease or condition*.

(emphasized words added). Adding this use-based requirement to the statutory text substantively alters the plain language Congress enacted into law and creates the same "legal effects" this Court identified in its prior decision invalidating the Final Rule. *See PhRMA*, 2014 WL 2171089, at *13. HHS's interpretation and the legal effects it creates are wholly inconsistent with the statutory text.

7. HHS has asserted that it will enforce the interpretation of § 256b(e) set out in the July 2014 Rule, which could include ordering manufacturers to issue refunds to covered entities or bringing enforcement actions for failing to comply with the agency's interpretation. Any such enforcement actions would be resolved in the first instance in HHS's internal dispute-resolution process, where the agency would be the judge and jury, and where manufacturers could face substantial monetary and other penalties. Thus, PhRMA's members must either: (a) accept the consequences of the July 2014 Rule, which are inconsistent with the statute; or (b) reject HHS's

interpretation, risk incurring significant penalties, and then challenge HHS's decision in an action under the Administrative Procedure Act only after the agency imposes those penalties.

8. PhRMA brings this action to stop HHS from imposing its flawed orphan drug interpretation despite what Congress wrote in § 256b(e), despite what this Court held in its decision vacating the Final Rule, and despite the agency's lack of legislative authority to regulate the scope of the statutory orphan drug exclusion. This Court should invalidate the July 2014 Rule as final agency action that is "arbitrary, capricious, an abuse of discretion, or otherwise not in accordance with law." 5 U.S.C. § 706(2)(A).

JURISDICTION AND VENUE

9. PhRMA brings this action under the Administrative Procedure Act, 5 U.S.C. §§ 551 *et seq*.

10. The July 2014 Rule is "final agency action for which there is no other adequate remedy." 5 U.S.C. § 704.

11. This Court has subject-matter jurisdiction pursuant to 28 U.S.C. § 1331 because PhRMA's causes of action arise under the laws of the United States.

12. Venue is proper in this district under 28 U.S.C. § 1391(e) because Defendant HHS resides in this judicial district, Defendant Sylvia Mathews Burwell performs her official duties as Secretary of HHS in this judicial district, a substantial part of the events giving rise to this action occurred in this judicial district, and Plaintiff PhRMA resides in this judicial district.

13. An actual controversy exists within the meaning of 28 U.S.C. § 2201.

14. This Court has the authority to grant declaratory and injunctive relief pursuant to 28 U.S.C. §§ 2201, 2202 and 5 U.S.C. §§ 705, 706.

15. This Complaint is timely filed under 28 U.S.C. § 2401(a).

PARTIES

16. Plaintiff PhRMA is a voluntary, nonprofit association representing the nation's leading research-based pharmaceutical and biotechnology companies. PhRMA's mission is to advocate public policies that encourage the discovery of life-saving and life-enhancing medicines. PhRMA serves as the pharmaceutical industry's principal policy advocate. It represents and protects its members' interests in matters before Congress, the Executive Branch, state agencies and legislatures, and the courts. PhRMA's members account for approximately 70% of the sales of prescription drugs in the United States and a significant portion of the orphan drugs sold in the United States. A list of PhRMA's members is available at http://www.phrma.org/about/member-companies.

17. PhRMA's members are dedicated to discovering medicines that help patients lead longer, healthier, and more productive lives. In 2013 alone, PhRMA's members invested an estimated $51.1 billion in efforts to discover and develop new medicines, including orphan drugs. In bringing this lawsuit, PhRMA seeks to vindicate the rights and interests of its members by removing an unlawful regulatory roadblock to the development and sale of orphan drugs.

18. Defendant HHS is an executive department in the U.S. Government. HHS is headquartered in Washington, DC.

19. Defendant Health Resources and Services Administration ("HRSA") is an administrative agency within HHS and is responsible for administering the 340B Program. HRSA is headquartered in Rockville, MD.

20. Defendant Sylvia Mathews Burwell is the Secretary of HHS. Her official address is in Washington, DC. She is being sued in her official capacity. In that capacity, Secretary

Burwell is responsible for overseeing the activities of HRSA, including its administration of the 340B Program.

21. Defendant Mary K. Wakefield is the Administrator of HRSA. Her official address is in Rockville, MD. She is being sued in her official capacity. In that capacity, Administrator Wakefield is directly responsible for administering the 340B Program.

BACKGROUND

The 340B Program

22. Congress established the 340B Program to help certain patients of specified categories of healthcare providers (referred to in the law as "covered entities") gain better access to outpatient prescription drugs. Under this price-control program, manufacturers are required, as a condition of Medicaid covering their products, to enter into a Pharmaceutical Pricing Agreement with the Secretary of HHS (the "Secretary"). 42 U.S.C. § 256b(a); *Astra USA, Inc. v. Santa Clara Cnty.*, 131 S. Ct. 1342, 1345 (2011). The Pharmaceutical Pricing Agreement obligates a manufacturer to charge 340B covered entities no more than a statutorily defined "ceiling price" on "covered outpatient drugs." 42 U.S.C. § 256b(a)(1). PhRMA supports the 340B Program and its purpose of providing uninsured patients better access to medicines.

23. The 340B ceiling price can be up to 50% lower than what other purchasers would pay for the same product. U.S. Gov't Accountability Office, GAO-11-836, *Drug Pricing: Manufacturer Discounts in the 340B Program Offer Benefits, but Federal Oversight Needs Improvement* 2 (2011) ("GAO 340B Report").

24. The number of covered entities participating in the 340B Program has grown exponentially in recent years, increasing from 8,605 sites in 2001, *id.* at 8 fig.1, to 26,848 sites in August 2014, HRSA, Covered Entity Database, opanet.hrsa.gov/opa/CESearch.aspx (select "All"

for "Entity Type;" then click "Search") (last visited Oct. 7, 2014). *See also* Pedley Decl. at ¶ 10, *PhRMA v. HHS*, No. 13-1501 (D.D.C. Dec. 13, 2013), ECF No. 24-3 (noting that there were 23,740 covered entity sites as of October 2013).

25. This substantial growth in the number of covered entities is due in part to the Affordable Care Act's expansion of the types of healthcare providers that are eligible to participate in the 340B Program. Only "disproportionate share hospitals" providing inpatient services to a specified percentage of low-income patients were originally eligible to participate. The Affordable Care Act expanded eligibility to new categories of entities, including certain children's hospitals, free-standing cancer hospitals, critical access hospitals, rural referral centers, and sole community hospitals. Pub. L. No. 111-148, § 7101 (2010) (codified at 42 U.S.C. § 256b(a)(4)(M)-(O)). Today, nearly one out of every three hospitals in the United States participates in the 340B Program. GAO 340B Report at 20.

Orphan Drugs and the Orphan Drug Exclusion

26. Orphan drugs are products designated by the Secretary of HHS for their potential to treat a rare disease or condition, which generally means a disease or condition affecting fewer than 200,000 people in the United States. 21 U.S.C. § 360bb(a)(2). Approximately 7,000 diseases meet this definition.

27. Recognizing the need to develop treatments for these rare conditions, Congress has created various research and development incentives that attach whenever a drug receives an orphan designation. *See* Orphan Drug Act, Pub. L. No. 97-414, § 1(b)(6) ("[I]t is in the public interest to provide . . . incentives for the development of orphan drugs."). For example, an orphan designation triggers access to seven years of market exclusivity, 21 U.S.C. § 360cc(a),

exemption from certain drug application fees, *id.* § 379h(a)(1)(F), research grants for clinical testing, *id.* § 360ee, and a clinical trial tax credit, 26 U.S.C. § 45C.

28. Separate from this orphan drug *designation*, a manufacturer also may obtain *approval* to market the orphan-designated product as a treatment for a particular rare disease or condition. *See* 21 U.S.C. § 360cc. As HHS explained in the Vacated Final Rule, "[t]he award of an orphan designation does not alter the standard regulatory requirements and process for obtaining marketing approval, which is a separate process." 78 Fed. Reg. at 44017. A drug that has obtained an orphan drug designation later may be approved for use in an orphan indication, may be approved for use in a non-orphan indication, or may not be approved at all. *See id.*; *see also* 21 C.F.R. §§ 316.23, 316.24; Pedley Decl. at ¶¶ 5-6, *PhRMA v. HHS*, No. 13-1501 (D.D.C. Dec. 13, 2013), ECF No. 24-3.

29. At the same time that Congress added the new categories of 340B-eligible entities in the Affordable Care Act, it also amended the 340B statute to exempt from the price controls orphan drugs sold to the newly eligible covered entities. Pub. L. No. 111-152, § 2302 (2010) (codified at 42 U.S.C. § 256b(e)). This orphan drug exclusion provides that, with respect to the newly eligible entities, a "covered outpatient drug" available for 340B pricing "shall not include *a drug designated by the Secretary* under section 526 of the Federal Food, Drug, and Cosmetic Act [("FFDCA")] for a rare disease or condition." 42 U.S.C. § 256b(e) (emphasis added). Section 526 of the FFDCA in turn authorizes the Secretary to designate "a drug" as "a drug for a rare disease or condition" if (1) "the drug" is being or will be investigated for a rare disease or condition; and (2) the marketing approval for "the drug," if granted, would be for treating such rare disease or condition. 21 U.S.C. § 360bb(a)(1).

30. As the U.S. Food and Drug Administration ("FDA") has explained, the orphan drug *designation* applies to the drug itself—that is, to the drug's specific "active moiety" or molecule. FDA, Developing Products for Rare Diseases & Conditions, Frequently Asked Questions (FAQ), *available at* http://www.fda.gov/ForIndustry/DevelopingProductsforRare DiseasesConditions/HowtoapplyforOrphanProductDesignation/ucm240819.htm (emphasis omitted) (last visited Oct. 7, 2014) (Ex. B). Thus, the *designation* that triggers exclusion under § 256b(e) is not limited to only orphan *uses* of the orphan-designated drug, as HHS contends. If the drug has an orphan designation, newly eligible entities are not entitled to purchase it at 340B prices regardless of how the drug is used, and must instead purchase the drug at the same price as all other, non-covered entity buyers. But if the drug does not have an orphan designation, newly eligible entities may purchase the drug at 340B prices regardless of how the drug is used. In short, how a drug is used has nothing to do with whether it qualifies for 340B pricing.

The Vacated Final Rule

31. In July 2013, HHS issued the Vacated Final Rule and replaced the *designation*-based exclusion of § 256b(e) with a *use*-based one. The rule rewrote § 256b(e) to apply only when a drug: (a) was designated by the Secretary as an orphan drug; (b) was sold to one of the newly covered entities added by the Affordable Care Act; and (c) then was "*used* for the rare condition or disease for which that orphan drug was designated under section 526 of the FFDCA." 78 Fed. Reg. at 44027 (emphasis added). The Final Rule also required covered entities "to maintain and provide auditable records" demonstrating their compliance with the regulation, and warned that "[f]ailure to comply" with the rule would "be considered a violation" of the 340B statute. *Id.* at 44028. The Office of Management and Budget designated the rule a "significant action" under section 3(f) of Executive Order 12866. *Id.* at 44025.

32. HHS promulgated the Final Rule through formal notice-and-comment rulemaking procedures after concluding that "a regulation is necessary to implement the[] changes" required by the agency's "interpretation" of § 256b(e). *Id.* at 44017.

33. Before the Final Rule became effective, PhRMA filed a complaint in this Court "seek[ing] declaratory and injunctive relief" to invalidate the rule and enjoin HHS from enforcing it. PhRMA, Compl. for Declaratory & Injunctive Relief ¶ 1, *PhRMA v. HHS*, No. 13-1501 (D.D.C. Sept. 27, 2013), ECF No. 1. PhRMA explained that the Final Rule was *ultra vires* because, among other things, "Congress did not empower HHS or HRSA to promulgate rules interpreting the orphan drug exclusion." *Id.* at ¶ 7. PhRMA also maintained that the use-based limit of the Final Rule was inconsistent with the plain language of the statute. *Id.* at ¶ 6.

34. In briefing on cross motions for summary judgment, HHS argued that Congress gave it authority to issue legislative rules interpreting § 256b(e). *See* HHS, Mem. in Supp. of Defs.' Mot. to Dismiss or Summ. J. 15-21, *PhRMA v. HHS*, No. 13-1501 (D.D.C. Dec. 13, 2013), ECF No. 24-1. The agency also contended that, if the Court were to conclude that HHS lacked statutory authority to promulgate a legislative rule regarding the orphan drug exclusion, the Final Rule should still be upheld "as an interpretive rule." *Id.* at 21.

35. In May 2014, this Court issued a Memorandum Opinion vacating the Final Rule. The Court held that Congress did "not confer upon HHS authority to issue the rule." *PhRMA*, 2014 WL 2171089, at *9. The Court also recognized that HHS had "ask[ed] this Court to uphold the rule as an interpretive, as opposed to a legislative, rule." *Id.* at *13. The Court indicated that it was "inclined to think" that the Final Rule could not survive as an interpretive rule, reasoning that the rule "*has a* '*legal effect*' on the parties so regulated because *the interpretation* of 'covered outpatient drug,' as well as the compliance procedures impose obligations on covered

entities and manufacturers alike." *Id.* (emphasis added). Nonetheless, the Court invited the agency to submit "more briefing" on the issue. *Id.* at *14.

36. HHS "decline[d] the Court's invitation to submit further briefing defending the challenged regulation as an interpretive rule," choosing instead to rest on the agency's prior briefing of the issue. HHS, Defs.' Resp. to Ct.'s May 23, 2014, Order 1, *PhRMA v. HHS*, No. 13-1501 (D.D.C. June 12, 2014), ECF No. 45.

37. The same day that HHS declined the Court's invitation, the agency announced on its website that it "continues to stand by the interpretation described in its published final rule." HHS, Orphan Drugs Exclusion, http://www.hrsa.gov/opa/programrequirements/orphandrugexclusion/index.html (last visited Oct. 7, 2014) (Ex. C). HHS's website posting also declared that the agency would "post updated" information "in order to assist all 340B stakeholders in complying with [HHS's] policy," as set out in the Vacated Final Rule. *Id.* And HHS warned that "340B hospitals subject to the orphan drug exclusion . . . are responsible for ensuring that any orphan drugs purchased through the 340B Program are not transferred, prescribed, sold, or otherwise used for the rare condition or disease for which the orphan drugs are designated under section 526 of the [FFDCA]." *Id.*

38. Immediately following HHS's website posting, PhRMA asked this Court either to require further briefing or to enter a judgment confirming that "the Final Rule is incapable of surviving as an interpretive rule." PhRMA, Reply to Defs.' Resp. to Ct.'s May 23, 2014, Order & Mot. for Misc. Relief 6, *PhRMA v. HHS*, No. 13-1501 (D.D.C. June 18, 2014), ECF No. 46-1. HHS opposed PhRMA's motion, asserting that the agency "is intending to issue a further interpretive rule" that "sets forth the same interpretation previously embodied in" the Vacated

Final Rule. HHS, Defs.' Opp'n to Pl.'s Mot. for Misc. Relief 2-3, *PhRMA v. HHS*, No. 13-1501 (D.D.C. July 14, 2014), ECF No. 50.

The July 2014 Rule

39. HHS promulgated its new "interpretive rule" on July 23, 2014, adopting the same interpretation of the orphan drug exclusion as set out in the Vacated Final Rule. The July 2014 Rule is entitled: "Interpretive Rule: *Implementation* of the Exclusion of Orphan Drugs for Certain Covered Entities Under the 340B Program." (emphasis added).

40. The July 2014 Rule reiterates verbatim the agency's use-based limit on § 256b(e), stating that the orphan drug exclusion is limited to orphan drugs that are "used for the rare condition or disease for which the drug was designated under section 526 of the Federal Food, Drug, and Cosmetic Act (FFDCA)." July 2014 Rule at 6. The July 2014 Rule also adopts the Final Rule's tracking requirements for covered entities: "If a covered entity lacks the ability to track drug use by indication, such entity would be unable to purchase drugs with orphan designations through the 340B Program. Updated on a quarterly basis, HRSA will maintain a list of covered entities that cannot or do not wish to purchase such drugs through the 340B Program." *Id.*

41. An HHS official, the Director of HRSA's Office of Pharmacy Affairs, attested in an affidavit to this Court that any manufacturer refusing to obey the agency's "interpretation" set out in the July 2014 Rule could face sanctions for noncompliance: "a manufacturer's or covered entity's failure to abide by [HHS's] interpretation of the statute could subject a manufacturer or covered entity to an enforcement action by [HHS]." Pedley Decl. ¶ 6, *PhRMA v. HHS*, No. 13-1501 (D.D.C. July 24, 2014), ECF No. 53-1.

42. In response to a question about whether the July 2014 Rule is "binding on manufacturers and covered entities," HHS's official website similarly provides that: "A manufacturer's or covered entity's failure to comply with the statutory requirements could subject a manufacturer or covered entity to an enforcement action by [HHS], which could include refunds to covered entities in the case of overcharges, as well as termination of a manufacturer's Pharmaceutical Pricing Agreement. . . . [HHS] has provided its interpretation of the statute to stakeholders in the 340B community by issuing the [July 2014] Rule." HHS, FAQs, http://www.hrsa.gov/opa/faqs/index.html (last visited Oct. 7, 2014) (follow link to "Orphan Drugs") (Ex. D).

43. On August 27, 2014, this Court issued an order entering final judgment in PhRMA's lawsuit challenging the validity of the Vacated Final Rule. *See* Order, *PhRMA v. HHS*, No. 13-1501 (D.D.C. Aug. 27, 2014), ECF No. 55. The Order reiterated that "HHS lacked the substantive rulemaking authority to implement the rule in the first instance." *Id.* at 1. The Court, however, declined to address the validity of the agency's underlying interpretation of § 256b(e). The Court noted that PhRMA "is free to challenge th[e] interpretive rule" in a new lawsuit, "but such a challenge is beyond the scope of the instant action." *Id.* at 4.

44. The July 2014 Rule requires PhRMA's members either to change their conduct and comply with the "interpretation" set out in the rule (but not in the statute) or risk substantial penalties. HHS, for example, could order manufacturers to issue refunds to covered entities or possibly even terminate the manufacturers' Pharmaceutical Pricing Agreement for failing to comply with the agency's construction of § 256b(e). HHS also could pursue other remedies against manufacturers in a mandatory administrative dispute resolution proceeding that HHS is required to create for the 340B Program. *See* 42 U.S.C. § 256b(d)(3). HHS will "establish and

implement" the rules and procedures of such a proceeding, will designate an agency official or committee of officials to resolve any disputes, and will issue a "final agency decision" that is "binding upon the parties involved." *Id.* The agency therefore will be the judge and jury of the proceeding, forcing PhRMA's members to face severe penalties in order to mount a future challenge to the construction and implementation of § 256b(e) set out in the July 2014 Rule.

CLAIM FOR RELIEF

COUNT I

(Violation Of The Administrative Procedure Act, 5 U.S.C. § 706(2)(A): The July 2014 Rule And Its Construction Of § 256b(e) Are Arbitrary, Capricious, An Abuse Of Discretion, Or Otherwise Not In Accordance With Law)

45. PhRMA re-alleges and incorporates paragraphs 1 through 44.

46. Section 256b(e) exempts from 340B pricing any drug sold to a newly covered entity and designated as an orphan drug. By its text, this orphan drug exclusion applies to the orphan-designated drug itself, not a particular use of the drug. The July 2014 Rule impermissibly rewrites the statutory text by adding a use-based restriction that Congress could have—but did not—include. That change fundamentally and substantively narrows the meaning of § 256b(e), to the detriment of PhRMA's members.

47. If Congress had intended to impose a use-based limitation on the orphan drug exclusion, it easily could have done so, just as it has done in other statutory orphan drug exemptions. *See, e.g.*, 42 U.S.C. § 1395l(t)(6) (permitting an additional "pass-through" payment for a drug or biological product designated as an orphan drug only when "*used for* a rare disease or condition" (emphasis added)); 26 U.S.C. § 45C(b)(2)(A) (providing a tax credit for clinical testing "carried out under an exemption for a drug *being tested for* a rare disease or condition" (emphasis added)); 21 U.S.C. § 379h(a)(1)(F) (exempting from the drug application user fee "a prescription drug product that has been designated as a drug for a rare disease or condition . . .

unless the human drug application includes an indication for other than a rare disease or condition" (emphasis added)).

48. HHS's rewrite of the statutory orphan drug exclusion forces PhRMA's members to choose between complying with the *designation*-based language Congress enacted into law or complying with the contradictory *use*-based language HHS added to the statute. Members that choose to follow the statutory text, as opposed to the July 2014 Rule, risk substantial losses and penalties, including an agency order directing them to issue refunds to covered entities, an enforcement action for civil monetary penalties brought within a dispute resolution proceeding overseen and decided by the very agency that issued the July 2014 Rule, and possibly even the termination of their Pharmaceutical Pricing Agreement.

49. The same fatal flaws would exist in any future agency pronouncement, however classified or labeled, that adopts the same construction of § 256b(e) as the July 2014 Rule. HHS cannot use interpretive rules, policy statements, frequently asked questions, administrative dispute resolution decisions, or any other type of agency action to add a use-based limit to the scope of the orphan drug exclusion. Any final agency action restricting the scope of that statutory provision, no matter how HHS classifies its action, would be inconsistent with the plain language of the statute and impermissibly create new legal effects on regulated parties that Congress did not enact into law.

50. For these and other reasons, the July 2014 Rule is arbitrary, capricious, and not in accordance with law, and should be vacated pursuant to 5 U.S.C. § 706(2)(A).

PRAYER FOR RELIEF

PhRMA asks this Court to:

1. Declare that Defendants violated the Administrative Procedure Act by taking arbitrary and capricious final agency action that rewrites the orphan drug exclusion in the 340B statute to apply only when an orphan drug is used for an orphan indication;

2. Declare that the July 2014 Rule and any future agency pronouncements, policies, rulings, rules, adjudications, or other action, regardless of how characterized, adopting the same interpretation of § 256b(e) as set out in the Vacated Final Rule and the July 2014 Rule would similarly violate the Administrative Procedure Act because they too would be arbitrary, capricious, and not in accordance with law;

3. Grant an order and judgment invalidating and permanently enjoining Defendants from implementing or enforcing the July 2014 Rule;

4. Award PhRMA costs and reasonable attorneys' fees, as appropriate; and

5. Grant any other relief the Court deems just and appropriate.

Respectfully submitted,

/s/ Jeffrey L. Handwerker

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