UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF COLUMBIA

PHARMACEUTICAL RESEARCH AND MANUFACTURERS OF AMERICA 950 F Street, NW Suite 300 Washington, DC 20004, Plaintiff, v. UNITED STATES DEPARTMENT OF HEALTH AND HUMAN SERVICES 200 Independence Avenue SW Washington, DC 20201, KATHLEEN SEBELIUS, 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 for the Health Resources and Services Administration 5600 Fishers Lane Rockville, MD 20857, Defendants.	Civil Action No. _____ **COMPLAINT FOR DECLARATORY AND INJUNCTIVE RELIEF**

COMPLAINT FOR DECLARATORY AND INJUNCTIVE RELIEF

Plaintiff Pharmaceutical Research and Manufacturers of America ("PhRMA") alleges as follows:

INTRODUCTION

1. In this action, PhRMA seeks declaratory and injunctive relief to block implementation of a final rule promulgated by the U.S. Department of Health and Human Services ("HHS") on July 23, 2013, in connection with the 340B Program administered by the Health Resources and Services Administration ("HRSA"), an operating division of HHS. *See* Exclusion of Orphan Drugs for Certain Covered Entities Under 340B Program, 78 Fed. Reg. 44016 (July 23, 2013) ("Final Rule"). The Final Rule purports to implement Section 340B(e) of the Public Health Service Act ("PHSA"), which excludes from the price controls imposed by the 340B Program orphan drugs sold to certain entities. The Final Rule violates the Administrative Procedure Act, 5 U.S.C. §§ 701 *et seq.*, because it is based on an erroneous reading of the statutory text that HRSA is seeking to implement and is outside the scope of HHS's rulemaking power. Thus, the Final Rule is "arbitrary, capricious, an abuse of discretion, or otherwise not in accordance with law" and "in excess of statutory jurisdiction, authority, or limitations, or short of statutory right." 5 U.S.C. §§ 706(2)(A), (C).

2. The 340B Program imposes price controls on "covered outpatient drugs" that are purchased by specified categories of health clinics and hospitals, referred to as "covered entities." 42 U.S.C. § 256b(a)(1). Pharmaceutical manufacturers may not charge more than a statutorily defined "ceiling price" for outpatient drugs sold to these covered entities as a condition of Medicaid reimbursement. PhRMA supports the 340B Program and its purpose of providing vulnerable uninsured patients with better access to life-saving medicines.

3. In the Patient Protection and Affordable Care Act of 2010, Pub. L. No. 111-148, as amended by the Health Care and Education Reconciliation Act of 2010, Pub. L. No. 111-152 (together, the "Affordable Care Act"), Congress expanded the categories of covered entities that

are entitled to the 340B ceiling price. *See* Affordable Care Act § 2302 (codified at 42 U.S.C. § 256b(a)(4)(M)-(O)). At the same time, however, Congress also determined that drug manufacturers would not be required to extend 340B pricing on "orphan drugs" sold to these new categories of covered entities.

4. Specifically, Congress added a new Section 340B(e) to the PHSA (the "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.

Affordable Care Act § 2302, as amended by the Medicare and Medicaid Extenders Act of 2010 (codified as amended at 42 U.S.C. § 256b(e)) (emphasis added). Section 526 of the Federal Food, Drug and Cosmetic Act ("FFDCA"), in turn, establishes procedures for identifying products as "orphan drugs." Orphan drugs are products designated by the Food and Drug Administration ("FDA") 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).

5. The statutory text of Section 340B(e) thus makes clear that the 340B orphan drug exclusion applies to any "drug" designated as an "orphan" drug pursuant to Section 526 of the FFDCA. Contrary to the statutory command in Section 340B(e), however, the Final Rule asserts that an orphan drug is exempt from 340B pricing requirements ***only*** when "***used*** for the rare condition or disease for which that orphan drug was designated." 78 Fed. Reg. at 44027 (to be

codified at 42 C.F.R. § 10.21(a)) (emphasis added). The text as rewritten in the Final Rule would read:

> 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 ***and used to treat*** ~~for~~ a rare disease or condition.

(emphasis and strikethrough added).

6. HHS's revision of the orphan drug exclusion through rulemaking is at odds with the plain statutory text. The statutory language makes clear that Congress intended the orphan drug exclusion to apply to any orphan drug sold to one of the newly covered entities, regardless of whether the covered entity uses the drug for an orphan indication. The Final Rule thus violates the Administrative Procedure Act because it is not in accordance with law.

7. Moreover, HHS lacked authority to issue the Final Rule. Congress did not empower HHS or HRSA to promulgate rules interpreting the orphan drug exclusion. No federal statute — including the 340B statute as amended by the Affordable Care Act — comes even remotely close to authorizing the agency to issue rules related to Section 340B(e). HHS thus acted *ultra vires* in promulgating the Final Rule, in further violation of the Administrative Procedure Act.

8. For these reasons, the Final Rule exceeds HHS's statutory authority. PhRMA asks this Court to declare that the Final Rule violates the Administrative Procedure Act, and to preliminarily and permanently enjoin HHS and HRSA from enforcing the Final Rule.

JURISDICTION

9. PhRMA brings this action under the Administrative Procedure Act. This Court has subject-matter jurisdiction pursuant to 28 U.S.C. § 1331 because PhRMA's causes of action all arise under the laws of the United States.

10. Venue is proper in this district under 28 U.S.C. § 1391(e)(1) because Defendant HHS resides in this judicial district, Defendant Secretary Sebelius performs her official duties 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.

11. This Court may grant declaratory and injunctive relief pursuant to 5 U.S.C. § 706 and 28 U.S.C. §§ 2201 and 2202.

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

PARTIES

13. 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.

14. PhRMA's members are dedicated to discovering medicines that help patients lead longer, healthier, and more productive lives. In 2012 alone, PhRMA's members invested an estimated $48.5 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.

15. Defendant HHS is an executive department in the United States Government. HHS is headquartered in Washington, D.C. Defendant HRSA is an administrative agency within HHS and is responsible for administering the 340B Program. HRSA is headquartered in Rockville, MD.

16. Defendant Kathleen Sebelius is the Secretary of HHS. Her official address is in Washington, D.C. She is being sued in her official capacity. In that capacity, Secretary Sebelius has responsibility for oversight of the activities of HRSA, including its administration of the 340B Program.

17. 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 the administration of the 340B program.

BACKGROUND

The 340B Program

18. Congress established the 340B Program pursuant to Section 602 of the Veterans Health Care Act of 1992, Pub. L. No. 102-585, 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, 1345-46 (2011).

19. The Pharmaceutical Pricing Agreement obligates a manufacturer to offer 340B "covered entities" statutorily defined ceiling prices on "covered outpatient drugs." 42 U.S.C. § 256b(a). The price at which a manufacturer is required to make its outpatient drugs available to covered entities is referred to as the 340B "ceiling price." *Id.* The 340B ceiling price is calculated as the difference between the manufacturer's Average Manufacturer Price and its per unit Medicaid rebate amount, as determined pursuant to the Medicaid Rebate Statute, Section 1927 of the Social Security Act, 42 U.S.C. § 1396r-8.

20. Manufacturers generally do not sell drugs to 340B entities directly; instead, manufacturers sell their drugs to wholesalers that resell them to 340B entities at the 340B ceiling price. The 340B entities receive the ceiling price as an upfront discount on the manufacturer's drug at the time of sale (and not, for example, as a rebate). The difference between the 340B price and the price at which the wholesaler purchased the drug is refunded by the manufacturer to the wholesaler through a process known as a chargeback.

21. The 340B ceiling price can be up to 50% lower than what other purchasers would pay for the same product. 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"). However, 340B entities are not required to pass these lower prices on to their patients.

22. The number of covered entities participating in the 340B Program has grown exponentially in recent years, from 8,605 sites in 2001 to 16,572 sites in 2011. *Id.* at 8 fig.1. This substantial growth is due in part to the Affordable Care Act's expansion of the types of covered entities that are eligible. The only hospitals originally eligible to participate in the 340B Program were disproportionate share hospitals serving a specified percentage of low-income

patients. The Affordable Care Act amended the statute to add the following new categories of 340B covered entities: (a) children's hospitals, (b) free-standing cancer hospitals, (c) critical access hospitals, (d) rural referral centers, and (e) sole community hospitals. Affordable Care Act § 2302 (codified at 42 U.S.C. § 256b(a)(4)(M)-(O)). Nearly one out of every three hospitals in the United States now participates in the 340B Program. GAO 340B Report at 20.

The Orphan Drug Exemption

23. Orphan drugs are products designated by the Secretary 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). The National Institutes of Health, Office of Rare Diseases Research, has identified approximately 7,000 rare diseases meeting this definition. According to the FDA's Office of Orphan Products Development, moreover, the Orphan Drug Act that contains incentives for orphan drug research has driven the development of more than 400 drugs and biologic products for rare diseases since 1983. Despite these gains, no treatment is available today for the vast majority of rare diseases or conditions, and more treatments are desperately needed.

24. Recognizing the need for these important treatments, Congress has long crafted special protections for orphan drugs, such as seven years of market exclusivity, *id.* § 360cc(a), exemption from the FDA new drug application user fee, *id.* § 379h(a)(1)(F), federal research grants for clinical testing, *id.* § 360ee, and a clinical trial tax credit, 26 U.S.C. § 45C. These protections create incentives for investment and innovation in products with smaller patient populations.

25. At the same time that Congress added the new categories of 340B covered entities through the Affordable Care Act, it amended the 340B statute to exempt from the mandated

340B ceiling price orphan drugs sold to the new categories of covered entities. Affordable Care Act § 2302 (codified at 42 U.S.C. § 256b(e)). The Medicare and Medicaid Extenders Act of 2010, Pub. L. No. 111-309, later removed the orphan drug exclusion for the new category of children's hospitals, because a similar category of children's hospitals had been eligible for 340B pricing before the Affordable Care Act.

26. The statutory orphan drug exclusion provides that, with respect to the newly eligible covered entities, a "covered outpatient drug" eligible for 340B pricing "shall not include ***a drug designated by the Secretary*** under [section 526 of the 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 "the drug" could be used to treat "such disease or condition." 21 U.S.C. § 360bb(a).

27. The Secretary may approve a drug to treat multiple different indications, only one or a subset of which must qualify as a "rare disease or condition." The Secretary lacks authority to designate a particular indication of a drug as "orphan," and instead must designate the product itself as an "orphan drug." The drug, therefore, is designated as an "orphan drug" in all of its uses when established as such in connection with ***any one*** indication to treat a rare disease or condition.

The Final Rule

28. Neither the PHSA nor any other statute authorizes HHS (or, by delegation, HRSA) to promulgate regulations related to the orphan drug exclusion in Section 340B(e) of the PHSA.

29. Although the 340B statute expressly directs the Secretary to promulgate regulations to implement other changes enacted as part of the Affordable Care Act, no provision

authorizes HHS or HRSA to issue rules concerning the orphan drug exclusion. *Cf.* 42 U.S.C. § 256b(d)(1)(B)(vi) (providing for "[t]he imposition of sanctions in the form of civil monetary penalties, which . . . shall be assessed according to standards established in regulations to be promulgated by the Secretary"); *id.* § 256b(d)(3) (providing that "the Secretary shall promulgate regulations to establish and implement an administrative process for the resolution of claims by covered entities").

30. On May 20, 2011, HHS issued a proposed rule that interpreted the orphan drug exclusion 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 "transferred, prescribed, sold, or otherwise used for the rare condition or disease for which that orphan drug was designated under section 526 of the FFDCA." Exclusion of Orphan Drugs for Certain Covered Entities Under 340B Program, 76 Fed. Reg. 29183, 29189 (May 20, 2011) (proposed 42 C.F.R. § 10.21(a)).

31. During the notice-and-comment period, PhRMA expressed its support for the 340B Program as an important and necessary way to deliver drugs to underserved and uninsured patient populations. PhRMA and other industry stakeholders also commented that the third prong of the proposed rule was inconsistent with the plain language of the statute and would violate the Administrative Procedure Act if adopted. *See generally* 78 Fed. Reg. at 44020 (summarizing comments); Letter from PhRMA to HRSA Office of Pharmacy Affairs (July 19, 2011), *available at* http://www.regulations.gov/#!documentDetail;D=HRSA-2011-0001-0003.

32. Despite these comments, HHS promulgated the Final Rule without material change on July 23, 2013. The Final Rule has an effective date of October 1, 2013. 78 Fed. Reg. at 44016.

33. The Final Rule retained the proposed rule's facially invalid interpretation of the orphan drug exemption in Section 340B(e). Contrary to the statutory text, the Final Rule asserts that an orphan drug is exempt from 340B pricing requirements ***only*** when "***used*** for the rare condition or disease for which that orphan drug was designated." 78 Fed. Reg. at 44027 (emphasis added). The text as rewritten by HHS in the Final Rule would read:

> 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 ***and used to treat*** ~~for~~ a rare disease or condition.

(emphasis and strikethrough added).

34. If Congress had intended to impose this use-based limitation on the orphan drug exclusion, it could easily have done so. Other language in the Affordable Care Act demonstrates that Congress knew how to write a more restrictive orphan drug exemption. Specifically, the Affordable Care Act exempts from the annual drug manufacturer fee "sales of any drug or biological product with respect to which a credit was allowed . . . under section 45C of the Internal Revenue Code" *except that* "[t]he preceding sentence shall not apply with respect to any such drug or biological product after the date on which such drug or biological product is *approved by the [FDA] for . . . any indication other than . . . the rare disease or condition with respect to which such credit was allowed*." Affordable Care Act § 9008(e)(3) (emphasis added).

35. Congress also has crafted such limiting language in relation to orphan drugs in several other contexts. *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 (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. § 360cc (providing seven years market exclusivity "for a drug designated under [section 526 of the FFDCA] for a rare disease or condition . . . *for such drug for such 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). These provisions are powerful evidence that Congress made a deliberate choice to define the orphan drug exclusion in Section 340B(e) without reference to the indication for which the drug is used.

36. On September 26, 2013, counsel for PhRMA contacted counsel for HHS and HRSA by telephone and sent a letter outlining PhRMA's concerns with the Final Rule, informing them of PhRMA's intent to file this action and requesting that HHS delay the scheduled October 1, 2013 implementation of the Final Rule pending resolution of this dispute.

37. On September 27, 2013, HHS informed PhRMA that HHS would not delay the effective date of the rule.

The Final Rule Has Harmed And Will Continue To Harm PhRMA's Members

38. The Final Rule adversely affects PhRMA's members in several ways. PhRMA's members, therefore, are aggrieved persons entitled to judicial review of HRSA's Final Rule. 5 U.S.C. § 702. PhRMA is also an aggrieved person entitled to judicial review within the meaning of 5 U.S.C. § 702 because it is an association whose membership consists of drug manufacturers and whose purposes include advocating on behalf of its members with respect to issues relating to federal programs, such as the 340B Program.

39. First, as a direct result of the Final Rule, PhRMA's members must provide orphan drugs to the new categories of covered entities at 340B prices when such drugs are used for non-

orphan indications, even though the statute expressly relieves manufacturers from any such obligation. PhRMA's members will suffer financial and other harms as a result of HRSA's rule extending these price controls beyond the clear boundaries of the 340B statute.

40. Second, in order to track when newly eligible covered entities dispense orphan drugs and to ascertain whether these entities are in fact purchasing outpatient orphan drugs for orphan indications at 340B prices, PhRMA's members will be forced to spend significant resources adapting their internal systems and requiring wholesalers that sell 340B drugs to adapt their systems as well. The covered entities too will need to develop systems to track the indications for which orphan drugs are ultimately used.

41. Third, PhRMA's members face significant uncertainty regarding their legal and practical ability to obtain restitution of the monies lost due to the Final Rule if the rule is deemed unlawful, given that: (a) HRSA has not yet taken steps to implement the mandatory dispute resolution process required by the Affordable Care Act, *see* 42 U.S.C. § 256b(d)(3); (b) the existing dispute resolution process is entirely "voluntary," *see generally* Manufacturer Audit Guidelines and Dispute Resolution Process, 61 Fed. Reg. 65406 (Dec. 12, 1996); and (c) the structure and size of the 340B Program make it untenable to seek and recover refunds from covered entities. According to the Final Rule, "as of April 1, 2013, 967 parent facilities and 2212 outpatient/child sites" of covered entities are subject to the orphan drug exclusion in Section 340B(e). 78 Fed. Reg. at 44026. Requiring each of PhRMA's members to seek monetary relief individually from thousands of 340B entities is not an adequate remedy.

42. Fourth, by limiting the scope of the orphan drug exclusion, the Final Rule also works against the Orphan Drug Act's incentives to develop new orphan drugs.

43. A decision by this Court preliminarily and permanently enjoining the Final Rule would redress these injuries. Because PhRMA is seeking aggregate, not individualized, relief, this suit does not require the participation of the individual PhRMA members adversely affected by the Final Rule.

CLAIMS FOR RELIEF

COUNT 1

(Violation Of Administrative Procedure Act, 5 U.S.C. § 706(2)(C): Defendants Did Not Have Authority To Promulgate The Final Rule)

44. PhRMA re-alleges and incorporates paragraphs 1 through 43 of this Complaint.

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

46. Under the Administrative Procedure Act, this Court is empowered to "hold unlawful and set aside agency action, findings, and conclusions found to be . . . in excess of statutory jurisdiction, authority, or limitations, or short of statutory right." *Id.* § 706(2)(C).

47. Neither the PHSA nor any other statute confers on Defendants the authority to promulgate regulations regarding the scope of the orphan drug exemption in Section 340B(e) of the PHSA.

48. Because Defendants lacked authority to promulgate the Final Rule, the rule is invalid.

COUNT 2

(Violation Of Administrative Procedure Act, 5 U.S.C. § 706(2)(A): The Final Rule Is Arbitrary, Capricious, Or Otherwise Not In Accordance With Law)

49. PhRMA re-alleges and incorporates paragraphs 1 through 48 of this Complaint.

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

51. Under the Administrative Procedure Act, this Court is empowered to "hold unlawful and set aside agency action, findings, and conclusions found to be . . . arbitrary, capricious, an abuse of discretion, or otherwise not in accordance with law." *Id.* § 706(2)(A).

52. Section 340B(e) of the PHSA exempts from 340B pricing all drugs "***designated by the Secretary*** . . . for a rare disease or condition" that are sold to the new categories of covered entities added by the Affordable Care Act. 42 U.S.C. § 256b(e) (emphasis added).

53. The Final Rule limits this exemption to those orphan drugs that are ultimately used to treat a rare disease or condition (*i.e.*, used for their orphan indication). 78 Fed. Reg. at 44027.

54. Defendants' interpretation of Section 340B(e) contravenes the plain language of the statute and is therefore arbitrary, capricious, or not in accordance with law.

PRAYER FOR RELIEF

PhRMA prays that this Court:

1. Declare that Defendants violated the Administrative Procedure Act in promulgating the Final Rule because they did not have the authority to issue the Final Rule;

2. Declare that Defendants violated the Administrative Procedure Act because the Final Rule is arbitrary, capricious, an abuse of discretion, or otherwise not in accordance with law;

3. Grant an order preliminarily enjoining Defendants from implementing the Final Rule;

4. Grant an order and judgment invalidating and permanently enjoining Defendants from enforcing the Final Rule;

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

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

Respectfully submitted,

By /s/ Jeffrey L. Handwerker

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