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, et al., Defendants.	Civil Action No. 1:14-cv-01685

MEMORANDUM OF POINTS AND AUTHORITIES IN SUPPORT OF PLAINTIFF'S MOTION FOR SUMMARY JUDGMENT AND OPPOSITION TO DEFENDANTS' MOTION FOR SUMMARY JUDGMENT

JEFFREY L. HANDWERKER (#451913)
MATTHEW T. FORNATARO
KRISTIN M. HICKS
BRANDON L. BOXLER

ARNOLD & PORTER LLP
555 Twelfth Street, NW
Washington, DC 20004-1206
+1 202.942.5000
+1 202.942.5999

Counsel for Pharmaceutical Research and Manufacturers of America

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TABLE OF CONTENTS

TABLE OF AUTHORITIES

Cases **Page(s)**

Statutes and Regulations

Other Authorities

* *Denotes cases and authorities on which PhRMA principally relies.*

INTRODUCTION

The orphan drug exclusion in 42 U.S.C. § 256b(e) speaks in plain terms. Under the heading "EXCLUSION OF ORPHAN DRUGS FOR CERTAIN COVERED ENTITIES," the statute mandates that, for entities the Affordable Care Act made eligible to participate in the 340B Program, the term "covered outpatient drug" does not include "*a drug* designated by the Secretary under [21 U.S.C. § 360bb] for a rare disease or condition." 42 U.S.C. § 256b(e) (emphasis added). This language is unequivocal, unambiguous, and requires no interpretation: if "a drug" is "designated" as orphan, it is excluded from 340B pricing requirements when sold to a newly eligible covered entity.

HHS clearly disagrees with Congress's policy choice. The agency has sought—on two separate occasions—to rewrite § 256b(e) to limit its scope to exclude only those drugs that are both designated as orphan and also used for the orphan indication. HHS's first attempted rewrite occurred in July 2013 when, after two years of notice-and-comment rulemaking, the agency issued a regulation that would have applied the statutory exclusion only when an orphan drug is "transferred, prescribed, sold, or *otherwise used for the rare condition or disease* for which that orphan drug was designated." Administrative Record ("AR") 676 (78 Fed. Reg. 44016, 44027 (July 23, 2013)) (emphasis added). In the preamble to that regulation, HHS acknowledged that "a regulation is *necessary* to implement these *changes*." AR 666 (78 Fed. Reg. at 44017) (emphasis added).

Congress, however, did not authorize HHS to issue substantive rules related to § 256b(e), and for that reason this Court vacated the agency's regulation as *ultra vires*. *PhRMA v. HHS*, No. 13-1501, 2014 WL 2171089, at *12 (D.D.C. May 23, 2014). And because the interpretation set out in HHS's regulation creates "legal effect[s]," this Court also was inclined to think that the

regulation could not survive as an interpretive rule. *Id.* at *13. Nonetheless, the Court invited HHS to submit further briefing defending the regulation as an interpretive rule if the agency wished to pursue that theory further. *Id.*

HHS *did* wish to pursue the interpretive rule theory further, but chose to do so without the benefit of this Court's analysis. The agency thus declined the Court's invitation and unilaterally issued a so-called "interpretive" rule that, as HHS acknowledges, reiterates the exact same use-based limit as the vacated regulation. *See* AR 680-86 ("July 2014 Rule"). The agency then followed up its July 2014 Rule with letters to manufacturers, ordering them to comply with the new rule and threatening enforcement actions and sanctions for noncompliance.

On these facts, it is remarkable that HHS now tries to escape judicial review, claiming that the July 2014 Rule is not "final agency action" under the Administrative Procedure Act ("APA"). The use-based interpretation adopted in that rule is the same one HHS adopted in the vacated regulation, the same one HHS acknowledged creates "changes" for which "a regulation is necessary to implement," and the same one this Court explained has "legal effect[s]." Those legal effects are the hallmark of final agency action, and HHS is implementing those effects with threats of enforcement actions and sanctions.

Moreover, the July 2014 Rule fails judicial review because it arbitrarily grafts onto § 256b(e) a use-based limit that Congress did not impose. If Congress had intended to include such a limit, it could have done so by including the word "use" or "indication," just as it has done in many other statutory provisions relating to orphan drugs. Congress's decision to write § 256b(e) differently (*i.e.*, no use-based language) means the statute has a different meaning (*i.e.*, no use-based limit). The Court should review and invalidate the July 2014 Rule as "arbitrary, capricious, an abuse of discretion, or otherwise not in accordance with law," 5 U.S.C.

§ 706(2)(A), and enter summary judgment in favor of Plaintiff Pharmaceutical Research and Manufacturers of America ("PhRMA").

BACKGROUND, PROCEDURAL HISTORY, AND STATEMENT OF FACTS

The 340B Program

Congress established the 340B Program to help uninsured and indigent patients gain better access to outpatient prescription drugs. *See* H.R. Rep. No. 102-384, pt. II, at 12 (1992). As a condition of Medicaid covering their products, manufacturers must enter into a "Pharmaceutical Pricing Agreement" with the Secretary of HHS. *See* 42 U.S.C. § 256b(a)(1); *Astra USA, Inc. v. Santa Clara Cnty.*, 131 S. Ct. 1342, 1345 (2011). That agreement requires manufacturers to charge covered entities no more than a statutorily defined "ceiling price" on "covered outpatient drugs." 42 U.S.C. § 256b(a)(1); *see also id.* § 1396r-8(a)(1), (5). The 340B ceiling price can be up to 50% lower than the non-340B price. *See* AR 675 (78 Fed. Reg. at 44026); *see also* 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").

The Affordable Care Act of 2010 caused an "unprecedented" increase in the number of covered entities. AR 404; *see also id.* at 041, 614. Generally speaking, "disproportionate share hospitals" that provide inpatient services to a specified percentage of low-income patients constituted the majority of the entities eligible to participate in the 340B Program, *see* Veterans Health Care Act of 1992, Pub. L. No. 102-585, § 602(a)(4), but the Affordable Care Act expanded eligibility to include additional categories of children's hospitals, free-standing cancer hospitals, critical access hospitals, rural referral centers, and sole community hospitals, *see* Pub. L. No. 111-148, § 7101(a) (2010) (codified at 42 U.S.C. § 256b(a)(4)(M)-(O)). Today, nearly

one out of three hospitals in the United States participates in the 340B Program. GAO 340B Report at 20.

The Orphan Drug Exclusion

The Affordable Care Act exempts from 340B pricing orphan-designated drugs sold to the newly eligible covered entities:

> EXCLUSION OF ORPHAN DRUGS FOR CERTAIN COVERED ENTITIES—For [newly] covered entities . . . , the term "covered outpatient drug" shall not include *a drug* designated by the Secretary under [21 U.S.C. § 360bb] for a rare disease or condition.

Pub. L. No. 111-152, § 2302 (2010) (codified at 42 U.S.C. § 256b(e)) (emphasis added).

The cross-referenced statute—21 U.S.C. § 360bb—sets forth the process for "orphan drug" designation. Under that process, the Secretary of HHS may designate a drug as orphan if the drug has the 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).

Separate from this orphan *designation* process, a manufacturer also may obtain *approval* to market an orphan-designated drug as a treatment for a particular disease or condition. *See* 21 U.S.C. § 360cc. "The award of orphan-drug designation does not alter the standard regulatory requirements and process for obtaining marketing approval for the drug, which is a separate process." HHS, Mem. in Supp. of Defs.' Mot. for Summ. J. 7 (Jan. 27, 2015), ECF No. 14-1 ("HHS Mot."); *see* AR 666 (78 Fed. Reg. at 44017) (same). After designating a drug as orphan, the Secretary may later approve the drug for use in an orphan indication, for use in a non-orphan indication, for use in both types of indications, or may not approve the orphan-designated drug at all. HHS Mot. 7; *see* AR 666 (78 Fed. Reg. at 44017). Congress has enacted various "incentives" to encourage "the development of orphan drugs," Orphan Drug Act, Pub. L. No. 97-414, § 1(b)(6) (1983), including 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. Unlike the orphan drug exclusion in § 256(e), these other incentives expressly apply to the orphan "use" or "indication." *See* AR 666 (78 Fed. Reg. 44017).

The July 2013 Final Rule

After Congress enacted the orphan drug exclusion, many covered entities and their trade associations tried to limit its scope or repeal it altogether. *See, e.g.*, AR 105, 113-22. For example, amicus Safety Net Hospitals for Pharmaceutical Access ("SNHPA") urged HHS to "work with Congress to remove the orphan drug exclusion expeditiously." AR 103. SNHPA also provided HHS with a list of "Possible Legislative and Administrative Fixes" to restrict the scope of § 256b(e), including convincing Congress to limit the exclusion to only those drugs that are *both* "designated" under 21 U.S.C. § 360bb *and* also "approved" for use under 21 U.S.C. § 360cc. AR 191. SNHPA also suggested that Congress could "fix[]" the statute by limiting the exclusion "to Orphan Indication Use Only." AR 191. If Congress did not enact such a use-based limit, SNHPA continued, HHS could impose the limit itself via administrative action, "read[ing] the statutory language to mean that the exemption only applies when the 340B covered entity uses the drug for the rare disease or condition for which the drug received its orphan drug designation." AR 191; *see also* AR 192.

HHS first announced its use-based "interpretation" of § 256b(e) in July 2013. *See* AR 665 (78 Fed. Reg. at 44016) ("July 2013 Final Rule"). Following two years of formal notice-and-comment rulemaking, HHS issued the July 2013 Final Rule, which provided that § 256b(e) would apply only when an orphan drug is "transferred, prescribed, sold, or *otherwise used for the rare condition or disease* for which that orphan drug was designated." AR 676 (78 Fed. Reg. at

44027) (emphasis added). The rule also required covered entities "to maintain and provide auditable records" demonstrating their compliance with the regulation. AR 677 (78 Fed. Reg. at 44028). HHS concluded in the July 2013 Final Rule that "a regulation is necessary to implement these changes." AR 666 (78 Fed. Reg. at 44017).

This Court struck down the July 2013 Final Rule in May 2014, holding that Congress did "not confer upon HHS authority to issue the rule." *PhRMA*, 2014 WL 2171089, at *9. The Court also explained that the July 2013 Final Rule probably would fail 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.* at *13 (emphasis added). The Court invited HHS to submit more briefing on whether the July 2013 Final Rule could survive as an interpretive rule, *id.* at *14, but the agency "decline[d]," 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.

One month after declining this Court's invitation, HHS announced that it "intend[ed] to issue" a new interpretive rule setting forth "the *same interpretation* previously embodied in" the July 2013 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 (emphasis added).

The July 2014 Rule

HHS released its new "interpretive rule" on July 23, 2014, *see* AR 680, and published an announcement of the release in the Federal Register, *see* AR 678. This July 2014 Rule reiterates verbatim the use-based interpretation of § 256b(e) set out in the vacated July 2013 Final Rule. *See* AR 685-86. It also revives the July 2013 Final Rule's tracking requirements, warning that 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." AR 685.

An HHS official, Commander Krista Pedley, attested in an affidavit to this Court that a manufacturer would face sanctions for not complying with the agency's use-based interpretation in the July 2014 Rule: "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." Declaration of Krista Pedley ¶ 6, *PhRMA v. HHS*, No. 13-1501 (July 24, 2014), ECF No. 53-1 ("Pedley Decl. II"). HHS's website similarly warns that a manufacturer failing to comply with the agency's "interpretation" could face "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." PhRMA, Am. Compl. Ex. D (Dec. 15, 2014), ECF No. 8-5. The agency also has published on its website the names of manufacturers that, according to HHS, are "not complying" with the July 2014 Rule. HHS, Manufacturer List: Orphan Drug Availability Update, http://www.hrsa.gov/opa/programrequirements/orphandrugexclusion/manufacturerlist.html (last visited Feb. 24, 2015). And HHS has sent letters to orphan drug manufacturers asserting that they are "out of compliance" with the use-based pricing requirements "described in [the July 2014 Rule]," accusing them of "violating . . . their Pharmaceutical Pricing Agreement," ordering them to issue "refund[s]" to covered entities, and directing them to "offer . . . the discounted price in the future." PhRMA, Am. Compl. Ex. E (Dec. 15, 2014), ECF No. 8-6; *see also* PhRMA, Am. Compl. Ex. F (Dec. 15, 2014), ECF No. 8-7 (noting that HHS "has told more than 50 drug manufacturers to [issue] refund[s]").

SUMMARY OF ARGUMENT

The July 2014 Rule satisfies both elements of the two-prong test for finality articulated in *Bennett v. Spear*, 520 U.S. 154 (1997). To qualify as "final," an agency action must "mark the consummation of the agency's decisionmaking process" and "be one by which rights or obligations have been determined, or from which legal consequences will flow." *Id.* at 177-78 (quotation marks omitted). HHS concedes that its July 2014 Rule "marks the consummation of [the] agency's decision-making process." HHS Mot. 2. The July 2014 Rule also creates legal consequences—the same ones this Court identified when analyzing the July 2013 Final Rule because, as HHS also concedes, the two rules adopt "essentially the same interpretation" of § 256b(e). HHS Mot. 2; *see also id.* at 10 (same).

HHS cannot evade judicial review by describing its July 2014 Rule as "interpretive" and "non-binding." HHS Mot. 11. Courts "do not classify a rule as interpretive just because the agency says it is." *Chamber of Commerce v. Occupational Safety & Health Admin.*, 636 F.2d 464, 468 (D.C. Cir. 1980). Nor do they take an agency at its word that a rule is non-binding, especially when the agency applies the rule "in a way that indicates it is binding" or treats the rule "as a norm" by which regulated parties must "shape their actions." *Gen. Elec. Co. v. E.P.A.*, 290 F.3d 377, 383 (D.C. Cir. 2002) (quotation marks omitted). Instead, courts "look behind the particular label applied by the agency" to discern the "real intent and effect" of the action. *Batterton v. Marshall*, 648 F.2d 694, 705 n.58 (D.C. Cir. 1980). Here, the real intent and effect of the July 2014 Rule is to obligate manufacturers to sell orphan drugs at 340B prices when such drugs are not used to treat a rare disease or condition—an obligation that HHS is enforcing as a binding norm, threatening sanctions for noncompliance. Where, as here, an "agency publicly articulates an unequivocal position . . . and expects regulated entities to alter their primary

conduct to conform to that position, the agency has voluntarily relinquished the benefit of postponed judicial review." *Ciba-Geigy Corp. v. E.P.A.*, 801 F.2d 430, 436 (D.C. Cir. 1986).

On the merits, the July 2014 Rule fails because it is flatly at odds with the text and history of the 340B statute. The orphan drug exclusion applies to any "drug" designated as orphan, not to only certain uses of those drugs. If Congress had intended to limit § 256b(e) to only certain uses of orphan drugs, it could have said so—most obviously by using the word "use" or "indication." The lack of any such use-based language in § 256b(e) is powerful evidence of congressional intent, particularly because Congress has added use-based language to other orphan drug-related provisions, including a provision of the Affordable Care Act—*the same statute* codifying the orphan drug exclusion. Congress also revisited and amended § 256b(e) less than a year after passing it into law, but refused to add a use-based limit despite requests to do so.

Congress's decision to tie § 256b(e) to orphan "designation" under 21 U.S.C. § 360bb further confirms that the orphan drug exclusion does not contain a use-based limit. An orphan designation attaches to "a drug"—the whole drug—regardless of how it is used. This drug-based process of orphan *designation* stands in stark contrast to the separate use-based process of orphan *approval*, which Congress did not reference anywhere in § 256b(e).

HHS is free to disagree with Congress's decision not to include a use-based limit on the scope of the orphan drug exclusion. The agency, however, does "not have inherent authority to second-guess Congress[]." *Pub. Citizen v. F.T.C.*, 869 F.2d 1541, 1557 (D.C. Cir. 1989). Nor may it "rewrite a statute's plain text" to make the statute correspond with the agency's policy preference. *Landstar Express Am., Inc. v. Fed. Mar. Comm'n*, 569 F.3d 493, 498 (D.C. Cir. 2009). To implement its preferred use-based policy, HHS should be working with Congress to

amend the statute, not dressing a vacated regulation in the new clothing of an interpretive rule and attempting to circumvent judicial review. The July 2014 Rule is reviewable final agency action and should be vacated, just like its twin predecessor.

STANDARD OF REVIEW

Summary judgment is appropriate when the pleadings and evidence demonstrate that "there is no genuine dispute as to any material fact and the movant is entitled to judgment as a matter of law." Fed. R. Civ. P. 56(a). Because this case involves review of agency action under the APA, the Court's role is "limited" to reviewing the administrative record and "deciding, as a matter of law, whether the agency action is . . . consistent with the APA standard of review." *AFL-CIO v. Chao*, 496 F. Supp. 2d 76, 81-82 (D.D.C. 2007). An agency rulemaking is invalid under the APA if it is "arbitrary, capricious, an abuse of discretion, or otherwise not in accordance with law." 5 U.S.C. § 706(2)(A).

ARGUMENT

I. The July 2014 Rule Is Final Agency Action Reviewable Under The APA

The APA empowers courts to review "final agency action for which there is no other adequate remedy in a court." 5 U.S.C. § 704. Agency action is "final" when it meets two conditions. First, the action must "mark[] the 'consummation' of the agency's decisionmaking process." *Sackett v. E.P.A.*, 132 S. Ct. 1367, 1372 (2012) (quoting *Bennett*, 520 U.S. at 177-78). HHS agrees that the July 2014 Rule meets this requirement: "It is uncontested that the [July 2014 Rule] at issue satisfies the first part of the *Bennett* test, as a consummation of the agency's decision-making process." HHS Mot. 11; *see also id.* at 2 ("Granted, the [July 2014 Rule] marks the consummation of an agency's decision-making process.").

The second element of finality is that "the action must be one by which rights or obligations have been determined, or from which legal consequences will flow." *Bennett*, 520

U.S. at 178 (quotation marks omitted). Courts apply this requirement in a "flexible" and "pragmatic way," focusing on the real-world effects of the action, not the label the agency has attached to it. *Abbott Labs. v. Gardner*, 387 U.S. 136, 149-50 (1967), *abrogated on other grounds by Califano v. Sanders*, 430 U.S. 99 (1977). "In particular, we look primarily to whether the agency's position . . . has a 'direct and immediate effect on the day-to-day business' of the parties challenging the action." *Ciba-Geigy*, 801 F.2d at 435-36 (quoting *F.T.C. v. Std. Oil Co. of Cal.*, 449 U.S. 232, 239 (1980)). Moreover, "[t]he term 'agency action' encompasses an agency's interpretation of law." *Id.* at 435. Thus, if HHS's interpretation of § 256b(e) set out in the July 2014 Rule determines rights and obligations, imposes legal consequences, or otherwise has a "direct and immediate effect on the day-to-day business" of PhRMA's members, the rule creates legal effects and is final agency action reviewable under the APA. The July 2014 Rule does all those things, and thus amply meets the second element of "finality."

A. HHS And This Court Already Have Described The Legal Effects Of The "Interpretation" Set Out In The July 2014 Rule

The finality analysis can begin and end with the prior conclusions of HHS and this Court. Both the July 2013 Final Rule and this Court's decision vacating that rule identified several ways the agency's interpretation of § 256b(e) alters legal rights and obligations. Those same legal effects also result from the July 2014 Rule because, as HHS concedes, the new rule "adopt[s] essentially the same interpretation" as the July 2013 Final Rule. HHS Mot. 10.

HHS recognized in the July 2013 Final Rule "that the[] *new requirements*" imposed by its use-based interpretation "will require" regulated parties to adopt "additional procedures and system capabilities." AR 670 (78 Fed. Reg. at 44021) (emphasis added). The agency also acknowledged that its interpretation "implement[s] a *revision* to the preexisting statutory recordkeeping requirement[s]," and would cause substantial "revenue losses" for manufacturers.

AR 674-75 (78 Fed. Reg. at 44025-26) (emphasis added). Regulated entities, HHS continued, "will need to determine" how to satisfy these new requirements even though complying with them "may be challenging." AR 669-70 (78 Fed. Reg. at 44020-21); *see also id.* (describing stakeholder concerns that the agency's interpretation will create new "costs," "burdens," "difficult[ies]," and "expenses").

Commander Pedley submitted an affidavit that described many other consequences of the July 2013 Final Rule. For example, her affidavit included a section entitled "Projected Impact of the Rule," which highlighted how the agency's interpretation of § 256b(e) would reallocate hundreds of millions of dollars among 340B stakeholders. Declaration of Krista Pedley ¶¶ 20-24, *PhRMA v. HHS*, No. 13-1501 (Dec. 13, 2013), ECF No. 24-3 ("Pedley Decl. I"). She also explained how "the *new requirements* of the [July 2013] Final Rule will require" newly covered entities to implement "additional procedures and system capabilities" to track how they use orphan drugs. Pedley Decl. I, ¶ 23 (emphasis added).

In light of these "revision[s]," "new requirements," and other legal effects flowing from the agency's use-based interpretation of the orphan drug exclusion, HHS correctly recognized in the July 2013 Final Rule that "a regulation is *necessary* to implement these *changes*." AR 666 (78 Fed. Reg. at 44017) (emphasis added). Indeed, the changes were so substantial that the July 2013 Final Rule was "the first time" in the history of the 340B Program that HHS chose to promulgate "a regulation and not a mere guidance document." *PhRMA*, 2014 WL 2171089, at *13 n.18 (citing AR 666 (78 Fed. Reg. at 44017)). The Office of Management and Budget similarly deemed the rule "significant action" under section 3(f) of Executive Order 12866. AR 674 (78 Fed. Reg. at 44025).

This Court also has articulated the substantial legal effects that flow from HHS's use-based interpretation. In its decision vacating the July 2013 Final Rule, the Court explained that the "effect of th[e] rule is that the discounted 340B price is not available to newly-added covered entities when purchasing orphan drugs for their intended orphan use." *PhRMA*, 2014 WL 2171089, at *3. Moreover, the agency's interpretation "imposes duties on the covered entities to maintain records of compliance." *Id.* Given these new rights and obligations, "the Court [was] inclined to think" that the July 2013 Final Rule could not survive as a mere "interpretive rule." *Id.* at *13. As the Court reasoned, "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).

The July 2014 Rule creates these same legal effects because it adopts the same interpretation and similar compliance requirements. *Compare* AR 676-77 (78 Fed. Reg. at 44027-28), *with* AR 685-86. The July 2014 Rule thus "alter[s] the legal regime to which [340B stakeholders are] subject" and is final agency action subject to review. *Bennett*, 520 U.S. at 178; *see also Barrick Goldstrike Mines Inc. v. Browner*, 215 F.3d 45, 48 (D.C. Cir. 2000) (holding that an agency "guidance document" was "final agency action" with "legal consequences" because, among other reasons, it altered the scope of an exception to a statutory requirement and imposed on certain companies an obligation "to keep track of . . . releases of toxic substances").

B. The July 2014 Rule Has Legal Effects Because It "Implements" The Orphan Drug Exclusion

The July 2014 Rule also is reviewable final agency action because its purpose and effect is to "implement" the orphan drug exclusion. Indeed, the title of the July 2014 Rule is "Interpretive Rule: *Implementation of the Exclusion of Orphan Drugs* for Certain Covered Entities Under the 340B Program." AR 680 (emphasis added). The text of the rule similarly

states that "[t]he purpose of this document is to describe the manner in which [§ 256b(e)] will be interpreted *and implemented by HHS*." AR 680 (emphasis added).

An agency action that implements a statutory provision is, by definition, a legislative rule with legal effects. "Legislative rules . . . implement congressional intent; they effectuate statutory purposes. In so doing, they grant rights, impose obligations, or produce other significant effects on private interests." *Batterton*, 648 F.2d at 701-02 & n.29 (citing cases); *see also Chrysler Corp. v. Brown*, 441 U.S. 281, 302-03 (1979) ('"Legislative, or substantive, regulations . . . implement the statute.'" (quoting *Batterton v. Francis*, 432 U.S. 416, 425 n.9 (1977))). By contrast, interpretive rules simply state what the agency "thinks the statute . . . means." *Chamber of Commerce*, 636 F.2d at 469.

A self-styled interpretive rule that implements a statutory provision is treated—and reviewed under the APA—as final agency action with legal effects. As the D.C. Circuit explained in a case on which HHS relies, "if an agency issues a statement that is labeled an interpretative rule . . . and it has all of the indicia of a final legislative rule, then the rule will be subject to review." *Am. Tort Reform Ass'n v. Occupational Safety & Health Admin.*, 738 F.3d 387, 395 (D.C. Cir. 2013) (HHS Mot. 12). In *Mendoza v. Perez*, 754 F.3d 1002 (D.C. Cir. 2014), for example, the court held that various purported "interpretive rules" were actually final agency action subject to review because they "endeavor[ed] to implement the statute, the effect of a legislative rule." *Id.* at 1023 (quotation marks omitted). The same was true in *Batterton*, where the court held that an agency document was not a mere interpretive rule but was instead a legislative rule subject to review because it set forth criteria to "implement[] . . . statutory provisions." 648 F.2d at 704. And in *Credit Union National Association v. National Credit Union Administration Board*, 573 F. Supp. 586 (D.D.C. 1983), this Court held that "it is

inescapable that [the agency action] is a legislative rather than an interpretative rule, despite [the agency's] own characterization," because the agency "clearly issued [the rule] to *implement*" a statutory provision. *Id.* at 591 (emphasis in original).

So too here. As the title and text of the July 2014 Rule demonstrate, HHS did not issue the rule simply to say what it thinks § 256b(e) means. Instead, HHS issued the rule to implement the orphan drug exclusion and impose a new set of norms that will control how orphan drugs are priced, bought, and sold in the 340B Program. Such legal effects "expose[] the [agency's] true intent" and satisfy the finality requirement of the APA. *Chamber of Commerce*, 636 F.2d at 469.

C. The July 2014 Rule Is "Final" Because HHS Expects Regulated Entities To Comply With The Rule's Interpretation Of § 256b(e)

In addition to its legal effects and express purpose of implementing the orphan drug exclusion, the July 2014 Rule is final agency action because HHS "views its deliberative process as sufficiently final to demand compliance with its announced position." *Ciba-Geigy*, 801 F.2d at 436. "This court has frequently held that an agency's interpretation of . . . [a] statute, with the expectation that regulated parties will conform to and rely on this interpretation, is final agency action fit for judicial review." *Indep. Bankers Ass'n of Am. v. Smith*, 534 F.2d 921, 929 & n.29 (D.C. Cir. 1976) (citing cases). When an agency "issue[s] a definitive statement of [its] legal position" and puts companies "to the painful choice between costly compliance and the risk of prosecution," the agency "cannot . . . evade judicial review" by claiming its action is not "final." *CSI Aviation Servs., Inc. v. U.S. Dep't of Transp.*, 637 F.3d 408, 412-13 (D.C. Cir. 2011).

In an affidavit submitted to this Court, in letters to manufacturers, and on its official agency website, HHS repeatedly has decreed that regulated entities must comply with the use-based interpretation in the July 2014 Rule. For example, Commander Pedley warned in her declaration that "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." Pedley Decl. II, ¶ 6. The agency also has sent letters to manufacturers threatening enforcement actions for being "out of compliance with the statutory requirements as described in [the July 2014 Rule]." PhRMA, Am. Compl. Ex. E (Dec. 15, 2014), ECF No. 8-6. And HHS's official website admonishes that any manufacturer failing to comply with the agency's "interpretation" could face "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." PhRMA, Am. Compl. Ex. D (Dec. 15, 2014), ECF No. 8-5. These are powerful threats of significant penalties: terminating a manufacturer's Pharmaceutical Pricing Agreement potentially would result in the loss of Medicaid and Medicare Part B reimbursement for all of the manufacturer's products. *See supra* p. 3. HHS clearly has "the expectation that regulated parties will conform"—so much so that the agency is willing to impose severe sanctions for not obeying its use-based interpretation of § 256b(e).

HHS does not dispute that it expects manufacturers to change their conduct and comply with the agency's interpretation, contending instead that the July 2014 Rule is not yet "final" because the agency has not yet sanctioned a manufacturer for noncompliance. *See* HHS Mot. 12-17. According to the agency, "until HHS initiates an enforcement action against a drug manufacturer *and* imposes a penalty for not complying with the statutory provision, there is no final agency action subject to judicial review." HHS Mot. 15 (emphasis added).

The agency is wrong. No enforcement action—and certainly no penalty—is required to make an action "final." Finality exists when an agency announces "a position it plans to follow [and] . . . insist[s] [others] comply with" it. *Appalachian Power Co. v. E.P.A.*, 208 F.3d 1015, 1022 (D.C. Cir. 2000). That is exactly what HHS has done here: it has announced its

interpretation of § 256b(e) and threatened enforcement actions and sanctions for noncompliance. A manufacturer's exposure to penalties "in a future enforcement proceeding" is a "legal consequence[]" that makes the July 2014 Rule "final" and reviewable under the APA. *Sackett*, 132 S. Ct. at 1371; *see also Frozen Food Express v. United States*, 351 U.S. 40, 44-45 (1956) (holding that an agency action was reviewable because the agency had "warn[ed]" companies that they "risk" incurring penalties if they failed to comply).

To support its "sanctions first, review later" refrain, HHS ironically cites *Abbott Laboratories* (HHS Mot. 15)—a case in which the Supreme Court in fact held that an agency action *was* reviewable *before* the agency initiated an enforcement action or imposed any sanctions. In language equally appropriate for this case, the Supreme Court described the pre-enforcement impact of the agency action as follows:

> [T]he impact of the [agency action] upon the petitioners is sufficiently direct and immediate as to render the issue appropriate for judicial review at this stage. The[] [agency action] purport[s] to give an authoritative interpretation of a statutory provision that has a direct effect on the day-to-day business of all prescription drug companies; its promulgation puts petitioners in a dilemma . . . . Either they must comply with the [agency's action] and incur the costs of changing over their promotional material and labeling or they must follow their present course and risk prosecution.

Abbott Labs., 387 U.S. at 152 (quotation marks omitted).

The July 2014 Rule puts manufacturers in the same compliance dilemma. PhRMA's members must either "make significant changes in their everyday business practices" or expose themselves "to the imposition of strong sanctions." *Id.* at 154. That coercive pressure makes the July 2014 Rule final. "[W]here threatened action by *government* is concerned, [courts] do not require a plaintiff to expose himself to liability before bringing suit to challenge the basis for the threat." *MedImmune, Inc. v. Genentech, Inc.*, 549 U.S. 118, 128-29 (2007) (emphasis in

original); *see also CSI Aviation*, 637 F.3d at 412 (holding that an agency letter was "final agency action" because it put companies in a "conundrum" of either complying or risking prosecution).[1]

Moreover, any enforcement action HHS brings would have a predetermined result. The Affordable Care Act requires the agency to establish a dispute-resolution process over which the agency itself will preside and issue final decisions that are "binding upon the parties." 42 U.S.C. § 256b(d)(3)(B)-(C). Under HHS's reasoning, manufacturers must go through this pre-ordained dispute-resolution process, receive an adverse decision, have their Pharmaceutical Pricing Agreements canceled, potentially lose Medicaid and Medicare Part B reimbursement for their products, and *only then* seek judicial review of the agency's interpretation of § 256b(e). Plaintiffs, however, do not have to "bet the farm" to obtain pre-enforcement review when faced with a "genuine threat of enforcement." *MedImmune*, 549 U.S. at 129. Where, as here, "severe[] and unnecessar[y]" penalties would result if judicial review waited until after the government brings an enforcement action, "access to the courts under the Administrative Procedure Act . . . must be permitted." *Abbott Labs.*, 387 U.S. at 153.

[1] The other decisions HHS cites for support do not remotely resemble this case, where the agency has consummated its decisionmaking process, issued a rule that implements a statutory provision and creates legal effects, and repeatedly threatened sanctions for noncompliance. In *Reliable Automatic Sprinkler Co. v. Consumer Product Safety Commission*, 324 F.3d 726, 731-33 (D.C. Cir. 2003) (HHS Mot. 15), the plaintiff admitted "that there ha[d] been no final agency action," and the agency merely had "stated an 'intention . . . to make [a] preliminary determination'" but had not yet "issued any order imposing any obligation." In *National Association of Home Builders v. Norton*, 415 F.3d 8, 11-15 (D.C. Cir. 2005) (HHS Mot. 14), the agency action declared "in both its title and text" that its terms were merely "recommended," and it "d[id] not contain any prohibitions or restrictions," did not suggest that compliance would "provide a 'safe harbor' from prosecution," and did not "contain[] any language compelling" action. In *Independent Equipment Dealers Association v. EPA*, 372 F.3d 420, 427-28 (D.C. Cir. 2004) (HHS Mot. 14), the agency sent a letter that "was purely informational in nature," "left the world just as it found it," and had no "concrete impact . . . whatsoever." In *Center for Auto Safety v. National Highway Traffic Safety Administration*, 452 F.3d 798, 807-09 (D.C. Cir. 2006) (HHS Mot. 14), the agency had "not commanded, required, ordered, or dictated" anything, had not made any "threats of enforcement," and had "emphasize[d] that [its] position . . . remains flexible." And in *AT&T Co. v. EEOC*, 270 F.3d 973, 975-76 (D.C. Cir. 2001) (HHS Mot. 14), the agency had not consummated its decisionmaking process and did not "force[] a party to change its behavior."

D. HHS's Remaining Arguments For Avoiding Judicial Review Fail

HHS makes two other arguments in an attempt to circumvent judicial review of its July 2014 Rule. First, the agency argues that the coercive pressure it has imposed on manufacturers is a "practical, not legal" consequence, and that practical consequences are irrelevant in the finality analysis. HHS Mot. 14. But the cases HHS cites actually refute this argument, and recognize that the practical effects of an agency action *do* factor into the finality analysis: "Finality resulting from the practical effect of an ostensibly non-binding agency proclamation *is* a concept we have recognized." *Norton*, 415 F.3d at 15 (emphasis added) (citing cases) (HHS Mot. 13, 14); *see also Indep. Equip. Dealers*, 372 F.3d at 427 (evaluating the "concrete impact" of an agency action in analyzing whether the action is "final") (HHS Mot. 14).

In any event, the July 2014 Rule does far more than impose practical consequences. It determines rights and obligations related to 340B pricing for orphan drugs, thereby forcing manufacturers to alter their business practices and comply with the agency's rule—or face severe sanctions. Analyzing these real-world pressures, dilemmas, and consequences is not improper, it is *required*, and it demonstrates why the July 2014 Rule is final agency action reviewable under the APA. *See Abbott Labs.*, 387 U.S. at 149-52 (analyzing how an agency action would affect "the day-to-day business" operations of companies in holding that the action was "final"); *see also Frozen Food*, 351 U.S. at 43-44 (analyzing the "practical impact" of an agency action in determining that the action was subject to review); *supra* Part I.C.

HHS's second argument is that the July 2014 Rule is interpretive because, "[i]n an enforcement action against a drug manufacturer, the agency would enforce the 340B *statute*, not the interpretive rule." HHS Mot. 3 (emphasis in original). But that argument simply proves the point: HHS's interpretation of § 256b(e) is so definitive, so binding, and so final that the agency intends to enforce it. In other words, HHS's argument that its future enforcement actions will

enforce "the statute" only confirms that the agency has made up its mind about what "the statute" means and expects everyone else to "fall in line" with that determination. *Appalachian Power*, 208 F.3d at 1023.

The agency's argument here is similar to the argument the D.C. Circuit rejected in *Chamber of Commerce*. In that case, an agency released a purported interpretive rule declaring that "'an employer's failure to pay employees for time during which they are engaged in walkaround inspections is discriminatory under [the Fair Labor Standards Act].'" 636 F.2d at 467 (quoting the rule). The agency tried to defend its "interpretive rule" as a mere explanation of what the Act required, urging the court to defer to the agency's "'expert conclusion'" and arguing that a company's failure to comply with the agency's interpretation '"will be charged with discriminating against their workers under Section 11(c) *of the Act*."' *Id.* at 467 & n.4 (quoting the agency) (emphasis added). That defense, the Court explained, demonstrated that the "interpretive rule" was final and reviewable because the agency—like HHS here—was treating its interpretation as a "binding" explanation of what the statute required. *Id.* at 467 n.4; *see also id.* at 468-69; *cf. Ciba-Geigy*, 801 F.2d at 436-39 (holding that a letter announcing an agency's definitive interpretation of a statute was final, reviewable agency action).

Regardless, the validity of HHS's argument that its enforcement actions would enforce "the statute" depends on the merits of the underlying issue—namely, whether the agency has correctly construed the statute. And because HHS's use-based interpretation of § 256b(e) is inconsistent with the statutory text, *see infra* Part II, any actions the agency brings to enforce its interpretation also would be contrary to—not in furtherance of—the statute.[2]

[2] Pre-enforcement judicial review is particularly appropriate here because this case presents "a purely legal question of statutory interpretation" and "review of the agency's legal position would not benefit from a more concrete setting." *CSI Aviation*, 637 F.3d at 412 (quotation marks omitted); *see also*

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II. The July 2014 Rule Is Inconsistent With The Plain Language Of The Orphan Drug Exclusion

HHS contends that, even if its July 2014 Rule is final agency action, the rule survives judicial review because the Court must conduct a "highly deferential" review that "presumes the agency's action [is] valid." HHS Mot. 17 (quotation marks omitted). Not so. The agency's interpretation of § 256b(e) violates the statutory text and is owed no deference under either *Chevron* or *Skidmore*. "We need not grapple here with the difficult question of the deference due an agency view . . . [because] 'no deference is due to agency interpretations at odds with the plain language of the statute itself.'" *John Hancock Mut. Life Ins. Co. v. Harris Trust & Sav. Bank*, 510 U.S. 86, 109 (1993) (quoting *Pub. Emps. Ret. Sys. of Ohio v. Betts*, 492 U.S. 158, 171 (1989)). The Court, therefore, can ignore HHS's "pleas for the deference described in *Skidmore* or *Chevron*." *Id.*

And even if § 256b(e) were ambiguous (which it is not), and even if *Skidmore* did apply (which it does not), the Court may defer to the agency's interpretation "only to the extent [it has] the 'power to persuade.'" *Christensen v. Harris Cnty.*, 529 U.S. 576, 587 (2000) (quoting *Skidmore v. Swift & Co.*, 323 U.S. 134, 140 (1944)). HHS's interpretation is not merely unpersuasive; it is plainly wrong.

A. The Text Of § 256b(e) Does Not Contain A Use-Based Restriction

The starting point in any question of statutory construction is, of course, the text. *See, e.g., Barnhart v. Sigmon Coal Co.*, 534 U.S. 438, 450 (2002). The orphan drug exclusion provides:

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CropLife Am. v. EPA, 329 F.3d 876, 884 (D.C. Cir. 2003) (reviewing a pre-enforcement challenge to agency action, in part, because the issue presented was "a purely legal question that does not depend upon consideration of particularized facts" (quotation marks omitted)).

> EXCLUSION OF ORPHAN DRUGS FOR CERTAIN COVERED ENTITIES—For [newly] covered entities . . . , the term "covered outpatient drug" shall not include a drug designated by the Secretary under [21 U.S.C. § 360bb] for a rare disease or condition.

42 U.S.C. § 256b(e). This language is clear. If a drug is "designated" as orphan under § 360bb, it is excluded from 340B pricing when sold to a newly covered entity. But if a drug is not "designated" as orphan, it is not excluded. *Designation* is the gatekeeper; *use* is irrelevant.

The use-based limit set out in the July 2014 Rule rewrites the plain language of § 256b(e). As rewritten by HHS, the orphan drug exclusion would provide:

> EXCLUSION OF ORPHAN DRUGS FOR CERTAIN COVERED ENTITIES *WHEN USED TO TREAT RARE DISEASES OR CONDITIONS*—For [newly] covered entities . . . , the term "covered outpatient drug" shall not include a drug designated by the Secretary under [21 U.S.C. § 360bb] for a rare disease or condition *and used to treat such rare disease or condition*.

(emphasized words added). HHS's interpretation injects into the statute a use-based limit that Congress did not include. According to the statute, a drug is exempt from 340B pricing if it is designated under § 360bb. But according to the July 2014 Rule, a drug is exempt only if it is *both* designated under § 360bb *and* used to treat an orphan disease or condition. The rule does not "interpret" the law; it changes it. Even HHS's amicus the American Hospital Association ("AHA") seems to recognize that the use-based limit is something the agency—not Congress—added to the statute, urging this Court to uphold "*[t]he Secretary's limitation* of the orphan drug exclusion." AHA Br. 8 (Feb. 9, 2015), ECF No. 19 (emphasis added).

If there were any doubt about whether § 256b(e) includes a use-based limit—and there is not—its all-caps section heading would dispel that doubt. Congress entitled § 256b(e) "EXCLUSION OF *ORPHAN DRUGS* FOR CERTAIN COVERED ENTITIES." (emphasis added). That heading, like the text of the provision itself, does not mention or even suggest that the exclusion applies only to certain uses or indications of orphan drugs. *See Fla. Dep't of*

Revenue v. Piccadilly Cafeterias, Inc., 554 U.S. 33, 47 (2008) ("[S]tatutory titles and section headings 'are tools available for the resolution of a doubt about the meaning of a statute.'" (quoting *Porter v. Nussle*, 534 U.S. 516, 528 (2002))). The plain text of the statute compels rejection of HHS's use-based approach.

B. The Scope Of The Orphan Drug Exclusion Is Tied To The Drug-Specific *Designation* Process, Not The Use-Specific *Approval* Process

Congress defined the orphan drug exclusion by reference to the orphan designation provision, 21 U.S.C. § 360bb. That cross-reference is significant. As FDA has explained, "[o]rphan drug designation is conferred to the *active moiety*"—that is, to the drug itself, not to certain uses of the drug. FDA, Developing Products for Rare Diseases and Conditions, Frequently Asked Questions (FAQ), http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/ucm240819.htm (last visited Feb. 24, 2015). Thus, when the Secretary of HHS designates a drug under § 360bb, the entire drug receives the orphan designation and falls within the scope of the orphan drug exclusion.

The drug-specific designation process of § 360bb that Congress cited in the orphan drug exclusion is distinct from the *approval* process of 21 U.S.C. § 360cc, which is "disease-specific" and occurs *after* a drug receives an orphan designation. *Sigma-Tau Pharm., Inc. v. Schwetz*, 288 F.3d 141, 145 (4th Cir. 2002); *see also Baker Norton Pharm., Inc. v. U.S. Food & Drug Admin.*, 132 F. Supp. 2d 30, 32 (D.D.C. 2001) ("[T]he drug is designated as an orphan drug before it is approved."). And, unlike designation under § 360bb, approval under § 360cc "*is* limited to the indication(s) or use(s) for which the designated drug is approved." 78 Fed. Reg. 35117, 35118 (June 12, 2013) (emphasis added); *see also id.* at 35134. Had Congress intended to limit the scope of the orphan drug exclusion to only those orphan drugs that are both designated and used

to treat rare diseases or conditions, it would have cross-referenced the statutory provision that already encompasses both requirements: § 360cc. Congress, however, cited the drug-specific designation provision of § 360bb and made no mention of orphan drug "use," orphan drug "indication," or § 360cc.[3]

C. Congress Expressly Limits Orphan Drug Provisions When That Is Its Intent

Congress knows how to add use-based limits to orphan drug provisions. It has done so many times, and its decision not to do so in § 256b(e) further underscores that Congress did not intend the use-based limit that HHS adopts in the July 2014 Rule.

One example of a provision containing such use-based language is Section 9008 of the Affordable Care Act—the same statute in which Congress enacted the orphan drug exclusion. Section 9008 imposes on drug manufacturers an annual fee based on a percentage of "branded prescription drug sales." Pub. L. No. 111-148, § 9008(b) (codified at 26 U.S.C. § 4001). Similar to the orphan drug exclusion, Congress provided in Section 9008 that "orphan drug sales" are exempt from the "branded prescription drug sales" calculation. *Id.* § 9008(e)(3). Unlike the orphan drug exclusion, however, Congress added a use-based restriction to Section 9008: the exemption from the annual fee for orphan drugs "shall not apply" to orphan drugs that are "approved . . . for marketing for *any indication other than the treatment of the rare disease or condition*." *Id.* (emphasis added).

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[3] Amicus SNHPA grounds much of its argument on the flawed premise that § 360bb and § 360cc "do[] *not* create a two-part scheme in which designation as an orphan 'drug' is wholly separate from the orphan uses." SNHPA Br. 13 (Feb. 9, 2015), ECF No. 20 (emphasis added). But creating a distinct two-part scheme is *exactly* what the two provisions do, as even HHS recognizes: "The award of orphan-drug designation does not alter the standard regulatory requirements and process for obtaining marketing approval for the drug, *which is a separate process*." HHS Mot. 7 (emphasis added); *see also* AR 666 (78 Fed. Reg. at 44017). This Court also has described the "two-step process" by which a manufacturer first obtains an orphan drug designation and then seeks "full FDA approval of its drug." *Genentech, Inc. v. Bowen*, 676 F. Supp. 301, 303-04 (D.D.C. 1987); *see also Baker Norton Pharm.*, 132 F. Supp. 2d at 32 ("Once the drug is designated as an orphan drug, it goes through the approval process . . . under 21 U.S.C. § 360cc.").

When a "limitation appearing in one part of a statute is not present in another, its absence creates a negative implication—that no limitation was intended." *NetCoalition v. S.E.C.*, 715 F.3d 342, 350 (D.C. Cir. 2013) (quotation marks omitted). Thus, when Congress added a use-based limit to one orphan drug provision of the Affordable Care Act (Section 9008), but omitted such a limit from another orphan drug provision of the same Act (§ 256b(e)), "it is generally presumed that Congress act[ed] intentionally and purposely in the disparate inclusion or exclusion." *Barnhart*, 534 U.S. at 452 (quotation marks omitted); *see also Loughrin v. United States*, 134 S. Ct. 2384, 2390 (2014) ("[W]hen Congress includes particular language in one section of a statute but omits it in another . . . this Court presumes that Congress intended a difference in meaning." (quotation marks omitted)). Where Congress wanted to add a use-based limit, "it did so explicitly." *Barnhart*, 534 U.S. at 452-53; *accord Sebelius v. Cloer*, 133 S. Ct. 1886, 1894 (2013).

The express use-based limit in Section 9008 is not an outlier. Congress has included similar limits in other statutory provisions relating to orphan drugs. For example:

- The Medicare statute permits a special "pass-through" payment for orphan drugs only when the drugs are "*used for a rare disease or condition*." 42 U.S.C. § 1395l(t)(6)(A)(i) (emphasis added).

- The Federal Food, Drug, and Cosmetic Act exempts from certain drug application fees "a prescription drug product that has been designated as a drug for a rare disease or condition pursuant to [21 U.S.C. § 360bb] . . . *unless the human drug application includes an indication for other than a rare disease or condition*." 21 U.S.C. § 379h(a)(1)(F) (emphasis added).

- The Internal Revenue Code provides a tax credit for clinical testing of orphan drugs "*only to the extent such testing is related to the use of a drug for the rare disease or condition for which it was designated*." 26 U.S.C. § 45C(b)(2)(B) (emphasis added).

Congress clearly knows how to add use- and indication-specific limits to orphan drug provisions, and its decision *not* to do so in § 256b(e) "is compelling evidence that Congress did

not intend to limit" the orphan drug exclusion in such a way. *D.C. Hosp. Ass'n v. District of Columbia*, 224 F.3d 776, 780 (D.C. Cir. 2000); *see also Dep't of Homeland Sec. v. MacLean*, 135 S. Ct. 913, 920-21 (2015) (reasoning that a statute did not distinguish "between different types of regulations" because "Congress knew how to distinguish between regulations" and had done so in "another federal statute . . . , but chose not to do so" in the statute at issue).

Ignoring these principles of statutory construction, HHS contends that the agency's interpretation is "consistent with the general statutory and regulatory treatment of the incentives for orphan-designated drugs" because "each incentive . . . applies only to the orphan indication." HHS Mot. 20. In other words, HHS argues that, because Congress expressly added use-based limits to other orphan drug provisions, Congress must have intended the same use-based limit for § 256b(e) even though § 256b(e), unlike the other provisions, says nothing about "use" or "indication." The AHA similarly argues in its amicus brief that § 256b(e) contains a use-based limit because other statutory provisions relating to orphan drugs "are replete with references to a drug's actual or intended use." AHA Br. 16.

HHS and AHA get it exactly backwards. Congress's decision to add limiting language to some orphan drug provisions but to omit such language from § 256b(e) is evidence that Congress intended § 256b(e) to have a *different* meaning. *See, e.g., Dole Food Co. v. Patrickson*, 538 U.S. 468, 476 (2003) (holding that a statute did not encompass "ownership in other than the formal sense" because "[v]arious federal statutes refer to 'direct and indirect ownership,'" but the statute at issue contained no similar language); *F.C.C. v. NextWave Pers. Commc'ns Inc.*, 537 U.S. 293, 302 (2003) (refusing to find a regulatory exception to a provision of the Bankruptcy Code because the provision did not contain the "clear[] and express[]" language Congress frequently

uses to create exceptions to bankruptcy law requirements); *accord MacLean*, 135 S. Ct. at 919-21; *Loughrin*, 134 S. Ct. at 2390; *Barnhart*, 534 U.S. at 452-53.

HHS also seeks refuge in the statutory phrase "for a rare disease or condition," which, according to HHS, "defines and delimits" the word "designation." HHS Mot. 20. But that argument ignores that other orphan drug provisions *also* contain the phrase "for a rare disease or condition" *and also* include express use-based limiting language. Consider, for example, the drug application fee exemption in 21 U.S.C. § 379h(a)(1)(F). That provision states: "A human drug application for a prescription drug product that has been designated as a drug *for a rare disease or condition* pursuant to section 360bb of this title shall not be subject to a fee under subparagraph (A), *unless the human drug application includes an indication for other than a rare disease or condition*." (emphasis added). If the first clause of that provision already was use-specific because it, like § 256b(e), includes the phrase "for a rare disease or condition," then the express indication-based language in the second clause would be superfluous. HHS's interpretation thus violates the cardinal principle of statutory construction that courts must "give effect, if possible, to every clause and word of a statute." *Duncan v. Walker*, 533 U.S. 167, 174 (2001) (quotation marks omitted); *cf. Smith v. City of Jackson, Miss.*, 544 U.S. 228, 233 (2005) ("[W]hen Congress uses the same language in two statutes having similar purposes, . . . it is appropriate to presume that Congress intended that text to have the same meaning in both statutes.").[4]

[4] HHS's amici advance a similarly flawed argument by relying on a 1982 House Committee Report for the Orphan Drug Act, which refers to "designating the use of the drug which is for a rare disease or condition." *See* SNHPA Br. 17; AHA Br. 17. That reference, however, appears in the context of explaining the process for determining whether a disease or condition is "rare." H.R. Rep. No. 97-840, at 9 (1982), *reprinted in* 1982 U.S.C.C.A.N. 3577, 3581. The reference does not mean—or even suggest—that an orphan designation under 21 U.S.C. § 360bb applies only to certain uses or indications of the drug.

D. The Legislative Record Confirms That HHS's Use-Based Limit Violates Congressional Intent

Even if this Court concludes that the text of § 256b(e) is ambiguous and thus turns to legislative history for guidance, that history would reinforce that Congress did not intend the use-based limit HHS has imposed in the July 2014 Rule.

Congress enacted the orphan drug exclusion in March 2010. *See* Pub. L. No. 111-152, § 2302. In December 2010, *the same 111th Congress* revisited the exclusion when considering and passing the Medicare and Medicaid Extenders Act of 2010, Pub. L. No. 111-309 ("MMEA"). At the request of covered entities and their trade associations, *see, e.g.*, AR 009, 011, 013, 016, 104, and with technical assistance from HHS, *see* AR 071, Congress included in the MMEA a provision that amended § 256b(e) by removing children's hospitals from the list of newly covered entities to which the exclusion applies, *see* MMEA § 204. Congress, however, did not amend § 256b(e) in any other way even though the covered entities and their trade associations also had lobbied Congress to repeal the exclusion or limit it to when an orphan drug is used for an orphan indication. *See, e.g.*, AR 058 ("We are still working on the solution with the Hill."); *id.* at 087 (letter from Westfields Hospital to Senator Kohl); *see also id.* at 85, 103, 109, 191.

Congress's decision in the MMEA to amend one aspect of the orphan drug exclusion yet leave the rest unchanged corroborates that Congress intended to preserve the unlimited exclusion it originally enacted. The Supreme Court drew a similar inference in *Kucana v. Holder*, 558 U.S. 233, 250 (2010), where the Court reasoned that Congress's decision to amend one aspect of the Immigration and Nationality Act without changing another provision in that Act reflected Congress's intent to "le[ave] the matter where it was." Likewise, in *International Union, United Mine Workers of America v. Mine Safety and Health Administration*, 823 F.2d 608, 619 (D.C.

Cir. 1987), the D.C. Circuit held that a statute did not authorize a type of temporary relief because, among other reasons, "Congress considered proposals which would have authorized the [agency] to grant temporary relief . . . and rejected them." And in *Miller v. Kerry*, 924 F. Supp. 2d 133, 140 (D.D.C. 2013), this Court reasoned that Congress did not waive sovereign immunity for compensatory damages under the Age Discrimination in Employment Act of 1967 ("ADEA") because, when "Congress amended Title VII in 1991 to allow for compensatory damages, it could have decided to do the same for the ADEA, but it did not."

Another illustrative example is *Gross v. FBL Financial Services, Inc.*, 557 U.S. 167 (2009). That case involved whether the ADEA allows plaintiffs to establish discrimination by showing that age was a "motivating factor" in an adverse employment action. To answer that question, the Court looked to another statute, Title VII of the Civil Rights Act of 1964, which also prohibits age discrimination. *See id.* at 174. The Court observed that Congress had amended Title VII to include the "motivating factor" standard, but Congress had "neglected to add such a provision to the ADEA." *Id.* By revising one statute to include the "motivating factor" standard and yet not revising the ADEA also to include that standard, Congress signaled its intent that ADEA plaintiffs should *not* be allowed to establish a prima facie case by using the "motivating factor" standard. *See id.* at 174-75, 177-78. "When Congress amends one statutory provision but not another, it is presumed to have acted intentionally." *Id.* at 174.

The evidence of congressional intent is even more powerful here. In *Gross*, the Court presumed that Congress had acted intentionally in amending the language of one statute (Title VII) but leaving unchanged the language of another separate statute (the ADEA). This case involves one statute, one provision of that statute, and only one Congress—the same one that enacted § 256b(e) in the first place. In enacting the MMEA, Congress unquestionably heard

complaints that the orphan drug exclusion applies to all orphan drugs, but it chose to amend the exclusion without adding the use-based limit it has included in other orphan drug provisions, that covered entities had requested, and that HHS has now adopted in the July 2014 Rule.

E. The Policy Concerns Of HHS And Its Amici Cannot Trump The Policy Choice Of Congress

The remaining arguments of HHS and its amici are rooted in what they perceive to be the goals of the 340B Program, the Affordable Care Act, and the Orphan Drug Act. HHS argues that its use-based interpretation "reasonably balances the goal of the 340B Program . . . with that of the Orphan Drug Act." HHS Mot. 24. SNHPA asserts that the July 2014 Rule "reasonably balances" the goals of the Affordable Care Act with the goals of the Orphan Drug Act. SNHPA Br. 20. And AHA presses that the rule "appropriately balances the interests in incentivizing orphan drug development with the interests in providing 340B discounts" to newly covered entities. AHA Br. 5 (emphasis omitted).

But § 256b(e) *already* "reasonably" and "appropriately" balances these various policy objectives. In that provision, Congress offset the Affordable Care Act's expansion of the types of healthcare providers eligible to participate in the 340B Program by providing that 340B pricing does not apply to orphan drugs sold to those newly covered entities. That policy choice might affect a program benefit for newly covered entities, but it preserves important incentives for manufacturers to research and develop new orphan drugs—a policy goal that Congress has endorsed for decades. *See* Orphan Drug Act, Pub. L. No. 97-414, § 1(b)(6) (1983) ("[I]t is in the public interest to provide . . . incentives for the development of orphan drugs."). HHS might disagree with the balance Congress reached in § 256b(e), but the agency "may not disregard the Congressional intent clearly expressed in [statutory] text simply by asserting that [its] preferred approach would be better policy." *Sierra Club v. E.P.A.*, 294 F.3d 155, 161 (D.C. Cir. 2002)

(quotation marks omitted); *see also Barnhart*, 534 U.S. at 462 ("We will not alter the text in order to satisfy the policy preferences of the [agency].").

HHS and its amici also severely exaggerate the impact of § 256b(e). The orphan drug exclusion does not affect covered entities that qualified for the 340B Program before Congress passed the Affordable Care Act: they all still receive 340B prices on *all* orphan drugs. And newly eligible covered entities receive 340B prices on all but a small subset of drugs (orphan drugs). Limiting the availability of 340B pricing for a subset of covered entities on a subset of drugs hardly "destroy[s]" the benefits of the 340B Program or causes "patently absurd consequences," as HHS hypothesizes. HHS Mot. 25 (quoting *United States v. Menasche*, 348 U.S. 528, 538 (1995), and *United States v. Brown*, 333 U.S. 18, 23 (1948)). Nor does applying the statute as-written "nullify" incentives for participating in the program, *id.*, as even SNHPA's made-for-litigation survey confirms, *see, e.g.*, AR 727 (92.9% of newly covered entities would register for the 340B Program even if they "would never have access to 340B discounts on orphan drugs"); *id.* (only 3 out of 70 newly covered entities would be "very likely" to withdraw from the 340B Program if they cannot purchase orphan drugs through the program).[5]

But these policy questions are ultimately a distraction. Congress determined in the Affordable Care Act—and re-affirmed in the MMEA—that § 256b(e) should apply to all orphan drugs regardless of how they are used. Because the July 2014 Rule is inconsistent with that congressional determination, the rule is "arbitrary, capricious, an abuse of discretion, or otherwise not in accordance with law," 5 U.S.C. § 706(2)(A), and should be set aside.

[5] AHA and SNHPA also rely on a letter from Representative Waxman and Senator Harkin (AHA Br. 17-18; SNHPA Br. 17-18), but that letter rests on a legal fiction—namely, that "[a] designation under [§ 360bb] consists of two components: the drug itself and the indication for which it is designated." AR 335. As noted above, and as FDA has explained, an orphan designation attaches to the drug as a whole, not to any particular use of the drug. *See supra* Part II.B.

CONCLUSION

For the reasons explained, the Court should deny HHS's motion for summary judgment, grant PhRMA's motion for summary judgment, and invalidate and vacate the July 2014 Rule.

Respectfully submitted,

/s/ Jeffrey L. Handwerker

JEFFREY L. HANDWERKER (#451913)
MATTHEW T. FORNATARO
KRISTIN M. HICKS
BRANDON L. BOXLER

ARNOLD & PORTER LLP
555 Twelfth Street, NW
Washington, DC 20004-1206
+1 202.942.5000
+1 202.942.5999

Counsel for Pharmaceutical Research and Manufacturers of America

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