340B ORPHAN DRUG INTERPRETIVE RULE STRUCK DOWN BY D.C. DISTRICT COURT: HHS AND HRSA LOSE IN SECOND ROUND OF LITIGATION OVER 340B ORPHAN DRUG RULES

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Health Care Alert

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In an Opinion issued October 14, 2015, D.C. District Court Judge Rudolph Contreras granted Pharmaceutical Research and Manufacturers of America's ("PhRMA") motion for summary judgment against the U.S. Department of Health and Human Services ("HHS") (and, by extension, the Health Resources and Services Administration ("HRSA")).[1] The court held that HRSA's recently issued "interpretive rule"[2] was contrary to the language of the 340B statute[3] when it limited the "orphan drugs" exempt from the 340B program to drugs "used" for their orphan-designated purposes, instead of exempting all drugs "designated" as an orphan drug under the Orphan Drug Act.[4]

Orphan drugs are drugs developed to treat a rare disease or condition—often referred to as an orphan disease. Prior to seeking Food and Drug Administration ("FDA") approval of a drug, manufacturers can request that FDA "designate" their drug as a drug for the treatment of an orphan disease.[5] If a drug that has been granted such an orphan designation is ultimately approved by FDA for the designated use, the drug's manufacturer will be entitled to seven-years of market exclusivity under the Orphan Drug Act.[6] The FDA can, and often does, approve orphan-designated drugs for uses other than the orphan use. Additionally, physicians frequently prescribe orphan-designated drugs "off-label" for non FDA-approved uses.

The 340B Drug Discount Program is a federal program operated by HRSA that requires drug manufacturers to provide outpatient pharmaceuticals to eligible covered entities at reduced prices. For certain covered entities, however, the 340B discounts do not apply to drugs designated as orphan drugs. Through an interpretive rule, HRSA took the position that 340B discounts on orphan drugs *would* be available to those covered entities if a drug was being prescribed for a non-orphan disease use, even if the drug had an orphan drug designation.

The impact of this decision is that the orphan drug interpretive rule is invalid and all drugs designated as orphan drugs under standards established by the FDA are exempt from receiving 340B pricing as to 340B-covered entities subject to the orphan drug exclusion, regardless of the "use" for which the drug is prescribed. This is the second defeat for HHS on the orphan drug issue. In May 2014, the same court held that HRSA did not have rulemaking authority to promulgate regulations regarding how the 340B statute applies to orphan drugs.[7] It also comes at a time when HHS has recently proposed a separate 340B "Mega Guidance," portions of which may be subject to a similar challenge.[8]

BACKGROUND ON ORPHAN DRUG RULE AND PHRMA'S CHALLENGE

As part of the Patient Protection and Affordable Care Act ("ACA"), Congress added several new categories of covered entities to the 340B program: children's hospitals that are excluded from the Medicare prospective payment system, free-standing cancer hospitals that are excluded from the Medicare prospective payment system, critical access hospitals, rural referral centers, and sole community hospitals.[9] However, in the Health Care and Education Reconciliation Act—which amended the ACA—Congress then excluded from the term "covered outpatient drug" those drugs "designated by the Secretary under section 526 of the Federal Food, Drug, and Cosmetic Act for a rare disease or condition" for only the newly qualifying covered entities above.[10] By further legislative action, Congress then clarified that children's hospitals, which previously had been eligible to participate in the 340B program, were not subject to the orphan drug exclusion.[11]

The orphan drug interpretive rule was originally issued as a legislative rule promulgated under the notice and comment provisions of the federal Administrative Procedure Act ("APA").[12] Under both the legislative rule and the interpretive rule, HRSA proposed to interpret the orphan drug exclusion to only apply to orphan drugs that are transferred, prescribed, sold, or otherwise "*used for the rare condition or disease for which that orphan drug was designated*" under section 526 of the Federal Food, Drug, and Cosmetics Act.

However, PhRMA challenged HHS's authority under the APA to broadly promulgate rules implementing the 340B statute —and regarding the orphan drug provisions of 340B in particular. In May 2014, the D.C. District Court held that HHS did not have broad rulemaking authority, but instead only had rulemaking authority as to three separate and discrete areas, which did not include the orphan drug provisions.[13] However, the court left open the possibility that the orphan drug rule may have been permissible as an interpretive rule, which would not require a specific grant of rulemaking authority. The court requested additional briefing by the parties on this issue.

HHS instead withdrew the proposed regulation, but then reissued substantively the same requirements as an interpretive rule. PhRMA again challenged HHS, bringing the suit at issue and arguing that the interpretive rule violated the language of the 340B statute. The court ultimately found that PhRMA would need to satisfy—and did satisfy—two elements to prevail. First, the interpretive rule would need to functionally be a "final agency action" in order for the rule to be subject to judicial review. If that element was satisfied, then the court would decide whether the interpretive rule was a valid interpretation of the 340B statute.

WHEN IS AN INTERPRETIVE RULE OR GUIDANCE A FINAL AGENCY ACTION?

The court articulated a two-part test for assessing when agency action qualifies as a final action sufficient to provide a plaintiff with the right to seek judicial review. First, the rule must be the consummation of the agency's deliberate decision-making process. The court was able to quickly move past this issue, as the agency conceded this point.

Second, the agency action must be one in which the plaintiff's rights or obligations have been determined or from which legal consequences will flow. This second element was contested by the parties. The court relied on the D.C. Circuit Court's factors in CSI Aviation Services, Inc. v. United States Department of Transportation[14] to find that the interpretive rule met this second test. Specifically, the court found:

- HHS had taken a definitive legal position in its guidance materials and enforcement letters;
- The case was one of pure legal interpretation, which did not require the development of a factual record; and
- The action imposed immediate and significant practical burdens on the plaintiff.

HHS attempted to argue that the court should only consider whether the rule had "legal effects" instead of "practical effects," but the court rejected this argument.[15]

WAS THE INTERPRETIVE RULE A VALID INTERPRETATION OF THE 340B STATUTE?

Finding that the matter was properly subject to judicial review, the court next considered whether the rule was a valid interpretation of the 340B statute. Regardless of the level of deference afforded to an agency's rule, the court notes that the predicate question is whether or not the statutory language is ambiguous. If it is ambiguous, an agency with rulemaking authority is afforded "Chevron Step-2" deference, where any reasonable interpretation is held to be valid, even if the court would arrive at a different interpretation. However, because HRSA lacked rulemaking authority on this issue, its guidance would only receive lesser "Skidmore deference," whereby the court would only adopt the HHS's interpretation to the extent it is persuasive.

However, the court's decision did not end up turning on the question of deference, as the court found the 340B orphan drug statutory language *unambiguous*. The court considered the plain language of the statute (which specifically references drugs "designated as orphan drugs"), compared the 340B statute to other statutes concerning orphan drugs, and concluded that Congress knew how to include "use" for orphan indications language with orphan drugs when it intended to do so.

As a result, the court held that the 340B statute unambiguously applies to all drugs designated as orphan drugs, regardless of their use. The court acknowledged that the statutory language is perhaps not fully in line with the policy objective of the 340B statute with regard to orphan drugs, but concluded that "Congress's chosen statutory language evidences that it struck a different balance and it is simply not for this Court to rewrite the statute."

IMPACT OF THIS DECISION

The most immediate impact of this decision is to invalidate the orphan drug interpretive rule and exclude all drugs designated as orphan drugs under the Orphan Drug Act from the 340B program for those covered entities added under the ACA, regardless of their use. The HRSA website now indicates, "As of October 14, 2015, the Orphan Drug Interpretive Rule has been vacated. . . . HRSA will provide additional information as it becomes available."[16] Accordingly, covered entities subject to the orphan drug exclusion will likely need to begin complying with the more expansive exclusion now.

HHS may appeal this decision and seek review before the D.C. Circuit Court of Appeal. However, barring reversal by the D.C. Circuit Court, this decision also establishes that it will take an act of Congress to rewrite the 340B statute to permit the covered entities added under the ACA to purchase orphan drugs at the 340B price for non-orphan uses. Whether such a change might occur is uncertain. There are conflicting political decision points

at issue, including the increasing costs of prescription drugs, incentives to develop orphan drugs, the appropriate level of government agency oversight, and the competing interests of pharmaceutical companies and health care providers regarding the 340B program.
Finally, this second defeat of HRSA could also serve to embolden parties seeking to challenge the separately issued 340B "Mega Guidance," if and when it is finalized.

Notes:

[1] PhRMA v. HHS, ___ F. Supp. 3d ___, 2015 WL 5996374 (D.D.C. Oct. 14, 2015).

[2] See 79 Fed. Reg. 42,801 (July 23, 2014).

[3] 42 U.S.C. § 256b.

[4] 21 U.S.C. § 360bb.

[5] *Id.*

[6] 21 U.S.C. § 360cc.

[7] Pharmaceutical Research and Manufacturers of America v. HHS, 43 F. Supp. 3d 28 (D.D.C. 2014).

[8] For a discussion of the impact of the 340B "Mega Guidance," see our prior September 3, 2015, health care alert titled "HRSA Issues 340B Program Omnibus Guidance," available at http://www.klgates.com/hrsa-issues-340b-program-omnibus-guidance-09-03-2015/.

[9] *See* Patient Protection and Affordable Care Act, Pub. L. No. 111–148, § 7101(a), 124 Stat. 119, 821–22 (codified as amended at 42 U.S.C. § 256b(a)(4)(M)–(O)).

[10] Health Care and Education Reconciliation Act, Pub. L. No. 111-152, § 2303, 124 Stat. 1029, 1083 (codified as amended at 42 U.S.C. § 256b(e)).

[11] Medicare and Medicaid Extenders Act of 2010, Pub. L. No. 111-309, § 204, 124 Stat. 3285, 3289–90 (codified as amended at 42 U.S.C. § 256b(e)).

[12] Exclusion of Orphan Drugs for Certain Covered Entities Under 340B Program, 78 Fed. Reg. 44,016 (July 23, 2013).

[13] The three areas of the 340B statute HHS that has rulemaking authority are: (i) promulgating rules governing the establishment of an administrative dispute resolution process, (ii) issuance of drug ceiling price methodologies, and (iii) the imposition of civil monetary sanctions.

[14] 637 F.3d 408 (D.C. Cir. 2011).

[15] By way of dicta, the Court further noted that the impact on PhRMA arguably involved both practical and legal effects.

[16]HRSA, *Orphan Drugs Exclusion*, http://www.hrsa.gov/opa/programrequirements/orphandrugexclusion/index.html (last visited Oct. 21, 2015).

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