DC District Court Strikes Down 340B Orphan Drug Rule

Written by Thomas Barker

June 5, 2014

On May 23, Judge Contreras of the U.S. District Court for the District of Columbia (DC District Court) ruled that the Health Resources and Services Administration (HRSA) did not have the statutory authority to promulgate its Orphan Drug Final Rule (case number 1:13-cv-01501; 78 Fed. Reg. 44016 (July 23, 2013)). HRSA's Rule, titled "Exclusion of Orphan Drugs for Certain Covered Entities Under 340B Program," implemented a provision of the Patient Protection and Affordable Care Act (ACA) that excludes orphan drugs from 340B pricing for the covered entities added to the 340B Program by the ACA. Under the Final Rule, orphan drugs were excluded from the 340B ceiling price only when the drugs were used for the indication for which they were granted the orphan designation.

The Court reviewed the rulemaking authority granted to HRSA under section 340B of the Public Health Service Act (PHSA), and concluded that this authority was very narrow. Absent the broad directive to "carry out" the section, the authority to issue regulations under 340B exists only with respect to limited provisions of the statute. The orphan drug rulemaking was not promulgated under any of these provisions, however, and therefore lacked statutory authority. Accordingly, Judge Contreras granted the plaintiff, Pharmaceutical Research and Manufacturers of America (PhRMA), a permanent injunction blocking the rule and a summary judgment.

Judge Contreras's opinion raises questions regarding the future of the orphan drug exclusion provision and also HRSA's upcoming "mega-rule" that the agency had planned to be published for comments in June 2014. The "mega-rule" was reportedly intended to address four topics: the definition of an eligible patient, compliance requirements for contract pharmacy arrangements, hospital eligibility criteria, and eligibility of off-site facilities. Since these topics are not directly related to HRSA's explicit rulemaking authority in section 340B, one option that HRSA may consider (in lieu of withdrawing the rule in its entirety) is to clarify that the "mega-rule" is in fact a "mega-interpretive-rule," and could convert the rule into a notice.

The 340B Program and the Orphan Drug Rulemaking

The 340B Program, created in 1992, requires manufacturers who participate in the Medicaid program to provide covered outpatient drugs at lower prices to certain safety-net healthcare providers (referred to as "covered entities"). The ACA amended section 340B in several important ways, the most significant of which was the addition of several categories to the covered entities list, including some children's hospitals, free-standing cancer hospitals, critical access hospitals, some rural referral centers, and some sole community hospitals. The ACA also provided HRSA, for the first time, explicit authority to issue regulations to implement certain provisions of the section.

With regard to drugs for rare diseases, the ACA included a provision specifying that, for the new list of covered entities added by the ACA, "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" (§ 340B(e)). In other words, under the provision, manufacturers do not have to provide orphan-designated drugs to some covered entities at the 340B ceiling price.

In 2011, HRSA proposed to interpret the ACA's orphan drug exclusion as only excluding orphan drugs when the drugs are used for the indications for which the orphan designation was granted. After receiving and replying to comments, HRSA finalized its interpretation in the 2013 Final Rule (78 Fed. Reg. 44016 at 44027):

> (a) General. For the covered entities described in paragraph (b) of this section, a covered outpatient drug does not include 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 FFDCA. A covered outpatient drug includes drugs that are designated under section 526 of the

FFDCA when they are transferred, prescribed, sold, or otherwise used for any medically-accepted indication other than treating the rare disease or condition for which the drug was designated under section 526 of the FFDCA.

This interpretation raised at least two objections from drug manufacturers: first, the interpretation required manufacturers to provide a 340B discount for drugs which have been granted an orphan designation, thus contradicting the statutory language of 340B(e); and second, the initial determination whether a drug is used for the rare indication was left to the covered entities, which directly benefit from determining that an orphan drug is not used for the rare indication for which it received an orphan designation.

DC District Court's Opinion in *PhRMA v. HHS*

Unable to affect the outcome of HRSA's rulemaking, drug manufacturers challenged HRSA in court, filing a complaint with the DC District Court in September 2013. The complaint asserted that HRSA lacked the authority to promulgate rules interpreting the orphan drug exclusion. HRSA retorted that the rulemaking was authorized by HRSA's statutory authority to promulgate regulations to implement an administrative dispute resolution mechanism, because the scope of the orphan drug exclusion provision would inevitably be raised as a dispute. The Court disagreed with HRSA however, concluding that HRSA was not authorized to issue "prophylactic," non-adjudicatory rulemaking. The Court noted that the statute enumerates the specific elements of the dispute resolution mechanism to be included in HRSA's rulemaking, such as designating a decision-making body and establishing deadlines for claims, without mentioning a non-adjudicatory approach. Therefore, the Court ultimately sided with PhRMA, granting a permanent injunction and a summary judgment in plaintiff's favor.

The Court noted that the statute does not grant HRSA a general authority to "carry out" section 340B, and that there are only three provisions in section 340B that authorize HRSA to promulgate *any* rulemaking: (1) implementation of an administrative dispute resolution mechanism for claims by manufacturers and covered entities, (2) regulatory issuance of standards and methodology for the calculation of ceiling prices, and (3) establishment of standards for the imposition of civil monetary penalties applicable to participating manufacturers.

HRSA also argued that the Orphan Drug Final Rule was an interpretive, as opposed to a legislative, rule. After reviewing the test to determine whether a rule is interpretive or legislative, the Court concluded that the rulemaking was in fact legislative because the rule (1) underwent notice and comment rulemaking and (2) had "legal effect" on the parties by imposing obligations on covered entities and manufacturers. The Court noted that interpretive rules are analyzed under a standard that is far less deferential to the government.

In sum, the Court's opinion re-casted HRSA's authority in administering the 340B Program as follows:

- HRSA is only authorized to issue rulemaking in implementing the three statutory provisions noted above. HRSA is not authorized to issue legislative rules to implement any other provisions of section 340B.
- In implementing the rulemaking statutory provisions, HRSA is not authorized to promulgate "prophylactic" rulemaking. In other words, the agency's rulemaking must follow the form prescribed by the statute.

Next Steps for the Orphan Drug Exclusion

The DC District Court's permanent injunction of the enforcement of HRSA's Orphan Drug Rule brings the parties back to the statutory provision that spurred the rulemaking in the first place. Under the Court's injunction of HRSA's rulemaking, manufacturers of covered outpatient drugs can refuse to grant 340B pricing to newly-added covered entities for drugs that were granted orphan designation, regardless of the indication for which the drugs are used.

Following a defeat in court, HRSA has several avenues going forward. One option was proposed by Judge Contreras, who suggested that HRSA promulgate the rulemaking as an interpretive rule. However, it is not clear how recasting the same rulemaking as an interpretive rule and repeating the same argument would change the opinion of the Court. Importantly, the Court did not address at all the extent of deference to which HRSA's interpretation of the underlying statute is entitled. But even so, unless the interpretation of the statutory exclusion is amended to better comport with the language of the statute, the "interpretive rule" would continue to have a "legal effect" on the parties, and therefore be seen as an impermissible legislative rule.

A second option for HRSA is to file an appeal to the U.S. Court of Appeals for the District of Columbia (DC Circuit). This approach raises the stakes, permitting a more favorable decision but also opening the door to a higher-impact negative decision limiting HRSA's and HHS's authority to implement the other aspects of the 340B Program. Because the agency has already suffered a one-sided setback in court, HRSA may decide that it would be in the long-term interest of the agency to wait for a stronger case to be elevated to the higher courts.

Without further action by HRSA, the issue of orphan drug exclusion is likely to be raised in the administrative dispute resolution process as a joint claim by covered entities against a manufacturer of orphan drugs. The question would then be resolved by the decision-making body appointed by HRSA, and that resolution could also be challenged in court, and may be granted very limited deference or no deference at all (depending on whether it represents the agency's position).

A third option for HRSA is to bring the issue back to Congress and lobby for a narrower orphan drug exclusion provision.

Under each of these options, it is likely that manufacturers and covered entities will continue dueling over all aspects of the 340B Program, including the exclusion of orphan drugs from 340B pricing. If HRSA decides to lobby Congress for greater rulemaking authority, manufacturers are likely to lobby against the proposal.

Future 340B Rulemaking

In addition to invalidating HRSA's Orphan Drug Final Rule, the DC District Court's ruling is also likely to impact the upcoming 340B "mega-rule" planned by HRSA for publication for comments in June 2014. According to HRSA's website, the agency is "working to formalize existing program guidance through regulation, designed to cover a number of aspects of the 340B Program." The upcoming regulation—which began Office of Management and Budget review on April 9—will address four topics, all of which have been the subjects of HRSA's past notices: (1) the definition of an eligible patient, (2) compliance requirements for contract pharmacy arrangements, (3) hospital eligibility criteria, and (4) eligibility of off-site facilities.

It is likely that HRSA will now review the "mega-rule" against the limits that the DC District Court's opinion placed on HRSA's rulemaking authority—a review that would delay the issuance of the rule.

HRSA's first inquiry must be whether the agency has the statutory authority to promulgate regulations addressing the four topics in the "mega-rule." More specifically, do the topics fall under either of section 340B's three rulemaking provisions, namely, administrative dispute resolution, the calculation of ceiling prices, and standards for civil monetary penalties? Based on the DC District Court's and HRSA's previous interpretation of these rulemaking provisions, the answer to this inquiry is negative. Neither of the four topics is directly related to the calculation of the ceiling price for a drug, nor are the topics included in any aspects of the process for civil monetary penalties for participating manufacturers. HRSA could mount an argument that the four topics are instrumental to the administrative dispute resolution mechanism because the topics provide much needed clarity to terms that are not defined in statute. While this is true, the DC District Court ruled that the rulemaking authority to implement an administrative dispute resolution process is limited "to creating a system for resolving disputes between covered entities and manufacturers—not to engaging in prophylactic non-adjudicatory rulemaking regarding the 340B program altogether." It appears therefore that HRSA does not have the statutory authority to promulgate legislative rulemaking addressing the four topics to be included in the "mega-rule."

Without the authority to promulgate rulemaking on these four topics, HRSA could instead implement the changes proposed in the "mega-rule" by converting it into an interpretive rule, such as a notice with guidelines, similarly to HRSA's existing notices. Downgrading the "mega-rule" to a notice would protect the notice from being invalidated by a court on the grounds that HRSA lacked authority to issue the document, but it would also present two weaknesses for HRSA that exist currently with all of its notices: (1) the changes in the notice may not effect substantive regulatory change to the statutory requirements, and (2) if challenged in court, HRSA's interpretation in the notice would only be granted limited deference. Despite these weaknesses, it appears that HRSA does not have the statutory authority to regulate these topics through a more authoritative rulemaking process. Therefore, the industry is likely to see another notice (or interpretive rule) from the agency rather than a legislative rule.

As with the orphan drug exclusion provision, HRSA has the option of requesting additional rulemaking authority from Congress to be included in section 340B, along with the consequent political considerations that would attend such a request. Until then, HRSA must continue administering the Program by guidance rather than rulemaking, with the three exceptions enumerated in the statute.

RELATED INDUSTRIES

- Healthcare
- Life Sciences

---

attorney-client relationship.

United States Treasury Regulations require us to disclose the following: Any tax advice included in this document was not intended or written to be used, and it cannot be used, for the purpose of avoiding penalties under the Internal Revenue Code.