The Coalition
for Government
Procurement

July 19, 2011

CDR Krista Pedley
Director, Office of Pharmacy Affairs (OPA)
Health Systems Bureau (HSB), Health Resources and Services Administration (HRSA)
5600 Fishers Lane
Parklawn Building, Room 10C-03
Rockville, Maryland 20857

Re: Proposed Orphan Drug Rule (RIN 0906-AA94)

Dear CDR Pedley:

On behalf of the Coalition for Government Procurement (CGP) the following comments are submitted in response to the proposed rule (RIN 0906-AA94) concerning the orphan drug exclusion from the definition of covered outpatient drug in the 340B statute established by section 2302 of the Health Care and Education Reconciliation Act of 2010 (HCERA) and section 204(a)(1) of the Medicare and Medicaid Extenders Act of 2010 (MMEA), which the Health Resources and Services Administration (HRSA) of the Department of Health and Human Services published in the Federal Register on May 20, 2011 ('proposed rule"). The proposed rule proposes a regulation to be codified in the Code of Federal Regulations at 42 C.F.R. 10.21

The Coalition for Government Procurement is a non-profit association of approximately 300 firms selling commercial services and products to the Federal Government. Our members collectively account for approximately half the commercial item solutions purchased annually by the Federal Government. Coalition members include small, medium and large businesses concerns and represent many different industries, including both brand and generic pharmaceutical manufacturers. The Coalition is proud to have worked with government officials over the past 30 years towards the mutual goal of common sense acquisition.

Introduction

The 340B program that is the subject of this proposed rulemaking is a purchase program implemented through the terms of standard contracts entered into between HRSA and pharmaceutical manufacturers that choose to participate. These contracts recite the responsibilities imposed by statute on participating manufacturers and HRSA. *See Astra USA, Inc., v. Santa Clara County*, No. 09-1273, slip. op. (U.S. March 29, 2011). In their contracts, manufacturers agree to charge no more than the statutorily specified price for covered outpatient drugs purchased by those health care providers whom Congress determines should benefit from the agreement. Under this statutory scheme set forth in section 340B of the Public Health Service Act, Congress specifies the terms of the contracts, and HRSA's role is to "superintend[]" the program. *Id.* Congress defined "covered outpatient drug" in section 340B by reference to the definition of that term in section 1927 of the Social Security Act, and this statutory provision is incorporated in manufacturers' agreements with HRSA. Congress subsequently amended the

definition of covered outpatient drug in health care reform legislation to exclude drugs designated by the Secretary under section 526 of the Federal Food, Drug, and Cosmetic Act (FFDCA) for a rare disease or condition. This exclusion, however, applies only to those new 340B covered entities added to the program through the same legislation. HRSA's role as administrator of the 340B program includes interpretation of vague statutory language incorporated in manufacturers' 340B contracts. As we discuss below, we believe the proposed rule does not merely interpret the statutory definition, but changes its meaning, which HRSA has no authority to do.

Until this proposed rule, HRSA previously published all interpretations of manufacturers' statutory/contract obligations as guidance or interpretive rules. The difference is significant. As the Federal Circuit pointed out in its decision on our challenge to the VA's interpretation of another Veterans Health Care Act agreement, an interpretative rule simply indicates an agency's reading of a statute, whereas a rule is substantive if it changes existing law and affects individual obligations. *See Coalition for Common Sense in Government Procurement v. Secretary of Veterans Affairs*, 464 F.3d 1306, 1317 (Fed. Cir. 2006). *See also Electronic Privacy Information Center et al. v. Dep't of Homeland Security*, slip op at 10 (D.C. Cir. July 15, 2011) (the distinction turns on whether the regulation effects a substantive change to the statutory scheme). The proposed rule does not purport to be an interpretive rule. Rather, it proposes to establish a new regulation, codified in the Code of Federal Regulations, which would further defines the term "covered outpatient drug" in order to limit the effect of the statute.

In order for HRSA to narrow statutory exclusions and eligibility criteria and thereby enlarge the scope of the 340B statute by binding regulation, the authority to engage in legislative rulemaking must be expressly delegated to it by Congress. The source of that rulemaking authority is in the delegation language of the statute. *See Gonzales v.Oregon*, 126 S. Ct. 904, 916 (2006). Satisfying procedural requirements for legislative rulemaking does not cure the necessity for such a delegation from Congress. In section 7102 of PPACA, Congress directed HRSA to develop and publish by policy or regulation precise standards and a methodology for calculating the ceiling prices specified in section 340B, and to develop various systems and processes for improving administration of the program. However, Congress did not authorize HRSA to promulgate regulations that change the substance of the statutory terms incorporated in the contracts and enlarge the scope of the obligations imposed by Congress. The regulation proposed by HRSA is incompatible with the statutory scheme and exceeds the authority delegated to HRSA.

Proposed C.F.R. Part 10 is, to a large extent, a place holder for future regulations authorized by section 340B, and all of the definitions in proposed 42 C.F.R. 10.3 parrot the statutory definitions set forth in section 340B. However, proposed 42 C.F.R. 10.21 would modify the statutory – and contract - definition of "covered outpatient drug" by adding a new condition to the statutory exclusion for orphan-designated drugs. Further, contrary to the 340B statute, the proposed rule would permit free-standing cancer hospitals to purchase a subset of covered outpatient drugs from GPOs – a clear statutory contradiction that cannot be implemented through regulatory action. The CGP is very concerned that the proposed regulation would impermissibly alter the language of the statute and the terms of manufacturers' contracts to suit a policy decision that, frankly, is not HRSA's to make. Moreover, manufacturers' agree to comply with statutory

provisions through the terms of their agreements, and their agreements do not incorporate any regulatory modifications to those terms. Therefore, we question whether a regulation that is not incorporated in the 340B contract and imposes greater responsibilities than those required by the 340B statute can be binding on the contractors.

The CGP objects to the proposed rule for five reasons.

1. HRSA lacks authority to unilaterally impose on manufacturers by regulation obligations that Congress had excluded from the manufacturers' contracts.

2. The statutory language created the orphan drug exclusion is clear and unambiguous and HRSA has no authority to interpret the statute to alter the exclusion inconsistent with its plain language.

3. The proposed rule changes the statutory program eligibility criteria for free-standing cancer hospitals.

4. The proposed rule lacks standards for application of a purchase exemption based on the subsequent use of the purchased drug by the health care provider, or specifics regarding the type of records that will establish that an orphan-designated drug was purchased for treating a non-orphan disease or condition.

5. Fifth, the proposed rule fails to address important compliance and unenforceability problems.

Specific Comments on Proposed 42 C.F.R. 10.21

Congress Did Not Authorize HRSA to Impose New Contract Obligations on Manufacturers

The 340B statutory scheme is an opt-in program implemented through contracts. It uses financial incentives – availability of federal payment under the Medicaid program for a manufacturer's covered outpatient drugs - to induce manufacturers to enter into contracts with HRSA for the benefit of covered entities. The terms of these non-negotiable contracts are specified in the 340B statute. The 340B program was not set up solely to provide covered entities with a vehicle to obtain reduced prices on prescription drugs, it was also designed to limit the obligations that manufacturers contractually assumed to those specifically required by statute. When this statute was first enacted, it contemplated that the provisions of the contracts would be fixed and Medicaid payment would not be used as leverage repeatedly to coerce manufacturers to agree to new statutory terms. Accordingly, Congress delegated no authority to HRSA to change manufacturers' contract obligations unilaterally by regulation, and promised manufacturers if a future Congress added new terms to the requirement for a contract in section 340B, manufacturers that chose to ignore the new term would still be deemed compliant with the statutory requirement for a 340B contract. In the Patient Protection and Affordable Care Act (PPACA), Congress amended this freezing provision in order to impose new statutory requirements on manufacturers through the terms of their 340B contracts. Importantly, Congress

did not alter the statutory scheme and authorize HRSA to increase manufacturers' substantive obligations by regulation.

The 340B program requires participating manufacturers to charge a deeply discounted price for covered outpatient drugs to hospitals and clinics eligible to purchase at that price under manufacturers' contracts, and does not prohibit these providers from selling the deeply discounted drugs to their insured patients directly or through contract pharmacies at a considerable profit. Thus, the 340B program essentially shifts a portion of the revenue realized on these sales from manufacturers to eligible providers. PPACA added several new categories of providers to the definition of "covered entity" in section 340B, including children's hospitals, which had not previously been included in the statutory definition. Consequently, Congress expanded the number of covered entities subject to manufacturers' contractual obligations by adding new subparagraphs (M)-(O) to subsection (a)(4) of section 340B, which represented millions of dollars in lost revenue to manufacturers. As a compromise, Congress limited manufacturers' new obligation to sell covered outpatient drugs to these new categories of covered entities at the 340B price. Section 2302 of HCERA, and section 204(a)(1) of MMEA amended section 340B of the Public Health Service Act by adding a new subsection (e):

> For covered entities described in subparagraph (M) (other than a children's hospital described in subparagraph M), (N), or (O) of subsection (a)(4), the term "covered outpatient drug" shall not include a drug designated by the Secretary under section 526 of the Federal Food, Drug, and Cosmetic Act for a rare disease or condition.

This provision unequivocally limits the new burden on manufacturers by simultaneously excluding sales of covered outpatient drugs that are designated as orphan drugs under section 526 from their contractual obligations.

The proposed rule does not merely interpret the statutory definition of "covered outpatient drug." It is a substantive regulation because it adds a new condition to the definition's orphan drug exclusion, *i.e.*, drugs designated under section 526 for a rare disease or condition *and used to treat that disease or condition,* which drastically alters the meaning and scope of that exclusion and thereby significantly increases the scope of manufacturers' contractual obligations. Reading an additional requirement into the statute is not a clarification of an ambiguity but a change in the law that affects manufacturers' substantive rights. Instead of limiting the new requirement to charge discounted prices to more categories of hospitals under the 340B contracts, as contemplated by the statute, the proposed rule inverts the statute, and obligates manufacturers to charge discounted prices on orphan drugs to newly eligible hospitals, except in those instances where the hospital indicated it has used the drug to treat the disease or condition upon which the orphan designation was based.

Respectfully, as a legal matter, it is irrelevant whether HRSA believes that restricting the newly eligible hospitals' ability to purchase orphan-designated drugs under manufacturers' contracts is bad policy. HRSA has no authority or discretion to implement policy by imposing greater substantive duties on individual contractors through legislative rulemaking. If the newly eligible

hospitals are dissatisfied with the benefits they receive under the statutorily specified terms of the 340B contracts, it is up to Congress, not HRSA, to change that policy.

Congress Unequivocally Excluded Drugs Designated Under Section 526 of the FFDCA from the Definition of Covered Drug

Section 2302 of the HCERA plainly and simply excludes from the definition of "covered outpatient drug" any drug designated for a rare disease or condition by the Secretary pursuant to section 526 of the FFDCA. Drugs so identified are excluded from the scope of drugs covered by the 340B program that the newly eligible hospitals can purchase under manufacturers' contracts, and from manufacturers' contractual obligations that incorporate the statutory terms. The provision is easily administered as orphan-designated drugs are listed on a public website. When a drug is designated under section 526 as an orphan drug, certain benefits are available that serve as incentives for the manufacturer to make the huge investment required to seek approval for that particular indication, and it remains so designated, whether or not the orphan-designated drug is approved for that indication or any other indication. Respectfully, there is no ambiguity in what it means to be "designated by the Secretary under section 526 of the Federal Food, Drug, and Cosmetic Act for a rare disease or condition." Yet, in seeking to implement the agency's policy goal of extending 340B prices on orphan-designated drugs to newly eligible covered entities (notwithstanding the statutory language), HRSA makes a huge leap to get from the Secretary's designation of a drug for a rare disease or condition under section 526 to its therapeutic use for that indication in treating a particular patient. In short, it is irrational for HRSA to interpret the action of designating a drug for an orphan disease or condition under section 526 to mean indicated for it.

Even if HRSA had authority to alter manufacturers' contractual responsibilities, which it does not, its interpretation clearly conflicts with the statutory scheme in which a particular drug is either covered by the contract or it is not. The proposed rule would make the same drug both a covered outpatient drug and a non- covered outpatient drug, depending on therapeutic use. In addition, the proposed rule cites no text in the statute or its legislative history to support the conclusion that Congress intended to impose an additional criterion for the exclusion of orphan-designated drugs based on therapeutic use. The proposed rule's attempt to draw a parallel between orphan drug development incentives and the statutory exclusion from manufacturers' 340B contract obligations makes no sense. By analogizing the exclusion from the 340B program to the benefits of a section 526 designation, the preamble to the proposed rule concludes, without any support, that the exclusion was intended to mirror the scope of the orphan drug development incentives. We respectfully disagree. There is no indication that Congress intended anything other than to devise a means to limit additional manufacturer obligations (and revenue losses) resulting from expansion of mandatory 340B discounts.

The preamble also ignores the fact that the benefits of section 526 designation are not restricted to drugs that are ultimately purchased and used to treat a rare disease or condition, as is the proposed rule. Tax credit and research grants apply to drugs designated for a rare disease or condition while the drug is in development, regardless of whether the designated drug is ultimately approved for that rare disease or condition, or is currently or subsequently approved for a common disease or condition. These benefits are intended to offset the enormous

investment required where recoupment through sales is inadequate should the drug even make it to market. Congress was aware that expanding the scope of the 340B program would reduce dollars available for drug development, and that curtailing expansion would free up those dollars. Thus, the statutory exclusion of orphan-designated drugs from the mandatory discount program is entirely consistent with a legislative policy intended to facilitate manufacturer investment.

In our view, HRSA offers no basis in the statutory language or legislative history for interpreting a drug designated under section 526 to mean not just designated for a rare disease or condition, *but also used to treat that disease or condition*. It appears from the legislative history that Congress intended just the opposite. Children's hospitals were not entitled to purchase orphan-designated drugs under the program and immediately sought legislative relief. If Congress meant for the purchase exclusion to apply on an indication by indication basis, it could have clarified the statutory language and no further action would have been necessary. Instead, Congress remedied the problem for children's hospitals by removing them from the exclusion, leaving the provision fully applicable as a limitation on manufacturers' new contract obligations.

The Proposed Rule Conflicts with the Statutory Eligibility Requirement for Free-Standing Cancer Hospitals

Prior to health care reform legislation, the hospitals within the definition of covered entity were so-called subsection (d) or disproportionate share hospitals. Pursuant to 42 U.S.C. 256b(a)(4)(L), a subsection (d) hospital is a covered entity only if it does not obtain covered outpatient drugs through a group purchasing organization (GPO) or other group purchasing arrangement. In the legislative history of the 340B statute, Congress recognized that hospitals authorized to access 340B prices may participate in group purchasing arrangements for purposes of obtaining discounts on pharmaceuticals and did not wish to disturb them for inpatient drugs. When Congress decided to expand the categories of hospitals within the definition of covered entity, it applied the existing GPO prohibition for covered outpatient drugs to children's hospitals and free-standing cancer hospitals. By statute, free-standing cancer hospitals qualify as covered entities eligible to purchase covered outpatient drugs *only* if they meet the requirements for subsection (d) hospitals in 42 U.S.C. 256b(a)(4)(L). Accordingly, free-standing cancer hospitals cannot be certified as covered entities if they obtain covered outpatient drugs from a GPO. *See* 42 U.S.C. 256b(a)(4)(M).

In addition to conflicting with the statutory definition of "covered outpatient drug" discussed above, the proposed rule conflicts with the distinct statutory requirement for a "covered entity" under subparagraph (M). Under the proposed rule, an orphan drug is a covered outpatient drug if used for a non-orphan indication. For many drugs, it stands to reason they would be used to treat common diseases or conditions more often than rare ones, and thus under the proposed rule they would frequently fall within the definition of covered outpatient drugs. Assuming that a covered outpatient drug could include an orphan drug if used to treat a non-orphan disease or condition, under the statute, in order for a free-standing cancer hospital to qualify as a covered entity eligible to participate in the 340B program, it could not purchase an orphan drug used to treat a non-orphan indication from a GPO, because, by definition, the hospital would be purchasing a "covered outpatient drug" from a GPO – a clear contradiction of the statutory prohibition. However, the proposed rule would permit such a hospital to purchase these particular covered

outpatient drugs from a GPO and remain a covered entity eligible to participate in the program if the hospital did not wish to maintain the requisite systems needed to segregate purchases of orphan drugs by therapeutic use. This regulatory exemption from a statutory and contractual requirement for the convenience of certain hospitals is no different than allowing hospitals to purchase outpatient drugs from GPOs to avoid other program requirements that may be costly to administer, including those necessary to prevent diversion or duplicate discounts. It simply cannot be squared with the statute, and HRSA has no authority to promulgate a regulation that contradicts the statute.

That this rule conflicts with both the language and intent of the statute is highlighted by the fact that, during the healthcare reform debate, Congress initially proposed statutory exemptions to the GPO prohibition in PPACA, then reconsidered and rescinded them in the HCERA, the very same statute that created the orphan drug exclusion. The proposed rule reasons that this provision is necessary to prevent those free-standing cancer hospitals that do not want to comply with the regulation from having to forego the price advantage of participating in a GPO arrangement. This reasoning, however, fails to reconcile this policy's clear conflict with the eligibility provisions of the 340B statute. As such, we believe the proposed rule is improper.

The Proposed Rule Lacks Standards for an Auditable Recordkeeping System Capable of Demonstrating a 340B Drug Was Not Used for Treatment of a Disease or Condition Covered by the Purchase Exclusion

The proposed rule would require affected covered entities to maintain distinct inventories and maintain auditable records that demonstrate 340B orphan drugs were not used to treat the rare disease or condition for which the drug has been designated. The proposed rule is unclear as to whether affected covered entities may purchase an initial supply of orphan drugs outside the 340B program as part of a virtual inventory and then replenish the drugs in inventory after the fact, based on indicated use by the entity, through a split billing arrangement. Assuming such a replenishment system is permitted, the rule provides no standards for what would constitute an auditable system. Clearly, there must be a direct connection between the purchase records and the patient records. Maintaining a purchase record system based on individual patient diagnosis and treatment requires accurate patient records and transmission of that information to the pharmacy purchasing department. As there are currently no standards for systems that split billing for drugs between hospital inpatient and outpatient departments, the added complexity for billing based on patient treatment makes implementation of the proposed rule very problematic.

Moreover, information on patient records must be transmitted to contract pharmacies that are purchasing in the name of the covered entity to ensure that prescriptions dispensed by the pharmacy with 340B drugs comply with the rule. There are thousands of contract pharmacies, but manufacturers have no visibility into these or other contractual arrangements covered entities enter into to provide drugs to patients. At present, prescription data available to manufacturers does not include information on whether the dispensed drug is a 340B drug much less the indication for which the drug was prescribed. As discussed below, we believe privacy standards and access to information are major hurdles in establishing an auditable system. HRSA needs to address how these issues can be resolved to ensure integrity of the rule.

The Proposed Rule Fails to Address Compliance Issues and Enforcement of Hospital Non-Compliance

If the statutory provision excluding orphan drugs were implemented as enacted, it would be easy to administer. As the proposed rule acknowledges, the list of drugs designated by the Secretary under section 526 is available on a public website. By contrast, the proposed rule raises hosts of compliance problems. Although the preamble to the proposed rule attempts to provide some assurance that orphan-designated drugs purchased under the program pursuant to the rule need not be considered in the manufacturer's best price for the drug, respectfully, HRSA does not administer the Medicaid drug rebate program and cannot speak for the Centers for Medicare and Medicaid Services (CMS). Likewise, it remains uncertain whether sales of orphan-designated drugs that the statute excludes from the program may be considered 340B sales for purposes of Non-FAMP, AMP and ASP reporting. Given the vast differences between the clear words of the statute mandating an exclusion for a drug and HRSA's proposed "interpretation" of this language as an indication-specific exclusion, if HRSA were to proceed with this rule, manufacturers would need verification from CMS that it interprets the statutory exclusion of orphan-designated drugs from the definition of covered outpatient drug as narrowly as HRSA does (*i.e.*, includes an additional condition) for purposes of Medicaid and Medicare price reporting.

There is no question that the dichotomy established in the proposed rule and the necessity to differentiate purchases by therapeutic use severely burdens an already complex program. Additionally, the proposed rule ignores the practical impossibility of enforcing a treatment-based classification for determining whether a purchase transaction is covered. We are greatly concerned that HRSA has no resources to audit hospital compliance with a whole new set of recordkeeping to implement an indication by indication orphan drug exclusion rule, as it currently lacks sufficient resources even to audit impermissible inpatient use. The proposed rule would also be very difficult if not impossible for manufacturers to audit, because the 340B program is a program concerning the purchase of drugs not the treatment of patients, and existing data sources do not provide information on patient treatment. Moreover, drug utilization data typically provided to manufacturers by health plans and their pharmacy benefit managers would not cover all patients to whom a manufacturer's orphan-designated drug has been provided.

Lacking consistent access to treatment information, manufacturers would generally have no means to enforce compliance except to review hospitals' records. Given the extensive use of contract pharmacies and affiliations, accessing the treating physician's records to determine indicated use is a major enforcement challenge. Moreover, compliance with the requirement that covered outpatient drugs be sold only to patients of covered entities has proven very difficult and costly to audit and has largely gone unenforced. We believe the ability to audit any indication-specific orphan drug exclusion will be exceedingly problematic and compliance will simply go unenforced. As a result, the proposed rule would exacerbate existing covered entity compliance verification problems and further jeopardize the integrity of the 340B program.

Even if record keeping standards are developed so that covered entities could trace a drug from purchase to treatment, and capture information on the use of the drug by a particular doctor in treating a particular patient, verification of this information would necessarily require access to patient treatment records. The proposed rule contemplates that manufacturers would be able to

audit the hospitals' records, but fails to address how they could access this private patient information. Undoubtedly, hiring a third party auditor that is HIPAA compliant and thus able to review patient records would add a significant 340B program cost, and therefore a significant administrative burden to manufacturers' audit efforts. Auditing hospital patient records would be so costly and difficult for manufacturers, as a practical matter, it might rarely happen.

Finally, the proposed rule gives rise to serious issues concerning covered entities' off-label use of purchased drugs. Although manufacturers are prohibited from promoting a drug for an unapproved indication, the purchasing provider generally may use a drug to treat patients for diseases or conditions for which the drug has not been approved. This disparity could create a significant promotional issue under the proposed rule that would not exist where entitlement to a purchase price is not dependent on treatment use (i.e., under a whole drug exclusion). The proposed rule includes in the definition of covered outpatient drug any orphan drug used to treat any disease or condition other than that for which the drug has been designated, regardless of whether the drug has been approved for that indication. If a drug is *only* approved for a designated orphan disease or condition, and a hospital purchases it at the 340B price as a covered outpatient drug to treat a different disease or condition, the covered entity is, in effect, informing the manufacturer that these purchases under the program are for off-label use. Such information may be imputed to manufacturer and creates a risk that discounting or promotion of the drug to the hospital might be misconstrued as off-label promotion by enforcement authorities. To avoid this risk, when a drug is only approved for an orphan disease or condition and the hospital seeks the 340B price, manufacturers should not be required to provide the discounted price.

In sum, the CGP believes HRSA lacks authority to alter by regulation contract obligations that are established by the statutory terms incorporated in those contracts. In addition, we believe the rule's attempt to include and exclude drugs from a purchase price agreement on the basis of therapeutic use by the purchaser is inconsistent with the statute, extremely burdensome, and impossible to administer and enforce. For these reasons, we urge HRSA to reconsider this proposed rule. An alternative that is consistent with the statute and easy to implement, and would alleviate the concerns of free-standing cancer hospitals, is to simply issue a policy guidance that orphan drugs are not subject to the GPO prohibition because they are not covered outpatient drugs.

We thank you again for the opportunity to submit these comments.

Sincerely,

Roger Waldron
President