



July 19, 2011

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

Re: RIN 0906-AA94; Exclusion of Orphan Drugs for Certain Covered Entities under the 340B Program; Proposed Rule

Dear CDR Pedley:

The American Society of Health-System Pharmacists (ASHP) is pleased to submit comments pertaining to the Exclusion of Orphan Drugs for Certain Covered Entities Under the 340B Program (Proposed Rule) as published in the Federal Register on May 20, 2011.¹ For more than 60 years, ASHP has helped pharmacists who practice in hospitals and health systems improve medication use and enhance patient safety. The Society's over 35,000 members include pharmacists and pharmacy technicians who practice in inpatient, outpatient, home-care, and long-term-care settings, as well as pharmacy students.

As the Health Resources and Services Administration (HRSA) notes, the 340B Program of the Public Health Service Act (PHSA) was established by section 602 of the Veterans Health Care Act of 1992 and created a condition of participation for manufacturers wishing to sell drugs to the Medicaid program. The program allows the Secretary of Health and Human Services (HHS) to enter into Pharmaceutical Pricing Agreements (PPA) with drug manufacturers. Should a manufacturer wish to participate in the Medicaid program, and signs a PPA, they agree that the prices charged for covered outpatient drugs to covered 340B eligible entities will not exceed

¹ Federal Register Volume 76, No. 98; 29,183 – 29,190

defined ceiling prices.² HRSA estimates that there are currently over 16,000 participating covered entity sites in the 340B Program. These entities receive an estimated 25 to 50 percent in discounts on the approximately \$6 billion in drugs that they purchase.³ At the time of passage, the Congressional report accompanying the bill states that the intent of the 340B program is to permit covered entities “to stretch scarce Federal resources as far as possible, reaching more eligible patients and providing more comprehensive services.”⁴

The Affordable Care Act of 2010 (ACA) introduced several changes to the 340B Program. Section 7101 of the ACA added several new categories of eligibility for program participants, allowing them to have access to 340B drug pricing except in the case of an orphan drug when used for a rare disease or condition. The entity types added to the list of eligible entities include:

- Children’s hospitals and free-standing cancer hospitals (340B(a)(4)(M)),
- Critical access hospitals (340B(a)(4)(N)),
- Rural referral centers and sole community hospitals (340B(a)(4)(O)).

As noted above, with the exception of children’s hospitals, the ACA extended 340B pricing on drugs to the newly covered entities with the following exception:

EXCLUSION OF ORPHAN DRUGS FOR CERTAIN COVERED ENTITIES—For covered entities described in subparagraph (M), (other than a children’s hospital described in subparagraph (M)), (N), or (O) of 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 (FFDCA) for a rare disease or condition.

While an orphan status designation by the FDA means that the drug has been found “promising” for treating a rare disease, it does not mean that the drug is currently marketed solely for that indication in the United States. Many drugs receive an orphan status designation but do not obtain marketing approval for that indication, but may be approved or otherwise be legally used for other indications that do not meet the requirements of orphan status.

HRSA proposes to apply an interpretation of the statutory language prohibiting purchase of orphan drugs through the 340B Program by certain newly covered entities that limits the

² The 340B ceiling price is calculated by subtracting the Unit Rebate Amount from the Average Manufacturer Price. Drugs purchased by covered entities through the 340B Program may not be sold or transferred to anyone other than the patients of the covered entities.

³ U.S. Department of Health and Human Services, Health Resources and Services Administration, “340B Drug Pricing Program New Covered Entity Webinar.” www.hrsa.gov/opa/340bnewlyeligible.ppt, Last Accessed July 14, 2011.

⁴ H.R. Rep. No.102–384(II), at 12 (1992).

prohibition to uses for the rare disease or condition for which the orphan drug was designated under section 526 of the FDCA.

ASHP supports the Agency's interpretation of the ACA provision that for the newly covered entities listed above, the entities may purchase the drug at 340B prices when using them for conditions for which they are approved, or other lawful use, except in the cases where the drug is used to treat the rare condition or disease for which it is granted orphan drug designation by the FDA.

We believe that this interpretation of statute is appropriate and correct for the following reasons:

The Proposed Rule is in the spirit of the 340B program by extending 340B savings to the newly-eligible covered entities within the limits of the law

As noted above, the Congressional intent of the 340B program at the time of enactment was "to stretch scarce federal resources as far as possible, reaching more eligible patients and providing more comprehensive services." We concur with HRSA that the newly-covered entities should benefit from the 340B Program savings so there is sufficient reason to participate in the 340B Program and use the savings that the program affords to expand access in terms of increased coverage and enhanced services for the underserved and to reduce the price of pharmaceuticals to the neediest of patients. For example, HRSA states that they understand free-standing cancer hospitals to be significant purchasers of orphan drugs. Excluding all purchases of these drugs from the 340B program would not recognize the savings necessary to these entities to provide expanded access to services.

The Proposed Rule preserves financial incentives for manufacturers to develop drugs to fight rare diseases or conditions

We agree that the Federal government should encourage the development and manufacture of drugs used to treat rare diseases or conditions by designating orphan drug status to those drugs that may be used to treat rare and potentially life-threatening diseases. This designation provides a number of financial incentives for the development of the orphan drug for the particular disease or condition; however some drugs may be used to treat multiple diseases or conditions in addition to those for which the manufacturer has received orphan drug designation. The FDA has interpreted the law as permitting the designation of a drug for a rare disease or condition in situations where the drug is also approved for a different disease or condition that does not qualify for such a designation. In those cases, the incentives apply only when the orphan drug is targeted or used to treat a rare disease or condition and not for other indications. In other words, the financial incentives extended by the government to a manufacturer developing an orphan drug apply only to activities directly related to those

associated with the orphan status and do not apply to other indications that have not received orphan drug designation

We believe that the Proposed Rule is consistent with FDA interpretation of the Orphan Drug Act of 1983 and supports the Federal incentives associated with orphan drug designation under current law. A broad exclusion – that is, to bar 340B pricing for all purchases of a drug that may have orphan status for one indication but be widely used for other non-orphan conditions would undermine the purpose of the 340B program and be inconsistent with the Congressional intent of the 340B program. The Society believes that this Proposed Rule does not create disincentives to develop a drug to treat a rare and serious condition (i.e., an orphan drug indication).

The Proposed Rule clarifies how newly-covered entities and manufacturers should treat drugs with orphan status designation under the 340B program

We understand that there has been general uncertainty among the newly-eligible entities and some manufacturers as to how to treat 340B purchases and sales respectively under the provisions of the ACA. ASHP believes that HRSA has provided adequate clarification of the law to relevant 340B participants and that newly-covered entities should follow the provisions of the Proposed Rule and purchase drugs with an FDA approval for an orphan indication outside of the 340B program only in those cases when the drug will be used to treat the rare condition or disease for which the orphan drug was designated. Further, as this Proposed Rule does not create any additional requirements for manufacturers in terms of price reporting, manufacturers should not wait until the Centers for Medicare and Medicaid Services (CMS) issues a policy on the treatment of orphan drugs to covered entities in the calculation of Best Price. Until such time as CMS issues guidance, manufacturers should participate in the 340B program, make reasonable assumptions in the calculation of Best Price, and not limit sales to newly-covered entities.

The Proposed Rule Includes Appropriate Compliance Safeguards

Just as manufacturers are required to accurately account for 340B sales in their Federal price reporting, entities eligible to participate in the 340B program have the responsibility to accurately account for all drugs acquired at significant discount through the 340B program to ensure that they are in compliance with the law and with HRSA's guidance. The Agency will require that 340B entities maintain and provide to HRSA upon request, auditable records verifying that orphan drugs purchased through the 340B program are not transferred, prescribed, sold, or otherwise used for the rare condition or disease for which the orphan drug received designation. In the event that entities are unable, or unwilling to maintain separate records to prove compliance, they may elect to notify HRSA that they will purchase all designated orphan drugs outside of the 340B program regardless of use. ASHP believes that it is

the responsibility of the newly-eligible entities to comply with the law as clarified by the Agency including maintaining separate records for orphan drugs.

For the reasons listed above, ASHP applauds HRSA for clarifying that, in the case of entities newly eligible for the 340B program as added by the ACA, drugs with orphan status designation are exempt from the definition of “covered outpatient drug” for newly-covered 340B entities under section 340B(e) of the PHSA only when they are transferred, prescribed, sold, or otherwise used for the rare condition or disease for which the orphan drug was designated. We believe that HRSA should finalize the provisions of the rule as written. The Society appreciates this opportunity to provide comments. Please feel free to contact me if you have any questions. I can be reached by telephone at 301-664-8806, or by e-mail at ctopoleski@ashp.org.

Sincerely,

A handwritten signature in black ink, appearing to read "Christopher J. Topoleski". The signature is fluid and cursive, with a large initial "C" and "T".

Christopher J. Topoleski
Director, Federal Regulatory Affairs