ALLIANCE FOR RURAL HOSPITAL ACCESS

Congress Should Fulfill the Intent of the 340B Program and Pass the Closing Loopholes for Orphan Drugs Act

In 2010, Congress expanded eligibility for certain safety net hospitals to participate in the 340B Program. However, many newly-eligible hospitals are being largely deprived of any 340B program benefits, due to federal court rulings interpreting a provision of the law excluding certain drugs with orphan designation from the Program's drug discount requirements. As such, Congress should clarify the scope and intent of the 2010 expansion and orphan drug exclusion provisions.

340B Program Background & Eligible Entities

In 1992, Congress expanded the Medicaid drug rebate program to require pharmaceutical manufacturers to extend discounts similar to what state Medicaid programs receive to certain qualifying safety net providers serving the nation's most vulnerable patient populations. This program is commonly referred to as the "340B Program" because the provisions were set forth in section 340B of the Public Health Service Act.

Under the 340B Program—which is administered by the Health Resources and Services Administration (HRSA)—certain covered entities may purchase outpatient drugs from manufacturers at discounted prices, provided they comply with certain program requirements. These covered entities include Migrant Health Centers, Black Lung Clinics, Community Health Centers and entities receiving assistance under the Ryan White Care Act. In addition, Congress enabled hospitals that treat a high rate of low-income patients to qualify, based on their Medicare Disproportionate Share Hospital (DSH) payments. Congress designated these provider types as covered entities because they each fulfill a special role in serving low-income, special-needs and otherwise vulnerable populations.

2010 Changes to Rural Hospital Eligibility & Orphan Drug Exclusion

In 2010, Congress again extended 340B Program eligibility by making it easier for freestanding cancer hospitals, Critical Access Hospitals (CAHs), Rural Referral Centers (RRCs) and Sole Community Hospitals (SCHs) to participate. Under this change, freestanding cancer hospitals and CAHs are eligible by virtue of their status as these providers. RRCs and SCHs are not automatically eligible, but Congress made it easier for them to qualify by lowering the DSH threshold for these facilities.

The RRC program was established to support certain high-volume hospitals that treat a large number of complicated cases and function as regional referral centers. The SCH program was created to maintain access to needed health services for beneficiaries in isolated communities. Together, RRCs and SCHs are facilities that provide rural populations—populations that are, on average, older than urban populations and therefore more dependent on the Medicare program, based on data in a recent HHS report—with access to a

range of health care services, and Congress has long appreciated their important role in the rural health care community and the need to afford RRCs and SCHs special protections to ensure their continued viability.

According to 2016 HRSA data, nearly 1,000 CAHs, three freestanding cancer hospitals, and approximately 185 RRCs or SCHs participating under the lower DSH threshold are now participating in the 340B Program. At the same time that Congress made it easier for these facilities to participate in the 340B Program, it also sought to ensure the program's discounts would not stifle investment in and development of drugs for rare diseases or conditions. Specifically, Congress included a provision that exempted from the 340B discount requirements any "drug designated by the Secretary under section 360bb of title 21 for a rare disease or condition" when purchased by one of the expansion entities. This provision effectively exempts any drug with orphan drug designation.

Many commonly used drugs have orphan designation for one or more indications, even though the drug also is approved for more common indications too. Indeed, a January 2017 study by Kaiser Health News (KHN) found that about one third of orphan approvals made by the FDA since the orphan drug program was enacted in 1983 have been either for mass market drugs repurposed for an orphan designation, or for drugs that received multiple orphan designations.[1] The FDA's orphan drug program provides a number of incentives—such as market exclusivity and tax credits—to encourage development of drug therapies for rare diseases or conditions, but each of these orphan drug incentives applies only when the drug is used to treat the rare disease or condition, and not when used for other indications.

HRSA Rulemaking & Ensuing Litigation

In 2011, HRSA published a proposed rule that sought to define the orphan drug exclusion established under the 2010 law by proposing that orphan drugs would be exempt from 340B discount requirements only when used for the rare condition or disease for which that drug received orphan designation. In 2013, HRSA published a final rule that largely adhered to the proposed rule's interpretation of the orphan drug exclusion.

Shortly after HRSA promulgated its final rule, the pharmaceutical industry—which had been urging HRSA to interpret the exception as applying to *any* drug with orphan designation, regardless of the clinical condition for which the drug was prescribed—sued the agency seeking to enjoin implementation of the final rule; the federal district court issued an opinion siding with the pharmaceutical industry. In 2014, HRSA responded by reissuing its notice as an interpretive rulemaking, which essentially announces the agency's interpretation of the statute, but does not include regulations enforcing it. The pharmaceutical industry responded with a new lawsuit challenging the interpretive rule; again the same court sided with the pharmaceutical manufacturers and invalidated the interpretive rule.

Congressional Action Needed on the Closing Loopholes for Orphan Drugs Act

Since the court decisions, many pharmaceutical companies are restricting access to 340B Program discounts on drugs with orphan designations, thereby undermining the benefits of the program for RRCs, SCHs, CAHs

[1] *Drugmakers Manipulate Orphan Drug Rules to Create Prized Monopolies*, Kaiser Health News, January 17, 2017: http://khn.org/news/drugmakers-manipulate-orphan-drug-rules-to-create-prized-monopolies/?utm_campaign=KHN%3A+Daily+Health+Policy+Report&utm_source=hs_email&utm_medium=email&utm_content=40780219&_hsenc=p2ANqtz--Iz5qttLkkNBVUJN3TerDq15vXUOZzQROhDe9_cERt1nPkP_T44hddg2bb5zf1AkZB00isTyHt_xt-4PcGIhjl7UwJ0w&_hsmi=40780219

and freestanding cancer hospitals. Many such hospitals report significant increases in drug spending since the court decision and are not realizing the full benefit of the 340B Program.

Congress established the orphan drug program to encourage development of drugs for the diagnosis and/or treatment of rare diseases or conditions, and the 340B orphan drug exclusion is, in effect, yet another incentive to promote investment these drugs. However, Congress could not have intended to extend this benefit to a drug use for which there is a substantial and lucrative market. As noted in the KHN study, seven of the ten best-selling drugs in the U.S. in 2015 were drugs with an orphan designation—even though some of these drugs were first approved for more common indications and only later received their orphan designation.

On February 4, 2021, Reps. Peter Welch (D-VT) and David McKinley (D-WV) introduced the Closing Loopholes for Orphan Drugs Act (H.R. 853) to fulfill the intent of the 340B Program. H.R. 853 seeks to clarify the orphan drug exclusion by amending the exemption to limit the carve-out only to those uses for which the drug received orphan status.

The Rural Hospital Coalition urges Member of Congress to please cosponsor the Closing Loopholes for Orphan Drugs Act. This important, bipartisan piece of legislation will ensure that RRCs and SCHs (as well as CAHs and cancer hospitals) benefit from the 340B Program to the extent that Congress intended, allowing these facilities to continue to provide rural communities with local access to important health care services.

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