July 26, 2023

Delivered Electronically via Bipartisan340BRFI@mail.senate.gov

RE: Request for Information on the 340B Program

Dear Senators Baldwin, Capito, Cardin, Moran, Stabenow, and Thune:

The Alliance for Rural Hospital Access appreciates the opportunity to provide comments on bipartisan policy solutions to ensure 340B program stability and oversight, so that the program can continue to achieve its full potential supporting safety net hospitals as they work to serve vulnerable communities.

The Alliance is comprised of hospitals designated as Medicare-Dependent Hospitals (MDHs), Rural Referral Centers (RRCs) and Sole Community Hospitals (SCHs) under the Medicare program. MDHs, RRCs and SCHs provide rural populations with access to a wide range of health care services. In doing so, MDHs, RRCs and SCHs localize care, minimize the need for further referrals and travel, and provide services at costs lower than their urban counterparts. These hospitals also commonly establish satellite sites and outreach clinics to provide primary and emergency care services to surrounding underserved communities, a function which is becoming increasingly important as economic factors force many small rural hospitals to close.

The Alliance is specifically submitting comments on the need to clarify the orphan drug exclusion that was part of the 340B program expansion enacted in 2010. This clarification will ensure that struggling rural hospitals have the resources needed to serve their patients and communities. Senator Welch's (D-VT) Closing Loopholes for Orphan Drugs Act would achieve this goal, and we encourage you to include it in any 340B legislation you work to advance this Congress.

340B Program Background & Eligible Entities

As you know, Congress expanded the Medicaid drug rebate program in 1992 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.

The 340B program, which is administered by the Health Resources and Services Administration (HRSA), allows certain covered entities to purchase outpatient drugs from manufacturers at discounted prices, provided they comply with certain program requirements. 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), RRCs and 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. The populations that RRCs and SCHs serve are on average older than urban populations and therefore more dependent on the Medicare program. These facilities provide rural populations with access to a wide range of health care services, and Congress has long appreciated their significant role in the rural health care community. Congress has also continued to recognize the need to afford RRCs and SCHs special protections to ensure their continued viability.

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 an orphan designation.

Many commonly used drugs have orphan designations for one or more indications, even though the drug also is approved for more common indications too. In fact, 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.

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[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_c ontent=40780219&_hsenc=p2ANqtz--Iz5qttLkkNBVUJN3TerDq15vXUOZzQROhDe9_cERt1nPkP_T44hddg2bb5zf1AkZB00isTyHt_xt-4PcGIhjl7UwJ0w&_hsmi=40780219

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

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 and freestanding cancer hospitals.

Congress established the orphan drug program to encourage development of drugs for the diagnosis and/or treatment of rare diseases or conditions. The 340B orphan drug exclusion is, in effect, yet another incentive to promote investment in 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.

For the past four Congresses, then-Rep., now-Sen. Welch has introduced legislation that would clarify the orphan drug exclusion by limiting the carveout to only those uses for which the drug received orphan status. The Closing Loopholes for Orphan Drugs Act (most recently, H.R. 853 in the 117th Congress) has been a bipartisan effort in each of the last four Congresses, and Sen. Welch intends to introduce the bill again now that he is a Senator.

To ensure that RRCs and SCHs (as well as CAHs and cancer hospitals) benefit from the 340B program to the extent that Congress intended, we encourage you to include the Closing Loopholes for Orphan Drugs Act in any 340B legislation you seek to advance this Congress. This clarification will help ensure rural hospitals have the resources needed to continue to provide their communities with local access to important health care services.

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Thank you for your consideration of these comments. Please contact me at 202.204.1457 or ezimmerman@mcdermottplus.com if you have any questions.

Sincerely,

Eric Zimmerman