
www.HaystackProject.org

July 28, 2023

Submitted to: Bipartisan340BRFI@mail.senate.gov

**RE: Request for Information on 340B Drug Discount Program**

Haystack Project appreciates the opportunity to respond to the bipartisan Request for Information (RFI) on potential refinements to the 340B drug discount program.

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease advocacy organizations to highlight and address systemic access barriers to the therapies they desperately need. Our core mission is to evolve health care payment and delivery systems toward spurring innovation and quality in care toward effective, accessible treatment options for Americans living with rare or ultra-rare conditions. Haystack Project is committed to educating policymakers and other stakeholders about the unique circumstances of extremely rare conditions with respect to product development, commercialization, and fair access to care.

Our responses to the RFI focus on the unique circumstances associated with research, development, and commercialization of innovative treatments for rare and ultra-rare diseases. We ask that any refinements to the 340B program fully consider our patient communities and the fragile balance of incentives and risks that can either drive or deter investment in rare disease treatments. As more fully discussed below, treatments targeted to conditions with very small patient populations tend to face disproportionate 340B exposure. It is, therefore, particularly important that program refinements increase transparency, predictability, and federal oversight to reduce the burden of program integrity enforcement currently resting on manufacturers and ensure that patients served by covered entities receive the benefits the program intended.

## Background

For our patient communities, the emergence of new, innovative therapies targeting specific disease mechanisms offers renewed hope for treatment options, and even a cure to their life-limiting and life-threatening conditions. The ever-expanding scope of the 340B discount drug program is particularly problematic for individuals with rare and ultra-rare conditions as it can deter innovators, and the investors supporting their work, from viewing development of treatments for extremely rare diseases as a worthwhile enterprise.

The innovators who take on the challenge of very rare treatments have to set their prices for new therapies based on a set of assumptions, including patient population size, payer mix, the Medicaid rebate, and projected volume of 340B discounted drug sales. Unfortunately, 340B exposure can be very high given the potential concentration of our experts in a limited set of "centers of excellence." The percentage of product sales subject to the 340B discount can easily, and overwhelmingly, exceed standard expectations. It can also diverge from quarter to quarter, and even climb steadily over time. Innovators in the extremely rare conditions our patients face must contend with a 340B market share well above the already high 25% toward half or more of total sales, and account for significant variability quarter over quarter.

Unexpectedly high 340B exposure can present dramatic revenue concerns that even price increases cannot address due to the "penny pricing" rules. For treatments addressing our extremely small populations, and those that require complex procedures or post-treatment care, it is possible that all, or nearly all, patients will be treated within teaching hospitals that have 340B covered entity status. In these instances, innovators are left choosing between setting a price that accounts for the 340B discount drug sales and risking financial viability of the treatment. Unfortunately, investors could easily conclude that, given the uncertainty and potential for extremely broad 340B discount exposure, any "innovation premium" that would justify absorbing the inherent risk of developing novel treatments could accrue primarily to 340B covered entities rather than to the innovator, its investors, and the patient community.

As patient advocates, we have worked hard to bring attention to the disorders impacting, and even threatening, our lives and/or the lives of our loved ones. We have forged strong relationships with innovators, often working hand-in-hand toward developing treatment options, and ultimately, cures. Our experience tells us that innovators share our goal of ensuring that their treatments advances will reach the patients who need them. The 340B program, however, imposes a forced discount on an uncertain volume of sales without a clear nexus to patient needs and/or access. We fear that it will increasingly tip the scales against innovation and ultimately deprive patients of new treatment options.

1. **What specific policies should be considered to ensure that the benefits of the 340B program accrue to covered entities for the benefit of patients they serve, not other parties?**

As discussed above, our communities are particularly concerned about the 340B program because treatment for very rare conditions is often concentrated in a limited set of facilities and/or within a narrow subset of specialists with disease-specific expertise. Patients often find access limited to a handful of institutions in the country. If those few institutions are 340B entities, they can gain considerable revenue from discount drugs given the relatively high cost for treatments targeted to rare and ultra-rare conditions. The cost savings, however, do not necessarily translate into improved access for patients seeking care.

One example that our patients frequently encounter is access to out-of-state or out-of-network providers. Although state Medicaid programs must enable out-of-state care when, for example,

a targeted treatment is not available in the patient's home state, there is no requirement on the out-of-state facility to treat the patient and accept the home state's Medicaid payment. Covered entities can accept out-of-state/network patients when insurance coverage results in favorable payment and decline to treat others.

Haystack Project supports changes to the 340B program that improve care for under-served populations, including those with rare and ultra-rare diseases. Covered entities should be required to report their patient mix, the aggregate acquisition cost of 340B Drugs and their aggregate reimbursement or the amount of savings achieved by using those drugs, as well as a detailed statement on how those savings were used. We also believe covered entities should be required to report the amount they have provided in charity care, as well as how the dollar value of the charity care was calculated and how the charity care was provided.

In addition, we suggest that Congress consider an approach outlining a set of specific community programs from which a covered entity could select and requiring that a specified percentage of 340B savings be spent on those programs. Mandating that the 340B savings go toward specific programs or initiatives not only helps ensure these funds directly benefit patients but could also be used to identify and address specific social determinants of health or community-specific issues. Rare disease specific programs could include:

- Broader access to care for patients unable to access a specific treatment within their state, network, or geographic area,
- medical geneticist consultation services for newborns,
- improved newborn screening,
- rare disease diagnostic pathways to reduce time to diagnosis,
- telemedicine hubs to connect patients with disease-specific experts,
- respite care for families struggling with rare and ultra-rare diseases, and
- remote therapeutic/physiological monitoring tools.

Alternatively, charitable services thresholds could be set. An entity would have to meet those thresholds in order to maintain eligibility in the 340B program. Regardless of the direction ultimately chosen, we believe that it is imperative to adopt a clear requirement on use of 340B funds for direct patient care rather than to advance the interests of the covered entity.

2. **What specific policies should be considered to ensure HRSA can oversee the 340B program with adequate resources? What policies should be considered to ensure HRSA has the appropriate authority to enforce the statutory requirements and regulations of the 340B program?**

Current 340B policies do little to ensure that manufacturer audits of covered entities promote integrity of the 340B program. While manufacturers are subjected to expensive and cumbersome procedures HRSA rarely takes action against covered entities that are shown to be out of compliance with program requirements, and these covered entities remain entitled to the benefits of the 340B discount. Manufacturers have little incentive to conduct these audits, even when they are in possession of overwhelming evidence that a covered entity is violating the duplicate discount or diversion prohibition.

Further, small manufacturers with low volume products have little to no ability to police the behavior of covered entities. Too often, it is these small manufacturers who are pursuing novel treatments for rare and ultra-rare diseases. As previously stated, because of the disproportional reliance on specialists in a handful of institutions and the disproportionate set of disease-specific providers within covered entities, manufacturers of treatments for rare or ultra-rare diseases are already facing narrow margins for profitability and cannot divert funding toward monitoring covered entities' compliance with 340B.

Haystack Project urges adoption of requirements on HRSA that strengthen its monitoring and enforcement of the 340B program with respect to covered entities. For example, HRSA should:

- Provide written notice of adverse audit findings to both covered entities and all affected manufacturers and ensure that all affected parties have an opportunity to participate in any related hearing.
- Condition continuing access to 340B drug discount upon a preliminary finding of covered entity noncompliance on the covered entity's demonstration that 340B funds are utilized to improve care for its under-served populations.
- Clarify that the failure of a covered entity to follow a corrective action plan can result in the covered entity's termination from the program and ensure that such plans are provided to all affected manufacturers.
- Publish on HRSA's website more details regarding covered entity audit results so that manufacturers can better understand the nature of any violation and how it may affect them.
- Where HRSA uncovers a compliance concern in a covered entity audit that may be common to other covered entities (for example, where a 340B tracking software vendor may be providing non-compliant software programs to multiple covered entities), publish on HRSA's website the adverse finding so that other covered entities can take any appropriate remedial action.

3. **What specific policies should be considered to establish consistency and certainty in contract pharmacy arrangements for covered entities?**

Contract pharmacies participation in 340B has led to expansion of the program well beyond what was likely contemplated by the original authors of the program. Currently, it appears that the 340B program has garnered the interest of large pharmacy chains – indicating a clear potential that contract pharmacy arrangements confer a substantial financial benefit to the contract pharmacy.

We support commonsense reforms of contract pharmacy policies that align with the statute's requirement that 340B discounts need only be offered to covered entities. To the extent that a covered entity does not operate an on-site pharmacy, use of a contract pharmacy provides a pragmatic means of ensuring that patients have access to 340B discount drugs. When, however, covered entities operate an on-site pharmacy and maintain a set of distant contract pharmacies, the nexus between the covered entity's patient population and those receiving discounted drug becomes more tenuous.

In addition, we believe that any economic benefit to a covered entity's contract pharmacy should be limited to its activities as "agent" in the covered entity's distribution of 340B discounted drugs to its patient population. Contractual arrangements that contemplate remuneration based on volume of 340B savings or otherwise transfer 340B revenue to contract pharmacies put those pharmacies closer to the role of purchaser rather than agent. Put simply, the costs associated with contract pharmacies should be aligned with what a covered entity would incur for operating an in-house pharmacy and should not contain incentives normally associated with business partners or sales forces.

Finally, Haystack Project supports direct federal oversight over contract pharmacies and their arrangements with covered entities.

4. **What specific policies should be considered to ensure transparency to show how 340B health care providers' savings are used to support services that benefit patients' health?**

As stated in paragraph 1, Haystack supports HRSA identifying specific uses for 340B funding. These uses could be identified in statute and augmented through public notice and comment procedures wherein the public and stakeholders could help identify any additional appropriate uses, thresholds, or measures to ensure the 340B program continues to support patients.

**Conclusion**

Haystack Project appreciates the opportunity to submit feedback on the 340B program and welcomes the opportunity for a continuing dialogue toward meaningful access to quality care for all patients including access to 340B drugs for those with rare and ultra-rare diseases.

Once again, we thank you for your consideration of our comments. If you have any questions, please contact Saira Sultan, J.D. at 202-360-9985.