

***BY ELECTRONIC DELIVERY***

The Honorable Thomas E. Price, MD  
Secretary  
Department of Health and Human Services  
200 Independence Avenue SW  
Washington, DC 20201

The Honorable Seema Verma  
Administrator  
Centers for Medicare & Medicaid Services  
U.S. Department of Health and Human Services  
Hubert H. Humphrey Building  
200 Independence Avenue, SW  
Washington, DC 20201

**Re: Medicare Program: Hospital Outpatient Prospective Payment and Ambulatory Surgical Center Payment Systems and Quality Reporting Programs; Proposed Rule [CMS–1678–P]**

Dear Secretary Price and Administrator Verma:

The undersigned organizations represent a diverse set of stakeholders, including life sciences companies and patient advocacy organizations, with a shared commitment in developing and ensuring access to treatments for the subset of rare disorders that impact extremely small patient populations.

We applaud the Centers for Medicare & Medicaid’s (CMS’) efforts to address 340B program growth with payment policy refinements, and appreciate the opportunity to offer our comments to the Proposed Rule entitled “Hospital Outpatient Prospective Payment and Ambulatory Surgical Center Payment Systems and Quality Reporting Programs” (the Proposed Rule).

Over the past decade, 340B drug purchases have quadrupled, and manufacturers can now expect that as much as 25% of sales could be subject to the steep 340B discounts. Since treatment for very rare conditions is often concentrated in a few facilities with expertise, any consolidation or 340B abuse can drive 340B market share well above this already-high 25%, and in very unpredictable ways. For orphan drugs used to treat ultra-rare conditions, the 340B program can have a catastrophic and intractable impact – potentially dramatic revenue shortfalls due to increased 340B volume cannot be “corrected” with price increases without risk of triggering “penny pricing.” Left uncurbed, and with little oversight, the 340B program presents a substantial and unpredictable threat to the viability of existing ultra-orphan drugs, as well as to innovation toward new treatment options for patients suffering from ultra-rare conditions.

Despite dramatically increased availability of novel treatment options, many individuals with rare diseases still face hurdles accessing lifesaving and life improving FDA-approved therapies. CMS' policies and initiatives in Medicare can impact providers as well as payers, and influence access to care for Medicaid and privately-insured patients as well as Medicare beneficiaries. Reimbursement mechanisms and hurdles can also tip the scales for or against pursuing a specific drug candidate for an ultra-rare disease indication.

For patient populations approaching the 200,000 orphan-disease population limit, the ODA incentives may be sufficiently robust to mitigate clinical trial and reimbursement risks. As affected populations dwindle below 20,000 or even into and below the hundreds, however, the balance is far more fragile. The significant growth of the 340B drug discount program over the past several years has increased the level of risk and uncertainty for manufacturers of ultra-rare disease treatments that could begin to hurt innovation and ultimately deprive patients of new treatment options. As noted above, our comments focus on the Proposed Rule's 340B-related Part B drug payment reduction, and CMS' stated interest in curbing 340B expansion and promoting program integrity, as summarized below:

- We ask that CMS and HRSA work together toward a greater understanding of the unique challenges to commercial viability for orphan drugs treating ultra-rare disorders and to consider mechanisms to protect these products from excessive and/or unpredictable 340B drug discount exposure;
- We urge CMS to monitor the impact of its proposed Part B drug payment reduction and make necessary adjustments to ensure that patients with ultra-rare disorders have access to treatment, including orphan drugs;
- We urge CMS to adopt a modifier applicable to both Medicaid and Medicare that identifies drugs purchased through the 340B discount program;
- We urge CMS to work with HRSA to identify and implement policy refinements that improve 340B program integrity, oversight, and covered entity accountability.

***We ask that CMS and HRSA work together toward a greater understanding of the unique challenges to commercial viability for orphan drugs treating ultra-rare disorders, and to consider mechanisms to protect these products from excessive and/or unpredictable 340B drug discount exposure and Medicaid double-dipping.***

The 340B discount drug program injects a complex interplay between providers, the Medicaid program, and manufacturers. The program's growth beyond its original intent creates challenges for the manufacturers of ultra-orphan drugs that frequently lack the resources or market share power to navigate the program's intricacies, much less to audit providers for suspected noncompliance.

We urge CMS to work with HRSA, and the undersigned stakeholders, to identify disproportionate 340B impact on treatments for ultra-rare diseases, including:

- Ultra-rare disease therapies encountering 340B drug discount sales volumes that substantially exceed industry averages; and/or
- Treatments for ultra-rare diseases customarily administered in the inpatient setting that have disproportionate outpatient department utilization in 340B covered entities.

We believe that the 340B program can play an important role in ensuring that vulnerable patient populations have access to medical care. Unfortunately, manufacturers of ultra-orphan drugs do not have the luxury of volume or price elasticity to counterbalance excessive 340B exposure. These entities need a safety valve for maintaining viability that is short of declining Medicaid participation. CMS has the claims data needed to identify and define the 340B program's impact on these unique treatments, but lacks the authority to implement 340B policy directives; HRSA has such authority but lacks sufficiently granular data. It is, therefore, imperative that the Agencies collaborate in identifying and implementing policies, such as volume limits on 340B discount drugs or a hardship exemption from penalties associated with price increases that would apply to orphan drugs for ultra-rare conditions facing disproportionate 340B exposure. This level of collaboration could also assist HRSA and manufacturers in identifying covered entity use of 340B discount drugs for Medicaid patients, and aid enforcement of the prohibition against "double dipping."

***We urge CMS to monitor the impact of its proposed Part B drug payment reduction and make necessary adjustments to ensure that patients with ultra-rare disorders have access to treatment, including orphan drugs.***

CMS points to the growth in the 340B program, as well as trends in drug costs, as the basis for its proposed alternative payment methodology, i.e., to reduce payment from ASP +6% to ASP - 22.5%. According to CMS, reducing the OPPS payment rate may curb unnecessary utilization and potential overutilization of drugs, as well as reduce out-of-pocket costs for Medicare beneficiaries responsible for the 20% Part B copayment.

We share CMS' concern that the 340B program has grown beyond its original intent, and support efforts to curb its continued expansion. We strongly support any initiative that would predictably decrease 340B drug use, increase transparency and oversight, and ensure that patients with ultra-rare disorders have access to treatment, including orphan drugs.

We are, however, concerned that the Proposed Rule creates an additional layer of 340B program complexity without (1) directly addressing the increasing breadth of the 340B program, (2) offering the transparency needed now to ensure covered entity compliance with existing Medicaid checks and balances, or (3) including sufficient mechanisms for manufacturer access to covered entity modifier use to ensure compliance with the proposed Medicare layer to 340B discount drug use.

We are also concerned that the proposed payment reduction may have a disproportionate impact on individuals with ultra-rare disorders requiring orphan drugs. These therapies are not subject to the potential for over-utilization that CMS seeks to curb with its payment reduction proposal. For many patients with ultra-rare disorders, outpatient departments are the most reliably-available site of care.

In discussing potential exceptions to the proposed payment reduction, CMS specifically mentioned in its Proposed Rule that patient access to care issues might shape future refinements to the proposed payment reduction. We urge CMS to maintain patient access in the forefront of its priorities as it finalizes the outpatient prospective payment program for 2018, and that (as detailed above) the Agency work with HRSA to implement guardrails to protect ultra-orphan drugs from the 340B variability and extremes to which they are vulnerable.

***We urge CMS to adopt a modifier applicable to both Medicaid and Medicare that identifies drugs purchased through the 340B discount program.***

We agree that the proposed modifier is a relatively simple mechanism that could greatly increase transparency on how 340B discount drugs are used, and aid in identifying noncompliance with program requirements. If CMS finalizes the payment policy articulated in the Proposed Rule, we urge it to implement use of the modifier to:

- Apply to both Medicare and Medicaid claims, so that CMS, the States, and/or manufacturers can more easily identify instances of duplicate discounts; and
- Facilitate timely manufacturer access to covered entity reporting on 340B discount and non-discount drugs provided to Medicare and Medicaid beneficiaries.

***We urge CMS to work with HRSA to identify and implement policy refinements applicable to Medicaid and Medicare that improve 340B program integrity, oversight, and covered entity accountability.***

Manufacturers of orphan drugs for ultra-rare disorders often lack the internal resources to efficiently navigate the complexities of the 340B program. This challenge is compounded by the paucity of guidance and support from HRSA to manufacturers encountering known or suspected covered entity non-compliance. We strongly urge CMS and HRSA to work together to improve 340B program integrity, oversight, and accountability, including by:

- Developing and maintaining a single, universal, and standardized identification system listing participating covered entities to enable manufacturers to quickly resolve quarter-to-quarter eligibility questions and disputes;
- Conducting a preliminary analysis and making a joint determination on whether the proposed payment reduction will curb 340B program growth without constricting access;
- Carefully monitoring the impact of payment reductions, including on Medicaid and Medicare patients, and in non-340B hospitals;
- Facilitating greater transparency so that 340B program integrity issues can be identified early, and manufacturers have information needed to appropriately refer enforcement concerns to HRSA for resolution.

## ***Conclusion***

Collectively, we are committed to preserving and building upon the innovation-driving environment enabled by the Orphan Drug Act of 1983 (ODA), and to ensuring that individuals with extremely rare disorders have meaningful access to medical care, including orphan drugs, in the setting that is most appropriate.

We appreciate the opportunity to offer our comments to the Proposed Rule and look forward to working with you to ensure that our innovations toward treating and curing ultra-rare disorders reach the patients who need them. If you have any questions or need further information from us, please contact Saira Sultan at 202.360.9985.

Sincerely,



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