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June 17, 2022

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS–1771–P
P.O. Box 8013
Baltimore, MD 21244–1850

**Re: Medicare Program; Hospital Inpatient Prospective Payment Systems for Acute Care; Hospitals and the Long-Term Care Hospital Prospective Payment System and Proposed Policy Changes and Fiscal Year 2023 Rates; Quality Programs and Medicare Promoting Interoperability Program Requirements for Eligible Hospitals and Critical Access Hospitals; Costs Incurred for Qualified and Non-qualified Deferred Compensation Plans; and Changes to Hospital and Critical Access Hospital Conditions of Participation (CMS-1771-P)**

Dear Administrator Brooks-LaSure:

Haystack Project appreciates the opportunity to submit comments to the Centers for Medicare & Medicaid Services' (CMS') above-referenced proposed rule updating and refining the hospital inpatient prospective payment system (IPPS) (the Proposed Rule).

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. We strive to amplify the patient and caregiver voice in disease states where unmet need is high, and treatment delays and inadequacies can be catastrophic. Our core mission is to evolve health care payment and delivery systems, 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 comments focus on CMS' Request for Information on potential mechanisms to ensure that IPPS MS-DRG based payment rates do not impede Medicare beneficiary access to rare disease treatments. Resolving this issue has been a key priority for Haystack Project and its member organizations since its inception. CMS recognized that the MS-DRG mechanism could impose

unintentional access barriers that compromise care for rare disease patients during its IPPS rulemaking cycle for FY 2013:

> As stated previously, we acknowledge and recognize the severity of symptoms that patients diagnosed with disorders of porphyrin metabolism may experience. We also are sensitive to concerns about access to care and treatment for these patients. We will continue to monitor this issue and determine how to better account for the variation in resource utilization within the IPPS for these cases.

CMS received similar requests during the FY2015 and FY2019 rulemaking cycles, with commenters reporting "significant difficulties encountered by patients with acute porphyria attacks in obtaining Panhematin® [hemin, for injection] when presenting to an inpatient hospital, which they attributed to the strong financial disincentives faced by facilities to treat these cases on an inpatient basis."[1] Haystack Project member organizations continue to report access hurdles despite CMS' repeated admonition in prior rulemaking on the inappropriateness of denying treatment to beneficiaries needing a specific type of therapy or treatment that involves increased costs.

We strongly urge CMS to use the information gleaned over time, including through this rulemaking cycle, to move beyond the "monitoring" announced over a decade ago and toward concrete action that improves and protects beneficiary access to care in the near-term and sufficiently reimburses providers for the items and services needed to appropriately treat rare disease patients over the long-term.

**The IPPS was not designed to adequately reimburse providers for the cost of rare disease treatments**

The diagnosis-based inpatient prospective payment system was created by the Social Security Amendments of 1983, before the incentives within the newly-drafted Orphan Drug Act spurred development of rare disease treatments. The MS-DRG groupings, and CMS' policies for creating new MS-DRGs, were largely based on a reality that did not consider a future where a treatment for one out of a hundred or more rare diseases could create substantial divergence in costs within an MS-DRG. The emergence of new inpatient treatments such ANDEXXA® (coagulation factor Xa (recombinant), inactivated-zhzo) creates added complications in adequately reimbursing providers due to the potential that inpatient stays requiring the product would be spread across multiple MS-DRGs. In addition, CMS was asked to consider the need for payment mechanisms to accommodate the cost for administering Zulresso® (brexanolone), the first

[1] See, IPPS Proposed Rule for FY2023, Federal Register :: Medicare Program; Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and the Long-Term Care Hospital Prospective Payment System and Proposed Policy Changes and Fiscal Year 2023 Rates; Quality Programs and Medicare Promoting Interoperability Program Requirements for Eligible Hospitals and Critical Access Hospitals; Costs Incurred for Qualified and Non-Qualified Deferred Compensation Plans; and Changes to Hospital and Critical Access Hospital Conditions of Participation

FDA- approved drug for postpartum depression (PPD) after it declined to implement a New Technology Add-On Payment (NTAP) due to the relatively small number of Medicare beneficiaries likely to become pregnant.

Unless an orphan drug is indicated for most inpatient stays within an MS-DRG, administration of the treatment (after NTAP expiration) will almost certainly result in an economic loss for the provider. Unfortunately, CMS data is an inherently poor information source for determining the existence and magnitude of cost divergence within an MS-DRG and the impact that insufficient reimbursement has on beneficiary access because:

- Hospital cost data after NTAP expiration will include inpatient stays in which beneficiaries did and did not receive the FDA-approved drug, with no clear indicator of the treatment provided. This will give an artificially low estimate of the cost for providing a standard of care incorporating an orphan drug. [2]
- Both before and after NTAP expiration, drug costs are rarely subject to a "mark-up" that is consistent with the cost-to-charge ratio. Drug acquisition costs, therefore, are generally not fully incorporated into rare disease MS-DRG payments.

The fact that CMS has been repeatedly asked to correct payment deficiencies associated with inpatient treatment for rare conditions shows that payment inadequacies for rare diseases under the MS-DRG system elude the presumption of continued access within a "system of averages."

Introduction of high-cost CAR-T therapies focused CMS on the unfortunate reality that unless reimbursement is rationally related to the cost of a rare disease treatment, the subset of providers willing to absorb a monetary loss can diminish rapidly and become a very real and impenetrable barrier to access. If CMS had declined to create a new MS-DRG for inpatient stays associated with CAR-T treatment, the extraordinary costs associated with this breakthrough therapy would have been averaged with the costs for the remaining stays under the previous MS-DRG(s). This would have led to an ever-diminishing set of willing CAR-T providers while simultaneously creating significant financial windfalls for facilities unable or unwilling to deliver CAR-T therapy. The result CMS avoided with CAR-T is the reality for other rare disease treatments administered in the inpatient setting, albeit on a smaller scale given the high cost of CAR-T. The incremental cost of rare disease treatments within or across MS-DRGs increases reimbursement for all inpatient stays in the group while maintaining an inadequate payment for appropriately administering the treatment. Facilities can, and do, reduce exposure to financial losses associated with orphan drugs by:

[2] Id at p. page 28196, discussing inpatient stays associated with porphyria attacks. "The commenters stated that, based on the lower than expected average cost per case and longer than expected length of stay for acute porphyria attacks, it appeared that facilities were frequently not providing Panhematin® to patients in this condition, and instead attempting to provide symptom relief and transferring patients to an outpatient setting to receive the drug where they can be adequately paid."

- Failing to stock orphan drugs that might be associated with economic loss. This is especially problematic for products that are generally ordered on an as-needed basis.
- Delaying or declining hospital formulary inclusion
- Attempting to resolve the patient's symptoms with off-label or potentially less effective treatment options. Delays in administering FDA-approved treatments due to cost considerations can lead to compromised patient outcomes and longer inpatient stays.
- Treating patients within outpatient departments based on financial considerations rather than patient status and potential care needs.

Haystack Project acknowledges that the MS-DRG framework of offsetting below-cost reimbursement on inpatient encounters with patient stays requiring fewer resources works for common conditions or groups of conditions with similar clinical and resource use characteristics. Unfortunately, when applied to rare disease treatments. the existing MS-DRG framework and volume thresholds for additions and refinements to diagnosis groupings impede rational alignment between cost of care and reimbursement. The IPPS, when applied to rare disease treatments, inadvertently perpetuates payment inadequacies by spreading the incremental cost of treating higher-cost rare disorders over a potentially diverse MS-DRG so that some conditions are "winners" and others are "losers." CMS has both the authority and obligation to ensure that its MS-DRG framework does not create a system of excess payments and treatment deficiencies for rare disease inpatient stays.

**The IPPS should incorporate mechanisms to ensure that payment for inpatient stays that include rare disease treatments is aligned with the cost of acquiring and administering those treatments.**

As noted above, high-quality health care for individuals with rare and ultra-rare conditions can be relatively high-cost and often requires highly specialized clinicians and timely access to orphan drugs. Haystack Project  believes that the underlying goal of Medicare payment policy – patient access to the right therapy at the right time and in the right setting – requires pragmatic "fixes" rather than strict adherence to policies that, like the 500-inpatient-stay volume threshold to MS-DRG change requests, are inherently unsuitable within the rare disease context. Similarly, CMS' longstanding focus on limiting the absolute number of MS-DRGs has led to groupings of rare disorders into extremely broad catch-all categories that have become increasingly irrelevant as orphan drugs are developed to address rare diseases. We support pragmatic approaches that:

- Carve out "clinical trial" inpatient stays to ensure that facility-reported costs that do not include drug acquisition do not adversely impact the MS-DRG payment rate
- View the "clinically similar" prong of the MS-DRG analysis from a treatment-specific perspective rather than purely on factors such as impacted body system or disease mechanism
- Use actual product acquisition costs in calculating payment rates for inpatient stays that include administration of an orphan drug. Haystack Project previously expressed its support when CMS implemented a similar policy for CAR-T therapy. Utilizing a cost-to-

charge ratio of "1" for orphan drugs reduces the impact that charge compression would otherwise have on payment rates
- Recognize that, for patients with serious, rare conditions, real-world Medicare beneficiary access to available treatments outweighs the Agency's interest in minimizing the number of MS-DRGs or adhering to a 500-inpatient-stay volume threshold in deciding whether an MS-DRG change request should be considered
- Ensure that the average cost ***for the standard of care for each diagnosis***, no matter how rare, within an MS-DRG is no more than 150% of the average costs upon which payment is calculated for the MS-DRG in general
- Engage stakeholders to identify specific rare disorders currently grouped within MS-DRGs for which the payment is well below the average cost for providing care.

We encourage CMS to carefully consider feedback from stakeholders in the patient and provider communities, as well as any insights and recommendations from orphan drug innovators. Haystack Project also offers rare disease inpatient payment options we have identified in consultation with our advocacy organization members.

- ***Create treatment-dependent subgroups analogous to the existing complications/major complications MS-DRG subgroups.*** Haystack Project suggests that CMS start with the rare disease treatments identified in the Proposed Rule, and the MS-DRGs to which inpatient stays requiring those treatments are currently assigned and incorporate additional therapies/diagnoses as they are identified within the IPPS annual rulemaking cycles and as NTAP payments for rare disease treatments expire. The payment would:
    - maintain the potential that there may be one, two or more diagnoses and one, two or more disease-specific treatments
    - be based on the MS-DRG payment, minus the cost of avoided treatments, plus the stated acquisition cost for the orphan drug during the inpatient stay
    - be available only when the diagnosis reported for the inpatient stay is appropriate for the specific treatment
    - not be available for inpatient stays where access to the treatment was unreasonably delayed

- ***Implement an add-on payment that is calculated in a manner similar to what is used for NTAP payments.*** Haystack Project again recommends that CMS start with the therapies identified in the FY2023 Proposed Rule and update the set of eligible treatments and diagnoses through the annual IPPS rulemaking process.
    - We strongly urge CMS to eliminate any "newness" or "substantial improvement" criteria for treatments that have evidence supporting use for specific diagnoses as medically accepted.

**As CMS considers rare disease payment options, it should implement sufficient oversight and enforcement to ensure adherence to its policy on inappropriate withholding of high-cost treatments for rare diseases.**

Haystack Project member organization American Porphyria Foundation (APF) has been instrumental in elevating a robust dialogue with CMS staff on the access hurdles rare disease patients face in the inpatient setting. We echo APF's appreciation for CMS' repeated statements asserting that facilities may not withhold or delay access to specific, FDA-approved rare disease treatments due to financial considerations. We understand that APF has previously relayed, within its comment submission(s), a series of patient reports on treatment deficiencies experienced in inpatient facilities. Haystack Project also shares APF's concern that the only mechanism CMS has offered is a "complaint" process after care has been denied. Patients generally, and rare disease patients in particular, have little or nothing to gain that would justify the time, focus, and potential for "anger" from a provider unless their complaint can influence the care they receive in the here and now.

We recommend that CMS implement a pragmatic and sufficient mechanism to remove the disincentives currently in place for rare disease treatments administered in the inpatient setting. Until a mechanism is implemented, however, we recommend that CMS offer rare disease patients a dependable, accessible, and simple mechanism for real-time resolution of complaints related to delays in receiving rare disease treatments.

**Haystack Project urges CMS to consider patients beyond the Medicare population when determining appropriate MS-DRG assignment and/or assessing the need for a new MS-DRG or subgroup.**

As we have noted in past comments, CMS' payment mechanisms, policies, and structures devised for the Medicare program can significantly affect inpatient reimbursement for both commercial payers and the state Medicaid programs. The patient and caregiver advocacy organization within Haystack Project's membership recognize that the current pace of innovation brings new hope for patients in desperate need of treatment options. For many rare and ultra-rare diseases, the inpatient setting may predominate until providers gain sufficient experience to move care to the outpatient setting. For patients suffering from acute, episodic "attacks" and other life-threatening disease symptoms, inpatient administration may remain the best option. Appropriate inpatient coding and payment mechanisms are, therefore, crucial in ensuring access to care for all patients.

While we understand that the MS-DRG system was designed and refined to accommodate the Medicare program and its beneficiary demographic, its structures drive inpatient payment for commercial plans as well as Medicaid programs in 26 states and the District of Columbia. Haystack Project remains concerned that decisions on MS-DRG structure and groupings that are based solely on statistical analyses in the Medicare program could have an unintended impact on pediatric and young adult access to care, including women of childbearing potential. We urge CMS to review MS-DRG assignment of rare and ultra-rare disorders in a holistic manner that places the nature of patients with specific disease states and their care needs paramount over volume of Medicare claims. For extremely low-volume diagnoses, this may be in the form of a "placeholder" MS-DRG, subgroup, or add-on payment that does not have a payment

amount derived from Medicare claims data. We believe that this approach is consistent with CMS' leadership within the overall health care system and that it will ensure that patients covered by other payers relying on CMS' MS-DRG structure are not overlooked.

**Conclusion**

Haystack Project appreciates the opportunity to offer its comments and suggestions as CMS finalizes its updates, refinements, and revisions to the Medicare IPPS. Despite dramatically increased availability of novel treatment options, patients with rare diseases still face hurdles accessing lifesaving and life-improving FDA-approved therapies. For emerging treatments administered in the inpatient setting, the MS-DRG system driving the IPPS is an added, significant, and potentially insurmountable hurdle.

We are encouraged by CMS' request for stakeholder input on IPPS payment mechanisms for rare disease treatments and look forward to a continuing dialogue toward ensuring that all patients receive the right care, no matter how rare their disease or condition.

If you have questions or need further information, please do not hesitate to contact M Kay Scanlan at 410-504-2324.


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