June 10, 2024

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS–1808–P
P.O. Box 8013
Baltimore, MD 21244–1850

RE: CMS–1808–P
Medicare and Medicaid Programs and the Children’s Health Insurance Program; Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and the Long-Term Care Hospital Prospective Payment System

Dear Administrator Brooks-LaSure:

Haystack Project appreciates the opportunity to submit its comments on the Centers for Medicare & Medicaid Services’ (CMS’) proposed rule updating and refining the Medicare hospital inpatient prospective payment system (IPPS) for fiscal year 2025 (the Proposed Rule).

Haystack Project is a 501(c)(3) non-profit organization with a membership of 140+ rare and ultra-rare disease patient advocacy organizations. Our core mission is to evolve health care payment and delivery systems with an eye toward spurring innovation and quality in care toward effective, accessible treatment options for all Americans. We strive to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access in disease states where unmet need is high and treatment delays can be catastrophic.

Our comments to the Proposed Rule largely reiterate Haystack Project’s longstanding request that CMS develop a payment mechanism to counterbalance the financial disincentives hospitals face when a rare disease treatment is not sufficiently reimbursed under the MS-DRG assigned to inpatient stays related to the rare disease.

Background

Haystack Project has previously applauded CMS for recognizing that introduction of high-cost CAR-T therapies created an unfortunate reality in which the subset of providers willing to absorb a monetary loss would diminish rapidly and become a very real and impenetrable
barrier to access. Had CMS 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. CMS was able to avoid the significant access issues that are the unfortunate reality for patients needing rare disease treatments administered in the inpatient setting.

It is not surprising that the IPPS, when applied to rare disease treatments, inadvertently perpetuates payment inadequacies. When Congress directed implementation of the inpatient prospective payment system, there were few available treatment options for any rare diseases. Over the past four decades, the Orphan Drug Act has, as FDA notes “finally provided for many of those orphaned among blockbuster treatments a hope of their own thanks to the work of many, not the least of whom were those patients and their advocates who had long championed the needs of the forgotten patients.”

Unfortunately, increased availability of novel treatment options has not always translated into access to lifesaving and life-improving FDA-approved therapies for all rare disease patients needing them. Access hurdles like those related to bundled Medicare payment rates for inpatient stays, continue to prevent too many patients from receiving what may be the only treatment available to slow the progression or ease the burden of their rare disease, or to resolve a life-threatening acute episode. The more rare the condition, the more acute and persistent this problem remains.

We understand that the IPPS is, in simple terms, a system of averages. The MS-DRG framework of offsetting below-cost reimbursement on some inpatient encounters with patient stays requiring fewer resources works for common conditions or groups of conditions with similar clinical and resource use characteristics. Unfortunately, the IPPS mechanisms that function as pragmatic tools to appropriately pay for most inpatient stays exact a likely unintentional, but often profound and disproportionate impact on stays for rare diseases treated with orphan drugs.

Haystack Project sees no reason to expect the “system of averages” CMS so carefully constructed should continue to create insurmountable access disparities as additional innovative treatments for rare conditions become available. Furthermore, CMS’ longstanding focus on limiting the absolute number of MS-DRGs has led to groupings of rare disorders into catch-all categories that have become increasingly irrelevant to the nature of the inpatient stay or the resources required. These conditions are often too rare to ever reach the thresholds CMS applies to consider creating a new MS-DRG. The updates designed to capture changes in standards of care and associated costs, will similarly fail patients with extremely rare disorders unless the diagnosis is within a MS-DRG with relatively homogenous treatment modalities and care costs.
Medicare beneficiaries should not have to bear the access burden caused by the MS-DRG system, its groupings, or CMS’ policies on when payment deficiencies must be addressed.

We believe the fundamental problem with treatments administered within extremely low volume inpatient stays is that the incremental cost of treating higher-cost rare disorders is spread over a potentially diverse MS-DRG so that some conditions are “winners” and others are “losers.”

We consistently hear treating clinicians tell us they are desperate to help their patients and yet are concerned that they may get called out if they provide a treatment within the MS-DRG or try to find a work-around to ensure their patients get what they need. We have met with the Agency repeatedly about these concerns, and we agree with CMS’ position that providers are required to treat patients within the standard of care regardless of the financial consequence but have learned that many patients with rare and ultra-rare conditions experience a very different reality. However, CMS needs to understand that this position is not backed up with enforcement, and if enforced, would likely force the few experts in a rare disease to stop seeing these patients.

Haystack Project similarly notes that access challenges are not limited to particularly “new” treatments or even to those that are, like CAR-T therapy, extremely costly. Moreover, as the multiple requests to “fix” the reimbursement for acute porphyria patients that CMS received over nearly a decade illustrate, these access barriers will not resolve over time. The medication at issue for porphyria patients has been available since 1983, it is recognized as the standard of care for resolving acute porphyria attacks, and its cost is just enough to deter hospitals from ordering the medication yet not expensive enough to hit the “outlier” thresholds required for Disproportionate Share Hospital payments. As CMS has noted, patients continue to struggle to get this treatment.

CMS has demonstrated that it has the authority to refine IPPS mechanisms when it determines that there is a problem that must be addressed. Access issues associated with low volume inpatient stays are a problem, and the problem must be addressed.

CMS must urgently address the access impediments it has been made aware of and act to ensure that new treatments do not fall down this predictable road.

CMS had the authority to develop a new approach as it did in connection with CAR-T therapies, and can, within the statute, ensure MS-DRG payments are sufficient to incentivize providers to administer the new gene therapies for sickle cell disease.

CMS has both the authority and an obligation to ensure its MS-DRG framework does disadvantage rare disease patients. Conclusory statements noting that hospitals must provide orphan drugs to patients regardless of the financial consequences have not helped patients and have not changed the behaviors of hospitals. Moreover, CMS cannot expect that rare disease
patients sick enough to require an inpatient stay will “police” hospitals and enforce CMS’
expectation that appropriate care be provided. In rural and other underserved communities,
patients may not have a single hospital within driving distance that is willing to furnish the
treatment they need in the inpatient setting. Haystack Project has sometimes referred to ultra-
rare patients as the “canary in the coal mine” with respect to reimbursement mechanisms with
the potential to impede access – the canary’s demise is a clear signal that something is very
wrong and action is necessary. The need for action is every bit as clear and urgent for rare
disease patients who cannot count on getting the care they need and find that effective
treatments that are “available” are not available to them.

Haystack Project urges CMS to develop a limited, pragmatic mechanism calculated to pay
hospitals for these treatments if and only if they are actually used in treating patients. The cost
of the treatment would not be included in MS-DRG payment calculations. We suggest that this
type of mechanism would apply to drugs used within extremely low volume stays, including
scenarios where:

- An orphan drug is used for one condition within a catch-all MS-DRG comprised of
dozens (or hundreds) of other conditions. This is the scenario applicable to acute
  intermittent porphyria patients.

- An orphan drug is used for multiple conditions across several MS-DRGs, e.g., to
  manage/treat complications of a condition or treatment. These treatments might
  include:
    o ANDEXXA® to reverse anticoagulant effects of certain drugs
    o Orphan drugs to treat/manage acute graft versus host disease

- A treatment is directed at a condition infrequently encountered in Medicare
  beneficiaries.
    o Zulresso® (brexanolone) for post-partum depression

- Medicare has recognized the potential for access issues because the cost of a new
  treatment is very high or other factors suggest potential health disparities and/or
  inequities.
    o CAR-T
    o New cell and gene therapies for sickle cell disease

- An MS-DRG includes inpatient stays for which non-drug resource use is similar but the
  cost of the underlying treatment is subject to a great deal of variability.
    o CAR-T and immunotherapies

We expect that CMS received suggestions in response to its request for information within the
FY 2023 rulemaking and ask that the Agency consider the proposal outlined above as well as
information gleaned from stakeholders over time. We strongly urge CMS to do what is
necessary to prevent new access hurdles from arising with new treatments by continuing NTAP
payments that are now in place for low volume inpatient stays unless and until the MS-DRG
calculations reflect the cost of the treatment. This is what the NTAP mechanism was intended
to do. Alternatively, CMS could apply its “fix” for CAR-T therapies to new treatments on an
interim basis by creating temporary DRGs until CMS has fully analyzed and considered
stakeholder feedback to its RFI. This is not an intractable problem, particularly in light of the
documented access barriers Medicare beneficiaries experience when their condition is too rare
to meet CMS’ volume requirements driving whether it will ever fix a payment deficiency within
MS-DRG groupings.

Haystack Project is losing all hope that CMS will move beyond the “monitoring” announced
over a decade ago and toward concrete action that improves and protects beneficiary access to
care and sufficiently reimburses providers for the items and services needed to appropriately
treat rare disease patients.

**Conclusion**

Haystack Project appreciates the opportunity to offer its comments and suggestions as CMS
finalizes its updates, refinements, and revisions to the 2025 Medicare IPPS. We look forward to
a continuing dialogue toward ensuring that all patients receive the right care in the right
setting, 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.

Very truly yours,

Kara Berasi
CEO
Haystack Project
Kara.berasi@haystackproject.org