VIA ELECTRONIC SUBMISSION

June 9, 2023

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

Medicare Program; Proposed Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and the Long-Term Care Hospital Prospective Payment System and Policy Changes and Fiscal Year 2024 Rates; Quality Programs and Medicare Promoting Interoperability Program Requirements for Eligible Hospitals and Critical Access Hospitals; Rural Emergency Hospital and Physician-Owned Hospital Requirements; and Provider and Supplier Disclosure of Ownership
(CMS-1785-P)

Dear Administrator Brooks-LaSure:

Haystack Project appreciates the opportunity to provide 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 2024 (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.

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 translated into access to lifesaving and life-improving FDA-approved therapies for patients needing them.

Access hurdles, often related to reimbursement structures such as inadequate bundled payment rates, high cost-sharing and/or payer coverage restrictions 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.

Our comments to the Proposed Rule ask, once again, that CMS ensure that when a rare disease patient receives care in the inpatient setting, they receive the care they need, including any FDA-approved treatment, even if the cost of the treatment exceeds the payment to the hospital. Although CMS has reiterated its position that providers are required to treat patients within the standard of care regardless of the financial consequence, many of our patients experience a very different reality. We also note that CMS has periodically responded to Haystack Project and other stakeholders' reports of access delays and denials with assurances that the Agency would consider available options for accommodating higher-cost, low-volume inpatient stays, including in 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.

We strongly urge CMS to use the information gleaned over time, including through its request for information within the FY 2023 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.

In addition, Haystack Project was disappointed with the direction CMS appears to be taking with the New Technology Add-On Payment (NTAP) process and its evolving implementation of the NTAP eligibility criteria. Patients with rare diseases awaiting development and approval of new inpatient treatment options had a measure of hope that the NTAP would enable providers to incorporate new treatments into their formularies and treatment protocols with a level of comfort that, at least in the short-term, they could bear the costs associated with providing the treatment. As more fully discussed below, CMS' apparent constriction of its "newness" and "substantial improvement" criteria – even for breakthrough drugs and biologicals – could make NTAPs for extremely rare disease treatments a thing of the past.

The MS-DRG Structure Must be Refined to Address Beneficiary Access Hurdles.

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. As we have highlighted repeatedly, the result CMS avoided with CAR-T is the reality for other rare disease treatments administered in the inpatient setting. 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." We continue to believe that 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.

In last year's proposed rule, CMS appeared interested in identifying and implementing mechanisms that would address access to treatments for rare diseases within the MS-DRG system. CMS noted that rare diseases "pose a unique challenge" and reiterated their continuing preference for "larger clinical cohesive groups within an MS-DRG" to provide "greater stability and thus predictability…" Since it is not feasible to make sure that a new treatment targeted to a single rare disease within an MS-DRG with dozens or even hundreds of other rare diseases will ever fulfill CMS' need for high volume, we once again ask that the Agency implement an alternative mechanism that would not place structural preferences over patient health. We expect that CMS received suggestions in response to its request for information and ask that it also consider implementing one or more of the approaches outlined below:

- Implement a mechanism similar to its "two-times" rule for unbundling items and services within the outpatient prospective payment system. This approach would ensure that patients with just one therapeutic option are not told that their local hospital does not provide the drug they need due to its high cost.

- Extend NTAP designation beyond the two- to three-year timeframe for rare disease products that address an unmet need and continue to satisfy the NTAP "cost" requirement.

- Develop a set of codes that could be reported with the applicable MS-DRG with a set payment for rare disease treatments within several defined cost ranges.

- Create rare disease MS-DRGs that pull specific rare diseases out of their current assignment if the treatment costs exceed the MS-DRG payment by a defined threshold. Although the diagnoses within the MS-DRGs may not be clinically similar, we expect that MS-DRGs could be created to ensure similarity in resources and cost of care.

Finally, we ask that CMS maintain transparency as it evaluates options for ensuring that the MS-DRG system of averages does not impede access to rare disease treatments and that it identifies an interim mechanism to ensure that patients requiring inpatient care are able to receive the standard of care for their specific condition, including any orphan drugs. This could

include oversight to align patient experience with CMS' assertion that hospitals may not decline to provide a specific item or service due to financial considerations.

CMS Should Apply the Presumptions Applicable to Breakthrough Devices to Evaluating whether Drugs and Biologicals with Breakthrough Status Satisfy NTAP criteria.

In its IPPS Rule for FY 2020, CMS made a significant, and pragmatic, change to how it evaluates breakthrough devices seeking NTAP eligibility. That change enabled medical devices within FDA's Breakthrough Devices Program that have received marketing authorization to rely on a presumption that the device is new and not substantially similar to existing technologies (the "newness" requirement). CMS also acknowledged that these devices would lack sufficient evidence to demonstrate "substantial clinical improvement" and does not subject breakthrough devices to an inquiry on that requirement. CMS' policy for breakthrough devices makes sense. It looks to determinations made by the FDA and accepts those determinations within the context of ensuring beneficiary access to new therapies.

Haystack Project urges CMS to revisit its determination that the policy applicable to devices is somehow inappropriate for drugs and biologicals. We are unaware of any rationale for concluding that FDA's determination that a device is entitled to breakthrough status is more reliable or supported by better evidence than the Agency's determination to grant that status to a drug or biological. Moreover, breakthrough drugs and biologicals, particularly those that are targeted to rare diseases face the same difficulties with establishing "substantial clinical improvement" that CMS found would cause an unreasonably delay in access for breakthrough devices. We strongly disagree with CMS' FY2020 assertion that the "current drug pricing system provides generous incentives for innovation" within the context of rare disease treatments paid under an MS-DRG with all other rare diseases impacting the same body system.

Finally, we ask that CMS consider that the set of drugs and biologicals with breakthrough status that would be utilized in the inpatient setting is likely far smaller than the number of NTAP applications received for breakthrough devices under the new policy. Expanding the policy to include drugs and biologicals is logical, reasonable, and would reduce access impediments for patients needing breakthrough drugs during an inpatient stay.

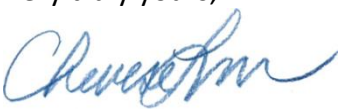
Conclusion

Haystack Project appreciates the opportunity to offer its comments and suggestions as CMS finalizes its updates, refinements, and revisions to the Medicare IPPS. As you are likely aware, 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 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,



Chevese Turner
CEO
Haystack Project
chevese.turner@haystackproject.org