



June 24, 2019

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

**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 2020 Rates; Proposed Quality Reporting Requirements for Specific Providers; Medicare and Medicaid Promoting Interoperability Programs Proposed Requirements for Eligible Hospitals and Critical Access Hospitals**

Dear Administrator Verma:

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

Haystack Project is a non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access. Our core mission is to evolve health care payment and delivery systems with an eye toward spurring innovation and quality in care, and effective, accessible treatment options for all Americans.

[The Rare Cancer Policy Coalition (RCPC) is a Haystack Project initiative that brings together rare cancer patient organizations. RCPC gives participants a platform for focusing specifically on systemic reimbursement barriers and emerging landscape changes that impact new product development and treatment access for rare cancer patients. It is the only coalition developed specifically to focus attention on reimbursement, access and value issues across the rare cancer community. Working within the Haystack Project enables RCPC participants and rare and ultra-rare patient advocates to leverage synergies and common goals to optimize advocacy in disease states where unmet need is high and treatment inadequacies can be catastrophic.]

While countless lives have been improved, or saved by new therapies enabled by Congress' set of incentives for orphan drugs, millions of Americans affected by a rare disease are still waiting and hoping for treatment or a cure:

- Approximately 50% of the people affected by rare diseases are children;
- 30% of children affected by a rare disease will not live to see their 5<sup>th</sup> birthday; and
- Approximately half of identified rare diseases do not have a disease-specific advocacy network or organization supporting research and development.

Despite dramatically increased availability of novel treatment options, many patients with rare diseases still face hurdles accessing lifesaving and life-improving FDA-approved therapies. These hurdles are often related to reimbursement structures such as inadequate bundled payment rates, high cost-sharing and/or payer coverage delays and restrictions on what may be the only treatment available to reduce a patient's disease burden.

### ***Background***

Advances in research and development such as regenerative medicine, gene therapy, and other targeted therapy innovations offer a renewed hope that a treatment could be on the horizon for any disease, no matter how rare. This sense of optimism is, however, tempered by increasing discussions about whether payers -- public and private -- will be willing and able to pay the high cost of these highly-targeted treatments. For emerging treatments administered in the inpatient setting, the MS-DRG system driving the IPPS is an additional, significant, and potentially insurmountable hurdle unless patient populations are sufficient to reach CMS' threshold for considering revisions to MS-DRG assignment.

When Congress enacted the Social Security Amendments of 1983 directing creation of a diagnosis-based inpatient prospective payment system, it likely did not envision the profound impact its newly drafted Orphan Drug Act would have on rare disorder treatment development, or recognize that the two initiatives could run at counter-purposes.

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 involving rare diseases treated with orphan drugs.

Payment inadequacies for rare diseases under the MS-DRG system, particularly where treatment involves orphan drugs, elude the "fixes" and adjustments available for more common conditions. The mechanisms for IPPS updates tend to perpetuate potentially large payment deficiencies for diagnoses with very few patients, while over-paying for the remaining diseases within the same MS-DRG. For extremely rare disorders with high-cost therapies, inadequate reimbursement can erect an impenetrable barrier to what may be the only treatment available to reduce disease burden. The unfortunate reality is that unless reimbursement is rationally related to the cost of treatment targeted to a specific rare disorder, the subset of providers willing to absorb a financial loss can diminish rapidly, and function as a de facto denial of adequate medical care.

The impact of insufficient inpatient reimbursement on patients and providers can include:

- shifting patients to other providers or to outpatient departments, particularly where 340B discount drugs available;
- decreased provider willingness to utilize orphan drugs indicated for the patient's condition; and/or
- perpetuating payment inadequacies by spreading the incremental cost of treating rare disorders over a potentially diverse MS-DRG so that some conditions are “winners” and others are “losers.”

Our comments reflect our commitment to individuals with rare and ultra-rare diseases and their families. [bulleted list once order and heading language are finalized]

**Haystack Project appreciates CMS' recognition of the unique challenges associated with emerging treatments and its interest in ensuring sufficient reimbursement for, and access to CAR-T in the inpatient setting.**

Over the past two years, CMS has considered challenges to the IPPS presented by the emerging CAR-T therapies, recognizing that (1) reimbursement mechanisms present potential access hurdles for CAR-T; (2) treating all therapies for all conditions equally can create and maintain inequities in access to important therapies in the most clinically appropriate setting; and (3) the above realities warrant treatment- and/or population-specific considerations, mechanisms, or exceptions. Modifications to reimbursement mechanisms, particularly in the inpatient setting, are both appropriate and necessary. The underlying goal – patient access to the right therapy in the right setting – requires similar “fixes” wherever IPPS structural deficiencies inhibit or may inhibit existing and future therapies.

Haystack Project believes that the issues CMS recognizes, and the solutions it creates, for inpatient CAR-T payment will have repercussions for existing and future treatments addressing rare diseases in the inpatient setting. We support pragmatic approaches that acknowledge the unique attributes of higher-cost drugs. Specifically, our understanding is that the acquisition cost for the current two CAR-T products is relatively consistent across geographic areas. Unfortunately, CMS' methodologies for calculating NTAP payments, and its outlier and MS-DRG payment calculations are at odds with each other and drive substantial variability in provider charging practices. While some hospitals will set charges for CAR-T to enable the full NTAP payment, others might seek to reflect the applicable cost-to-charge ratio. Provider concerns about the optics of drug charges in the millions of dollars can drive other facilities to set charges approximating acquisition costs. None of these charging practices will “work” across CMS' inpatient payment methodologies, and the aggregate data on costs associated with CAR-T will remain inherently unreliable.

We fundamentally believe that CMS' reliance on using cost-to-charge ratios and applying geographic adjustments result in payment distortions rather than reflecting the cost of care. We urge CMS to use the actual product acquisition cost in its NTAP and outlier formulas for higher-

cost drugs so that hospitals can recoup the cost of the drug without employing charging mechanisms to neutralize the impact of charge compression.

We are, however, concerned that if payment inadequacies converge with coverage requirements under a Coverage with Evidence Development (CED) paradigm like that proposed for CAR-T, Medicare beneficiary access will be limited to facilities willing to absorb both a financial loss and an additional administrative burden. Haystack Project expects that as newer, high-cost targeted therapies emerge to address very small patient populations, Medicare Advantage plan requests to initiate the national coverage process in the wake of product launch will become the rule, rather than the exception. The ability to trigger a carve-out of costs associated with a new treatment for payment under Medicare fee-for-service is a substantial financial incentive that plans are unlikely to forego.

As detailed more fully below, access issues proliferate in the face of inpatient reimbursement deficiencies even when the standard of care is long-established. Patients can, however, take a level of comfort in the fact that Medicare participating hospitals cannot lawfully avoid financial losses by declining to provide appropriate treatment. They can also maintain the hope that CMS will enforce hospitals' obligations with real-time recourse for patients. If, however, CMS responds to a national coverage request with CED coverage requirements for CAR-T or any future emerging therapy, facilities can avoid losses associated with deficient Medicare payment without running afoul of the conditions of participation, and without taking any affirmative action. Hospitals could simply avoid compliance with the CED requirements, and provide patients with notice that the service would be non-covered.

Haystack Project urges CMS to ensure that its payment policies for existing and emerging treatments are sufficient to meet Medicare's obligation to cover and pay the costs of medically necessary care in all settings, regardless of the cost of treatment or the rarity of the disease.

***Haystack Project urges CMS to devise a mechanism to account for costs of treating rare and ultra-rare disorders with orphan drugs.***

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 to accommodate the cost of existing and emerging treatments. 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 that is relatively homogenous on treatment modalities and care costs.

In CMS' 2019 update to the IPPS, the Agency considered whether patient-reported access hurdles and an average cost of care that doubled the MS-DRG average were sufficient justifications for changing the MS-DRG assignment for porphyria patients. CMS ultimately decided that the relatively low volume of Medicare claims for this extremely rare disease was the determinative factor in declining to act. The Agency did, however, acknowledge the impact that inpatient reimbursement deficiencies could have on patients and their access to care, suggesting

that CMS would consider devising mechanisms to account for the costs of treating one high-cost rare diagnosis within a larger MS-DRG. The stark realization that patients with a very rare condition cannot reliably access a standard of care that has been recognized for over 35 years must be met with action, not acquiescence. We urge CMS to make this a priority.

We urge the Administration to build upon its learnings from the CAR-T example and:

- 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 for the MS-DRG as a whole;
- Continue its stakeholder outreach in an effort to identify specific rare disorders currently grouped within MS-DRGs for which the payment is well below the average cost for providing care;
- Develop a methodology to calculate and recalibrate MS-DRG relative weights and assignments to accurately account for patients with rare diseases requiring new or existing orphan drugs; and
- Consider applying an extended NTAP period for treatments addressing extremely small patient populations.

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

The policies, structures, and payment mechanisms CMS devises and refines for the Medicare program can significantly impact inpatient reimbursement for both commercial payers and the state Medicaid programs. As noted above, the technologies that have emerged in recent years present new challenges for CMS, and we believe the Agency's response could shape the landscape for future innovations across payers and patient populations. We believe that patients and caregivers can be instrumental in informing CMS toward policies that enable, rather than impede, access to life-saving treatment innovations for patients with rare diseases, including children and young adults.

The patient and caregiver advocacy organization participants in Haystack Project 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 some patients, inpatient administration may remain the best option. Appropriate inpatient coding and payment mechanisms are, therefore, crucial in ensuring access to care for all patients.

The MS-DRG system, though designed and refined to accommodate the Medicare program and its beneficiary demographic, drives inpatient payment for many commercial plans as well as Medicaid programs in 26 states and the District of Columbia. Haystack Project is concerned that decisions based solely on statistical analyses in the Medicare program could have an unintended impact on pediatric and young adult access to care. 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.

This approach will protect access for Medicare beneficiaries while ensuring that patients covered by other payers relying on CMS' MS-DRG structure are not overlooked.

**Haystack Project encourages CMS to increase the maximum NTAP payment from 50% to a level that is sufficient to compensate providers for their acquisition costs.**

Haystack Project agrees with CMS' goal of ensuring that reimbursement structures keep pace with emerging treatments that address serious unmet health care needs, and potentially deliver sustained improvement in patient outcomes. We, therefore, applaud CMS' recognition that the existing NTAP rate is likely insufficient to enable patient access to these new treatments and technologies. Although we agree that the proposed increase in NTAP rate is a clear improvement over the existing calculation, we remain concerned that the marginal increase will not fully address the access concerns expressed by stakeholders and shared by CMS

We are concerned that a one-size-fits-all rate calculation ignores the fact that, as long as the NTAP fails to fully address reimbursement deficiencies associated with incorporating a new technology, the magnitude of any financial shortfall will always be proportional to the cost of the technology. We expect that when higher-cost treatments generate a proportional financial loss, access hurdles will also correlate with the cost of the new technology. We ask that CMS devise an NTAP rate calculation that will be sufficient to ensure that financial losses associated with Medicare reimbursement do not hamper patient access to new treatment options offering substantial benefit. We believe that the most accurate calculation would subtract cost of treatments avoided from the acquisition cost of the new technology replacing those treatments and urge CMS to adopt this methodology. CMS should, at a minimum, consider rate calculations that incorporate a graduated percentage based upon acquisition cost. This would disrupt the direct proportionality between treatment cost and hospital financial shortfalls that is inherent in a flat-percentage methodology, and could reduce the likelihood of significant access hurdles as treatment costs rise.

Haystack Project supports CMS' interest in addressing NTAP payment inadequacy as a crucial step toward ensuring that Medicare beneficiaries maintain access to innovative treatments. We also urge CMS to consider more frequent reviews of NTAP applications. Quarterly updates of the set of treatments eligible for NTAP payment, would substantially reduce the access hurdles encountered in the timeframe between first marketing of a new treatment and assignment of an NTAP.

**Haystack Project urges CMS to refine its NTAP review criteria and incorporate patient-centered considerations into its assessments.**

Haystack Project believes that the NTAP process has significant potential to improve Medicare beneficiary access to the emerging treatment options that may most appropriately address their condition(s). We have, however, remained concerned that CMS' review of NTAP applications has evolved away from the intended NTAP goal of ensuring that providers are appropriately paid for treatment involving new technologies. We strongly support updates to the NTAP process to

make it more patient-focused so that new technologies offering improvements on outcomes patients care most about are adopted into the healthcare delivery system.

When evaluating new technologies for hospital inpatient use, CMS requires a “substantial clinical improvement” over existing technologies. In assessing the existence or magnitude of “improvement,” however, the Agency generally fails to consider clinical improvements that may be of particular relevance to Medicare beneficiaries. Instead, CMS has historically focused on whether the technology “reduces mortality, decreases the number of hospitalizations of physician visits, or reduces recovery time comparable to the technologies previously available.” This very narrow view of treatment efficacy conflicts with both CMS’ stated goal of placing patients at the center of its policy decisions and FDA’s incorporation of patient reported outcomes in its approval decisions. Haystack Project views the NTAP application review process as a clear example of an area for which refinements incorporating patient values and patient-centered outcomes are essential.

Haystack Project supports CMS’ proposal to apply a presumptive approach to the newness and not substantially similar prongs of the NTAP criteria, whereby devices that have received breakthrough designation from the FDA would satisfy both of those criteria. We agree that a breakthrough technology, designated as such by FDA, should be deemed to meet these NTAP criteria without further CMS inquiry. We are concerned, however, with CMS’ preliminary decision not to apply this pragmatic and efficient approach to drugs because “the current drug pricing system provides generous incentives for innovation, but too often fails to deliver important medications at an affordable cost.” The only cost inquiry provided in the statutory provisions authorizing the NTAP was a simple assessment of whether the cost is sufficient to meet a threshold, not whether the new technology is over-priced in relation to CMS’ perception of the value conveyed by new drugs and biologics generally.

Haystack Project is concerned that CMS has conflated its system-wide priority of reducing drug costs with the NTAP inquiry on whether a technology is new and not substantially similar to an existing therapy. We agree that the presumptive approach is sensible and that it reduces the uncertainties inherent in a more subjective approach. We urge CMS to incorporate it even-handedly in determining whether a new treatment with breakthrough status meets the NTAP requirements regardless of whether it is a device, drug, or biologic.

We note that CMS has also sought feedback on potential revisions to the substantial clinical improvement criterion for IPPS NTAP that may inform future rulemaking activities. Haystack Project strongly urges CMS to implement infrastructural mechanisms for patient engagement so that it is better able to align NTAP review with FDA’s patient-centric approach. We believe that patient preferences are an essential element of any “substantial improvement” analysis, and even more so for decisions that, like NTAP review, could shape new technology adoption and patient access.

## **Conclusion**

Haystack Project appreciates the opportunity to offer its comments and suggestions as CMS finalizes its update to the Medicare IPPS. Now more than ever, science and innovation are

progressing at a rapid pace that offers individuals with serious, life-limiting conditions and their families real hope of seeing a treatment or cure in their lifetime. We are encouraged by CMS' proposed rule, and look forward to a continuing dialogue.

If you have questions or need further information, please do not hesitate to contact Saira Sultan at 202-360-9985.