June 17, 2022

Ms. Chiquita Brooks-LaSure  
Administrator  
Centers for Medicare & Medicaid Services  
U.S. Department of Health and Human Services  
7500 Security Boulevard  
Baltimore, MD 21244

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:

The Rare Access Action Project, (RAAP) appreciates the opportunity to comment on the Centers for Medicare & Medicaid Services’ (CMS) proposed payment updates to the fiscal year 2023 Hospital Inpatient Prospective Payment System for Acute Care, Long-Term Care and Quality Programs (CMS-1771-P) (Proposed Rule).1 RAAP appreciates CMS’ numerous proposals to modify the NTAP process but urges CMS to treat all clinical trial information submitted in the NTAP application as confidential proprietary information not to be publicly disclosed until FDA approval. Additionally, RAAP agrees with CMS that a new reimbursement methodology should be established for therapies treating rare diseases that are often low volume and urges the agency to separately reimburse these drugs post NTAP based on the transparent and market based Average Sales Price Methodology.

RAAP is a registered 501(c)(4) non-profit organization that is a coalition of life sciences and patient stakeholders that explore creative policy solutions to address structural issues in access and coverage. Our priority is to help ensure rare

disease patients have access to the care and treatments they need and submits the following comments consistent with that objective.

I. Background: Rare Diseases and Orphan Products

As CMS states, the Orphan Drug Designation Program provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the US, or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug. Rare diseases include more familiar conditions, such as cystic fibrosis, Lou Gehrig’s disease, and Tourette’s syndrome, as well as less familiar conditions, such as aromatic L-amino acid decarboxylase (AADC) deficiency, Duncan’s Syndrome, Madelung’s disease, and acromegaly/gigantism. These conditions are complex and often not well understood, which causes great challenges to the diagnosis and treatment as well as research efforts.

Rare disease treatments range from curing the disease, modifying how the disease functions, or treating the symptoms. Truly curative treatments are rare. Disease-modifying therapies target the underlying pathology of a disease to prevent it from worsening. Symptomatic treatments seek to temper symptoms or to maintain physical, emotional, and mental functioning.

Only 5% of rare diseases have a treatment approved by the Food and Drug Administration (FDA) and for one-third of individuals with a rare disease, it can take between one and five years to receive a proper diagnosis. Patients with rare diseases often seek treatment in clinics where the condition has never been seen before and have symptoms that are absent, masked, misunderstood, or confused, which often leads to delayed diagnosis further complicating the patient’s and family’s arduous journey. Half of all patients diagnosed with a rare disease are children, and as many as 3 in 10 children with a rare disease will not live to see their 5th birthday. RAAP agrees with CMS that there are several barriers that providers face in treating beneficiaries with these orphan designated drugs in the Medicare hospital inpatient setting. RAAP therefore appreciates CMS’ willingness to engage the healthcare stakeholder community for feedback on how “to mitigate any

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unintended negative payment impacts to providers serving patients with rare
diseases or conditions that are represented by low volumes in our claims data.”5

II. CMS Should Separately Reimburse Low Volume High-Cost
Drugs Post NTAP

RAAP encourages and thanks CMS for its efforts to create accurate and
transparent payment rates to promote access to rare disease therapies that face
many access issues in the Medicare hospital inpatient setting. As CMS states “it is
not appropriate for facilities to deny treatment to beneficiaries needing a specific
type of therapy or treatment that involves increased costs.”6 Consequently, RAAP
believes that CMS should hold these facilities more accountable for their denials and
delays and therefore urges CMS to publicly state the time it takes for each facility
to cover and/or dispense the rare disease therapy thereby holding hospitals
accountable for their unnecessary delays in access.

Similarly, RAAP knows that this same problem exists in Medicaid. Some
Medicaid programs employ a review process that can take between 180 to 365 days
(or longer) to conclude, before there is any real access to a rare disease medicine.
This is unacceptable when many of those rare diseases are life threatening, and
patients, many of whom are pediatric, do not have the luxury of time to wait for
Medicaid to make a therapy available to patients one or two years after FDA
approval. RAAP believes that FDA-approved drugs with a rebate agreement must be
covered from the outset; there is no prescribed legal period to delay coverage
based on the theory that a state must review it or make a coverage determination.
So, while a state may review a drug through its Pharmacy and Therapeutics (P&T)
process, that drug must be made available prior to and post review according to its
medically accepted indication. Consistent with CMS health equity principles, RAAP
therefore urges CMS to issue a State Release reminding states that all drugs,
particularly orphan drugs must be covered upon FDA approval according to the
label.

RAAP supports CMS’ conclusion that when “Medicare reimbursement is
insufficient to cover the costs of certain therapeutics that treat patients with rare
diseases, a disincentive can be created in addressing these conditions.”7 RAAP is
pleased that CMS seeks comments for potential solutions to offset this barrier.

5 Id. at 28,197.
7 87 Fed Reg. at 28,197.
RAAP greatly appreciates, however, the inherent conflict that exists with certain potential solutions. Specifically, because MS–DRGs are a classification system intended to group together diagnoses and procedures with similar clinical characteristics and utilization of resources, MS-DRGs cannot work well for rare disease treatment. Rare diseases, by definition, are conditions that are represented by low volumes in CMS’ claims data, thereby posing a unique challenge to the MS-DRG methodology as these conditions affect small subsets of the population.

To resolve this conflict, RAAP urges CMS to develop a policy that maximizes beneficiary access to the rare disease therapy over historical approaches MS-DRG development. As such, RAAP recommends that CMS reimburse hospitals the Average Sales Price (ASP) of the orphan drug as published in the HOPD Addendum B file. CMS can implement this policy pursuant to §1886(d)(5)(I) that states “[t]he Secretary shall provide by regulation for such other exceptions and adjustments to such payment amounts under this subsection as the Secretary deems appropriate.” This is the same authority that the Agency relied on to make the recent COVID payment adjustments. RAAP believes that the ASP methodology will preserve access to the therapy, can be consistently applied, is transparent and is market based. Further, this methodology is the same as the outpatient setting such that CMS can eliminate reimbursement from the site of care decision. ASP can also help hospitals meet their own transparency requirements, and therefore is the best option.

III. RAAP Urges CMS to Extend All Low Volume High Cost NTAPs That Are Scheduled to Expire September 30, 2022

CMS proposes to discontinue the new technology add-on payment for FY 2023 for those technologies that were set to expire on September 30, 2022, and that were extended due to the COVID-19 public health emergency. In light of CMS’ seeking comments on how to preserve access to orphan drugs post NTAP, RAAP urges CMS to continue making NTAP payments for these drugs. CMS can again use its authority under SSA §1886(d)(5)(I) to extend these payments by at least one year. The one-year extension will afford CMS the time to develop a

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9 In circumstances where ASP is not available, ARM urges CMS to reimburse hospitals based on WAC consistent with §1847A(c)(4)(A)(i).
transition policy for these drugs thereby ensuring consistent and appropriate access to these therapies for newly diagnosed beneficiaries.

IV. New Diagnosis Codes Should Be Implemented as Soon As Possible and Greater Clarity into the Process is Needed

The lack of an ICD-10 Code for every rare disease presents many challenges in our healthcare system. Without an ICD-10 code for each rare disease identified; there is no way to determine the prevalence of the approximately 7000+ rare diseases in the US and also the cost of treating each rare disease. This lack of data from not having an ICD-10 code is frustrating. Scientists have no way of knowing which diseases can be fast tracked for a treatment when they do not know the numbers that make up that disease or if there is a need for more research in a specific indication if the prevalence is not identified. The urgency of getting these ICD-10 Codes identified will enable these therapies to be recognized and covered much sooner contributing to a much better quality of life for our rare disease patients and caregivers.

Therefore, RAAP requests further detail from CMS/CDC on some of the decision-making processes in determining a modification to the diagnosis code set. Currently, proposals for a new code should include, a description of the code(s)/change(s) being requested, rationale for why the new code/change is needed (including clinical relevancy) and supporting clinical references and literature.11 Further, the CDC/NCHS states that each proposal should be consistent with the structure and conventions of the classification.12 Finally, once proposals are reviewed by the Committee all requestors will be contacted as to whether the proposal has been approved for presentation at the ICD-10 Coordination and Maintenance Committee meeting or not.13

What is not clear to RAAP or other stakeholders are the criteria that are used to determine if a proposal has been included to the presentation agenda at the ICD-10 Coordination and Maintenance Committee meeting. This basic information is not stated in the CDC/NCHS’ website or any Agenda packet. Consequently, RAAP urges the Agency co-chairs to clarify the criteria it relies on for a request to be presented

11 [https://www.cdc.gov/nchs/icd/icd10_maintenance.htm](https://www.cdc.gov/nchs/icd/icd10_maintenance.htm)
12 Id.
13 Id.
publicly at an ICD-10 Coordination and Maintenance Committee meeting. RAAP believes that this information will provide needed transparency to the application process that in turn will increase the quality of each request.

Should a request be presented at a public meeting there is no publicly available information that details the criteria connecting the results of that presentation to acceptance in the code set. In other words, the agency co-chairs do not share any of the public comments it receives related to any proposal, does not disclose its responses to these comments and in general provides no guidance from the time the meeting concludes to rulemaking as to whether the proposal will be included in the ICD-10 CM code set. Rather, the CDC/NCHS publishes final codes in the IPPS proposed rule and only provides stakeholders with the ability to comment on which MS-DRGs those codes are mapped. As an initial step, the CDC/NCHS should include some public rational regarding why it made its decisions on which codes to add and why to exclude the remaining codes. RAAP and the patient groups and physician specialty societies that we work closely with deserve greater clarity and detail into why these decisions are made on such important issue as new diagnosis codes.

RAAP therefore urges CMS to work with the CDC/NCHS to use this rule making process to establish an expedited diagnosis code modification process as of April 1, 2023 and provide the much-needed clarity on many of the criteria the CDC/NCHS uses in considering a modification to the ICD-10-CM code set and the transparency behind its decision-making process.

V. Conclusion

Thank you for the opportunity to submit these recommendations to the Agency’s Proposed Rule. We look forward to working with CMS to further develop policies that maximize access to therapies treating rare diseases. Please feel free to contact me by email at Mike@rareaccessactionproject.com.

Sincerely,

Michael Eging