February 21, 2024

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Blvd.
Baltimore, MD 21244-1850

Dear Administrator Brooks-LaSure,

The National Organization for Rare Disorders (NORD) is writing to request CMS' assistance in ensuring that Medicare beneficiaries with rare diseases have access to the treatments they need in the hospital inpatient setting. CMS has previously acknowledged that hospitals who provide care for rare disease patients may receive insufficient reimbursement in the inpatient setting, which may create a disincentive for hospitals to use costly drugs to treat rare disease patients.[1] To help ensure rare disease patients have access to necessary therapies while hospitalized, NORD urges CMS to rectify this problem in the forthcoming Hospital Inpatient Prospective Payment System (IPPS) rule.

NORD is a unique federation of non-profit and health organizations dedicated to improving the health and well-being of people with rare diseases by driving advances in care, research, and policy. NORD was founded 40 years ago, after the passage of the Orphan Drug Act (ODA), to formalize the coalition of patient advocacy groups that were instrumental in passing this landmark law. Since that time, NORD has been advancing rare disease research and funding to support the development of effective treatments and cures; raising awareness and addressing key knowledge gaps; and advocating for policies that support the availability of and access to safe and effective diagnostics and therapies.

DRGs are set according to the average resources required to care for cases in a particular DRG. However, rare disease patients are, by definition, not average. The National Center for Advanced Translational Sciences (NCATS) has found that individual medical costs for people with a rare disease may be 3-5 times higher than individual medical costs for people without a rare disease.[2] Resource intensity is frequently significantly higher for rare disease patients, resulting in increased costs to the provider without commensurate payment. While providers treating patients in a hospital outpatient setting are able to bill directly for the cost of the treatment, providers

[1] 87 FR 90, 28195 (May 10, 2022)
[2] Tisdale, A., Cutillo, C.M., Nathan, R. *et al.* The IDeaS initiative: pilot study to assess the impact of rare diseases on patients and healthcare systems. *Orphanet J Rare Dis* **16**, 429 (2021). https://doi.org/10.1186/s13023-021-02061-3

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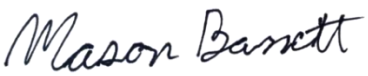
treating patients in the inpatient setting are required to accept the DRG for the primary diagnosis of the patient if a secondary DRG is not available that accounts for the cost of the treatment. While we recognize that current mechanisms exist to mitigate extraordinary losses for high-cost cases, such as NTAPs and outlier payments, these supplemental payments are frequently unable to recoup the full extent of the losses suffered by health systems when treating rare disease patients. Indeed, a 2020 study found that in fiscal year 2019, 6 of the 18 technologies approved for an NTAP may still incur potential hospital losses of greater than $10,000, including 3 greater than $50,000, despite the NTAP.[3]

Beyond fiscal challenges to the hospital, we are concerned that insufficient reimbursement could disincentivize the use of clinically indicated therapies. Anecdotally, we have heard that certain hospitals and health systems have developed stringent access protocols to limit prescription and administration of rare disease products that the patient may otherwise be able to access according to their regular regimen in the outpatient setting due to the limited reimbursement they would receive.

NORD requests CMS identify options to rectify this issue in the forthcoming 2025 IPPS Proposed Rule, which could include a cost to charge ratio of 1:1 for certain rare disease drugs, add-on payments for products identified as receiving insufficient reimbursement, or a rare disease specific DRG. In 2021, NORD launched the "Rare Disease Centers of Excellence" program to recognize and collaborate with hospitals and medical institutions across the country dedicated to diagnosing, treating, and researching all rare diseases. NORD's Centers of Excellence could be an invaluable resource for gaining firsthand perspective from rare disease clinicians on the scope of the issue and best options for amelioration and NORD would welcome the opportunity to work with CMS to better understand the issue and identify potential solutions.

With any questions, please do not hesitate to reach out to Mason Barrett, Policy Analyst at mbarrett@rarediseases.org or Karin Hoelzer, Director of Policy and Regulatory Affairs at khoelzer@rarediseases.org.

Thank you for your consideration,



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[3] Manz CR, Bekelman JE, Doshi JA. The Changing Characteristics of Technologies Covered by Medicare's New Technology Add-on Payment Program. *JAMA Netw Open.* 2020;3(8) doi:10.1001/jamanetworkopen.2020.12569