June 25, 2018

The Honorable Seema Verma, Administrator
Centers for Medicare and Medicaid Services
Department of Health and Human Services
Hubert H. Humphrey Building, Room 445-G
200 Independence Avenue, SW
Washington, D.C. 20201

RE: Comment on CMS-2018-0046-0002 ("Medicare Programs: Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and Long Term Care Hospital Prospective Payment System and Proposed Policy Changes and Fiscal Year 2019 Rates; etc.")

Dear Administrator Verma,

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) would like to thank the Centers for Medicare and Medicaid Services (CMS) for the opportunity to provide comments on the proposed rule titled, "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 2019 Rates."

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. Since 1983, we have been committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD strongly believes that all patients should have access to quality and affordable health care that is best suited to their medical needs. One of NORD's responsibilities is to ensure that we represent the patient each and every time a decision by the Federal Government can influence, or perhaps hinder, access to care.

Many individuals with rare diseases rely on the Medicare program to cover their hospital needs, making the Inpatient Prospective Payment Systems (IPPS) influential in ensuring they receive appropriate care. When implemented rigidly or without consideration of advances in innovation, however, it is possible for these systems to harm our patients by creating a barrier to access. Notably, we are concerned about the potential for the IPPS to inflexibly and problematically reimburse for innovative therapies for rare diseases.

Overall, we believe several of the proposals within this proposed rule are a step in the right direction. While CMS proposes many policies within this proposed rule, our comments will

focus on those that we believe could particularly influence our patients' access to medically necessary care.

Proposed Changes to Medicare Severity Diagnosis-Related Group Classifications

Obtaining appropriate care can be particularly challenging for rare disease patients. Currently, it is estimated that it takes, on average, five to seven years for a rare disease patient to acquire a diagnosis. Further, once a patient secures a diagnosis, it is unlikely that there will be a corresponding treatment that has been approved by the Food and Drug Administration (FDA), given that approximately 95 percent of the 7,000 rare diseases are still without a therapy indicated to treat the disease.

Fortunately, science continues to advance rapidly and patients that were once without any possibility of receiving targeted treatments for their condition now may soon receive such therapies. Therefore, we at NORD are particularly eager to ensure these exciting new treatments will be accessible to the Medicare population upon approval.

Accordingly, we are pleased that CMS is seeking to ensure that the IPPS is not inappropriately limiting access. For example, CMS' IPPS proposals for one such innovative new class of therapies, Chimeric Antigen Receptor (CAR) T-cell therapy, is illustrative of such efforts and is hopefully a signifier of future policymaking.

Presently, CMS utilizes the New Technology Add-on Payment (NTAP) system to facilitate access to new technology. However, it is our understanding that this system may be insufficient for enabling access to innovative therapies, as is the case for CAR T-cell therapies. We are concerned that access to medically-necessary rare disease care in the hospital setting may be hindered if CMS is unable to implement a more permanent and effective policy for reimbursing innovative new orphan products.

For these reasons, we are appreciative of CMS' consideration of alternative reimbursement approaches to such innovative therapies for rare disease patients. We will leave the intricacies of deciding whether to assign CAR T-cell therapies to a new Medicare Severity Diagnosis-Related Group (MS-DRG), implement a cost to charge ratio (CCR) of 1.0, or create an entirely new MS-DRG to the experts on the IPPS. We do, however, wish to emphasize our support for a system that recognizes the value that these transformative therapies bring to our patients.

Further, in the proposed rule, CMS acknowledges that, "many members of the public have noted that the combination of the new technology add-on payment applications, the extremely high cost of these CAR T-cell therapy drugs, and the potential for volume increases over time present unique challenges with respect to the MS-DRG assignment."[1]

While CAR T-cell therapies may present unique challenges compared to other available therapies, we anticipate many similarly innovative new orphan drugs will present the very same challenges in the near future. Therefore, we urge CMS to move forward with a sustainable approach that will ensure access to all future innovative therapies. We believe that whatever

[1] CMS-1694-P. 2018. Pg. 20189.

CMS decides to proceed with must contain enough flexibility and foresight to incorporate what we can only hope will be an immense growth in paradigm-shifting therapies.

Finally, while we recognize and appreciate CMS' desire to incentivize lower drug prices, we urge CMS to ensure this valid effort does not hinder patient access to life-changing, even life-saving therapies. Patients are in great need of innovative therapies, and their health and wellbeing cannot be compromised by fiscal reservations.

Requirements for Hospitals To Make Public a List of Their Standard Charges via the Internet

NORD is greatly appreciative of CMS' efforts to increase pricing transparency for patients, and we share CMS' goals. Our patients have experienced many of the issues that CMS explains, including facing unexpected physician out-of-network charges for in-network hospital stays and receiving incomprehensible pricing information from their hospitals.

We do not wish to comment at this time on the specific proposals CMS has put forward as we are unfamiliar with the nuanced impacts that each of these proposals will have on participating hospitals. However, we strongly encourage CMS to continue these efforts, and we support CMS promulgating regulations that will increase transparency in hospital-related costs for our patients.

NORD thanks CMS once again for the opportunity to comment. We look forward to working with CMS to ensure that rare disease patients will have access to transformative therapies. For questions regarding NORD or the above comments, please contact me at pmelmeyer@rarediseases.org or 202-545-3828.

Sincerely,

Paul Melmeyer
Director of Federal Policy