July 10, 2020

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-1735-P ("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 2021 Rates; etc.")

Dear Administrator Verma,

The National Organization for Rare Disorders (NORD) thanks the Centers for Medicare and Medicaid Services (CMS) for the opportunity to provide comments regarding the "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 2021 Rates; Quality Reporting and Medicare and Medicaid Promoting Interoperability Programs Requirements for Eligible Hospitals and Critical Access Hospitals." (IPPS) proposed rule for fiscal year 2021.

NORD is a unique federation of voluntary health organizations dedicated to helping the 30 million Americans living with a rare disease. Many individuals with rare diseases rely on the Medicare program to cover their hospital needs, making the IPPS influential in ensuring they receive timely and appropriate care. NORD strongly believes that all patients should have access to quality and affordable health care that is best suited to their medical needs. We appreciate CMS's ongoing commitment to reducing financial barriers to treatment, and are pleased to offer comments on the following provisions of the IPPS proposed policy changes:

1. Proposed Changes to Specific MS-DRG Classifications, Chimeric Antigen Receptor T-Cell Therapies
2. Proposed Changes and Technical Clarification to The Alternative Pathway for Certain Antimicrobial Products

New MS-DRG for Chimeric Antigen Receptor (CAR) T-cell Therapy

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 an accurate diagnosis. Further, once a patient secures a diagnosis, it is unlikely that there will be a

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corresponding treatment that has been approved by the Food and Drug Administration (FDA), given that approximately 95 percent of the 7,000 known rare diseases are still without a therapy indicated to treat the disease.

Fortunately, science continues to advance rapidly, and many rare disease patients who were once without any possibility of receiving targeted treatments for their condition may soon have new therapeutic options. As representatives of the rare disease community, NORD is particularly eager to ensure that groundbreaking new therapies are accessible to the Medicare population upon approval. Inadequate reimbursement can significantly limit the ability of rare disease patients to access these innovative treatments, as providers are unlikely to offer care and treatments for which they will not be sufficiently compensated.

Accordingly, we are pleased that CMS has proposed to create a new Medicare Severity Diagnosis-Related Group (MS-DRG) specific to Chimeric Antigen Receptor T-Cell (CAR-T) therapy. This change would provide a predictable payment rate for hospitals administering CAR-T and is an approach that we hope will be replicated in future policymaking.

NORD anticipates that many similarly innovative orphan drugs and therapies will present comparable reimbursement and coverage challenges in the future. We encourage CMS to finalize this proposal, and recommend the further development of a sustainable approach to ensure future innovative therapies are accessible to individuals with rare diseases.

Proposals for New Technology Add-On Payment Pathway for Certain Antimicrobial Products

NORD is supportive of CMS's proposal to streamline the new technology add-on payment (NTAP) pathway and facilitate add-on payments for medical products that have received FDA approval through the Limited Population Pathway for Antibacterial and Antifungal Drugs (LPAD). Many rare disease patients are immune-compromised, putting them at increased risk of contagion and serious illness. As advocates for the rare disease community, NORD has a keen interest in insuring that appropriate incentives exist to promote the development of new antimicrobial products.

If this proposal is finalized, antimicrobial drugs approved under the LPAD pathway would be considered new, and not substantially similar to an existing technology and would not need to demonstrate that it meets the substantial clinical improvement criterion. The antimicrobial drug would still need to meet the cost criterion for NTAP payments. This proposal simplifies the NTAP pathway for these drugs, as is currently the case for Qualified Infections Disease Products (QIDPs).

In addition, we reiterate our comments from previous years that CMS should expand upon this idea, and seek greater alignment with all of the expedited FDA pathways. For example, programs at FDA such as the Fast Track, Accelerated Approval, and Breakthrough Therapy may also be appropriate candidates for a similarly streamlined pathway to obtain an NTAP. There are many conditions in the rare disease community for which immediate access to treatment

can prevent grave harm, possibly even death. For these patients, CMS should be doing everything possible to reduce the amount of time between approval and coverage in a responsible manner. NORD believes that the NTAP, by supplementing reimbursement for truly innovative therapies, is a critical aspect of ensuring access.

CMS also proposes to give conditional NTAP approval to QIDPs and LPAD designated products to allow add-on payments to begin in the quarter following the FDA marketing approval. We support this proposal and again encourage CMS to apply this policy more broadly, and allow conditional approval for other designations. Currently, there is only one annual opportunity for products to apply for an NTAP. This means that many months may pass between the marketing approval for a new therapy, and patients access to the treatment. Conditional NTAP approval of therapies may substantially reduce access issues encountered by rare disease patients.

Conclusion

NORD is a unique federation of health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services. NORD aims to ensure that the perspective of the patient is considered each time a decision by the Federal government can impact access to care.

We once again thank CMS for the opportunity to comment on this proposed rule. We look forward to continuing to work with the agency to ensure that rare disease patients have timely access to transformative therapies. For questions regarding NORD or the above comments please contact Corinne Alberts at calberts@raredisease.org.

Sincerely,

Rachel Sher
Vice President, Policy and Regulatory Affairs