June 24, 2019

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-2019-0073-0003 ("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; etc.")

Dear Administrator Verma,

On behalf of the 30 million Americans with one of the over 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 2020 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, affordable health care that is best suited to their medical needs. 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.

Obtaining appropriate care can be particularly challenging for rare disease patients. It can often take 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 over 90 percent of the 7,000 rare diseases are still without an approved therapy indicated to treat the disease.

Fortunately, science is advancing rapidly and patients who were once without any possibility of receiving targeted treatments for their condition now may soon have an approved therapy available to them. NORD believes these treatments should be accessible to the Medicare population upon approval.

NORD is concerned about potential barriers to access resulting from the current Medicare hospital inpatient prospective payment systems (IPPS). NORD recognizes that the high cost of rare disease therapies also creates a barrier to access and is determined to tackle this barrier as well. However, addressing the high cost of drugs does not eliminate the need to guarantee that systems for reimbursement are not slowing the process of delivering innovative therapies. As new therapies and devices emerge, NORD urges CMS to ensure that the systems in place to reimburse for these technologies are capable of setting rates that are appropriate for the value these therapies bring and can adequately incentivize providers to offer patients access.

Consequently, we thank CMS for the reforms contained within this proposed rule that reflect the concerns of the rare disease community. Specifically, our comments below are focused on the provisions of the proposed rule pertaining to the new-technology add-on payment (NTAP).

Request for Information on the NTAP Substantial Clinical Improvement Criterion

NORD believes that the NTAP, by supplementing reimbursement for truly innovative therapies, is a critical aspect of ensuring access. NORD appreciates CMS' acknowledgement of the necessity for greater clarity around what qualifies as a "substantial clinical improvement" in the context of an application for an NTAP. As mentioned in the proposed rule, a better understanding of what this criterion demands will allow for greater predictability and will allow manufacturers to better identify what information to include in their application. NORD encourages CMS to work closely with FDA to further outline the requirements of this criterion so as to eliminate redundancies and facilitate faster access to truly deserving therapies.

NORD commends CMS for identifying FDA's Breakthrough Devices Program in the proposed rule as being a metric worthy of unique consideration. NORD believes CMS could expand upon this idea, however, and seek greater alignment with all expedited FDA pathways, such as the Fast Track, Accelerated Approval, and Breakthrough Therapy programs.

Proposed Alternative Inpatient NTAP for Transformative New Devices

NORD is highly supportive of CMS's proposal to create a distinct pathway for devices that have received FDA marketing authorization through the Breakthrough Devices Program. The devices that are a part of FDA's Breakthrough Devices Program hold the promise of tremendous benefit to their indicated patient populations, and we appreciate CMS' recognition that they should not be held up by determinations that are duplicative of ones that have already been made by FDA.

The Breakthrough Devices Programs, however, is not the only program at FDA that is deserving of this paradigm. NORD asks that CMS partner with FDA to determine further potential for shared metrics. Programs at FDA such as the Fast Track, Accelerated Approval, and Breakthrough Therapy programs may also be appropriate candidates for a similarly distinct 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 access in a responsible manner.

Proposed Revision of the Calculation of the Inpatient Hospital NTAP

NORD applauds CMS for taking stakeholder concerns into consideration and raising the maximum add-on payment from 50 to 65 percent. This is undoubtedly a step in the right direction. NORD seeks to eliminate the possibility that providers might decline using new technology for fear that they will not be able to procure the reimbursement necessary to avoid financial loss. Raising the maximum add-on payment to 65 percent helps to diminish that possibility. NORD will monitor implementation of this policy to assess whether an additional increase may be necessary in the future.

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 rsher@rarediseases.org or 202-545-3970.

Sincerely,

/s/

Rachel Sher
Vice President of Policy and Regulatory Affairs