National MPS Society

Support for Families. Research for a Cure.

June 13, 2017

BY ELECTRONIC DELIVERY

The Honorable Thomas E. Price, MD
Secretary
Department of Health and Human Services
200 Independence Avenue SW
Washington, DC 20201

Seema Verma
Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Ave, SW
Washington, DC 20201

RE: CMS-1677-P -- 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 2018 Rates; Quality Reporting Requirements for Specific Providers; Medicare and Medicaid Electronic Health Record (EHR) Incentive Program Requirements for Eligible Hospitals, Critical Access Hospitals, and Eligible Professionals; Provider-Based Status of Indian Health Service and Tribal Facilities and Organizations; Costs Reporting and Provider Requirements; Agreement Termination Notices and Request for Information

Dear Secretary Price and Administrator Verma:

As you work to finalize the Centers for Medicare and Medicaid Services (CMS') Hospital Inpatient Prospective Payment System (IPPS) rule, the National MPS Society respectfully urges you to make access to appropriate treatments for rare diseases a priority.

The National MPS Society provides support to families and promotes research initiatives for Mucopolysaccharidoses (MPS) and related genetic lysosomal storage diseases (LSD) caused by the body's inability to produce specific enzymes. Normally, the body uses enzymes to break down and recycle materials in cells. In individuals with MPS and related diseases, the missing or insufficient enzyme prevents the proper recycling process, resulting in the storage of materials in virtually every cell of the body. As a result, cells do not perform properly and may cause progressive damage throughout the body, including the heart, bones, joints, respiratory system and central nervous system. While the disease may not be apparent at birth, signs and symptoms develop with age as more cells become damaged by the accumulation of cell materials.

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Many Medicare beneficiaries with rare diseases like LSDs face hurdles accessing lifesaving and life improving Food and Drug Administration ("FDA") approved therapies as a result of barriers inherent in the design of and process for updates to the MS-DRG system upon which payments are based. We applaud your leadership in requesting input from the stakeholder community on how the DRG system specifically, and the IPPS in general can be improved toward patient-centered care.

Through decades of advocating for new and improved technologies for our patients, we have seen a demonstrable and direct correlation between innovation and access. Scientific advances have spurred development of new therapies that not only satisfy unmet medical need, but also significantly improve the standard of care. The MS-DRG system, however, has not kept up with innovation in diagnosing and treating rare disorders. For individuals affected by debilitating and life threatening rare diseases, this disconnect can create a serious disparity in care.

The very nature of rare diseases – the small impacted population – makes placement of rare disease diagnoses into broad MS-DRGs with potentially divergent disorders the norm. As a result:

- Claims for treating patients with rare diseases that are both very infrequent and very high cost are likely "trimmed" as outliers in CMS' payment update calculations; and/or
- The increased cost associated with these inpatient stays triggers a very small payment increase across diagnoses within the DRG, but is of marginal impact in offsetting the costs of treating the rare disease.

Unless there is a mechanism for ensuring a rational relationship between the cost of treating patients, including the cost of orphan drugs, and the payment to hospitals for patient care, commercial realities will increasingly mitigate against developing new treatment options (and even using existing ones) to appropriately care for people with rare diseases.

We urge you to work toward aligning Medicare's approach to rare disease treatments with the public policy goals of the *Orphan Drug Act* and the *21st Century Cures Act*. Rare disease patients often spend years without a proper diagnosis and have few, if any, treatment options. It is absolutely critical to ensure that these patients have access to proven, valued therapeutic regimens.

We appreciate your consideration of our position on this important issue.

Sincerely,

Mark A. Dant
President and CEO
National MPS Society

cc: Tom Price, Secretary of Health and Human Services