BY ELECTRONIC DELIVERY

The Honorable Chairman Orrin Hatch (R-UT)
United States Senate
104 Hart Senate Office Building
Washingtin, DC 20510

The Honorable Ranking Member Ron Wyden (D-OR)
United States Senate
221 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Chairman Kevin Brady (R-TX)
United States House of Representatives
1011 Cannon House Office Building
Washington, DC 20515

The Honorable Ranking member Richard Neal (D-MA)
United States House of Representatives
341 Cannon House Office Building
Washington, DC 20515

RE: Hospital inpatient access to rare disease treatments

Dear Chairmen Hatch and Brady and Ranking Members Wyden and Neal,

We are writing to ask for your help in ensuring that the hope and promises of the Orphan Drug Act are fulfilled for patients with rare diseases treated in the inpatient setting -- either administratively, by asking that the Centers for Medicare & Medicaid Services (CMS) fix Diagnostic Related Group (DRG) and New Technology Add-On Payment (NTAP) insufficiencies involving existing and new orphan drugs, or through legislation.

As patient advocates and stakeholders representing the diverse community impacted by rare disorders, we are committed to advancing development of, and access to, treatments for these serious, and often life-threatening conditions. In the thirty-four years since Congress enacted the Orphan Drug Act, the lives of countless patients with rare disorders worldwide have been improved, or even saved, by new treatments. The breakthrough therapies now available to treat many rare disorders, particularly those impacting extremely small patient populations, likely would not exist today absent the innovation incentives for orphan drugs.

The physician office and hospital outpatient payment systems create a relatively level playing field and enable access to medically necessary treatment options for rare conditions. Unfortunately, the inpatient prospective "system-of-averages" framework is implemented to create, and perpetuate, payment deficiencies in the treatment of rare diseases. Both the magnitude and intractability of DRG payment inadequacies are often inversely proportional to the number of patients treated. In other words, the more rare the disease, the worse the problem

We understand the over-riding premise of the DRG system assumes that providers may face (and accept) below-cost reimbursement for some patients since deficiencies are offset by other patient stays requiring fewer resources. This system works as long as the MS-DRGs are comparing apples to apples. When a rare disease is assigned to a relatively broad or clinically diverse MS-DRG, a therapeutic breakthrough widens the chasm between that disease and others within the payment group. Providers have increasingly responded by shifting patients to extended outpatient stays, using a step-therapy approach that delays access to the orphan product indicated for the condition, or stabilizing and discharging the patient to seek care from another facility. Adoption of new technologies for hospital inpatients may be delayed in many

communities – new technology add-on payments are usually not implemented until well after the Medicare outpatient system grants pass-through status, if at all, and they are not designed to compensate the hospital inpatient provider appropriately. The criteria of newness is not being implemented correctly, as it should reflect those new technologies' costs are not accounted for in the DRG. For the few technologies able to garner an inpatient new technology add-on payment, the amount of the add-on payment is usually insufficient to support acquisition and administration of the orphan drug. Too often, patients are simply unable to get their only FDA-approved treatment when they need it.

The patient advocacy community and Congress have worked hard and accomplished much in removing barriers to treating rare diseases. Inpatient hospital impediments to orphan drug access are unfair to the patients for whom treatment options are available, but not accessible, today, as well as to patients with untreatable rare disorders counting on an incentive framework to advance tomorrow's breakthrough.

CMS' proposed inpatient rule for 2018 did not address payment deficiencies for rare diseases requiring orphan drugs, but the Agency did request suggestions on how the DRG system can be improved. We ask that you join us in supporting the needs of the rare disease community by writing CMS and the HHS Secretary to urge that they identify specific rare diseases that are currently grouped within MS-DRGs for which the payment is below the average cost for providing the standard of care, and make any adjustments necessary to ensure that the MS-DRG assignment for each rare disorder ICD-10 will result in adequate reimbursement for the cost of care, including orphan drugs. We also ask that you request that CMS' system moving forward be modified to accommodate each new orphan drug used in hospital inpatient facilities. CMS has authority to make these urgently-needed adjustments through its DRG updates or within the new technology add-on payment process.

We appreciate your consideration, and thank you in advance for joining us to ensure hospital inpatient payments adequately reimburse providers treating patients with rare diseases.

Sincerely,

<INSERT GROUP NAMES>