



December 6, 2018

Don Thompson
Director, Division of Acute Care
Hospital and Ambulatory Policy Group
Centers for Medicare and Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

Dear Mr. Thompson:

The undersigned advocacy organizations are committed to advancing treatment options and improving the quality of life for individuals with exceedingly rare, life-limiting conditions. We are, in essence, a small community of small communities, and are driven entirely by parents, patients, and caregivers working directly with researchers, providers, and other patients.

We write to:

- Open an ongoing dialogue with the Agency to ensure that the Administration's policy priorities within Medicare and Medicaid consider our needs, and any "solutions" ensure that we do not compromise our patients.
- An immediate example of the need for such an ongoing dialogue is the pending multi-year effort and current request that the Medicare inpatient system work properly for extremely rare disorders like porphyria so that patients have reliable access to the drug treatment that is the standard of care;

Extremely Rare Diseases in the Inpatient Hospital Setting

While we agree with CMS that modifications are needed to the inpatient system to account for the costs of treating extremely rare disorders, we need actual solutions. Everyone – patients, providers, and CMS – seems in complete agreement that the Medicare payment for a porphyria attack is way below what it would cost the hospital to provide the standard of care. Hospitals are not willing to absorb the financial loss, CMS is not willing to pay them sufficiently, and patients are the ones who absorb the life threatening consequences.

While most of us are working toward the day when a treatment option is available, porphyria patients are lucky enough to have an approved treatment. We hear their stories of awful attacks, debilitating pain, and hospitals unwilling to treat them appropriately because of financial disincentives in the inpatient system. We find it unacceptable that this could happen to any of our patients. Every Medicare beneficiary is entitled to receive medically necessary care -- low volume does not make solutions more difficult or patients less worthy.

Each of our organizations advocates for patients suffering from diseases that, like porphyria, are rare enough that treatment costs will not be reflected in the hospital reimbursement. When we read that CMS would not be able to help porphyria patients because the disease is too rare for the Agency to perform the administrative tasks needed to pay hospitals appropriately, there was a collective recognition that our "job" is far more than the discovery of a treatment. As you know, many diseases and conditions with existing and emerging treatment options are simply too rare to ever reach the thresholds CMS applies to make changes.

Putting the burden on very sick patients to enforce getting the standard of care is not a realistic solution. The immediacy, medical emergency moment when a patient is faced with a life threatening or debilitating situation and a hospital is denying an orphan drug that is standard of care cannot be sanctioned in CMS' payment structures. We know CMS does not want this either. So we ask that CMS develop a way to ensure hospitals can afford to treat our patients now or that it enforce, **in real-time**, the requirement that hospitals provide the treatment despite payment deficiencies.

Ongoing Engagement with the Extremely-Rare Disease Community

As patient advocates, we share your goal of ensuring that all patients can afford the treatments they need. We are well-aware of the financial hardship families absorb when a loved one is diagnosed with an extremely rare disease, and are committed to advocating for policies that make needed medical care more affordable. No family should have to choose between paying household bills and maintaining access to healthcare.

As the Administration and CMS move toward policies that reduce the costs of drugs, we believe that our perspective is essential. Through an ongoing dialogue, our communities can better inform CMS of the potential impact of its proposals on the health of patients with extremely rare conditions, and the real-world impact of newly-implemented policies.

We would like to organize a regular meeting with those at CMS that can affect real change in the inpatient and other settings of care in Medicare and Medicaid to ensure innovations toward treating and curing ultra-rare disorders reach the patients who need them.

If you have any questions or need additional information, please contact Saira Sultan at 202-360-9985.



