

BOARD OF TRUSTEES

Chairman
James V. Young
Chicago, Illinois

Executive Director
Desiree Lyon Howe
Houston, Texas

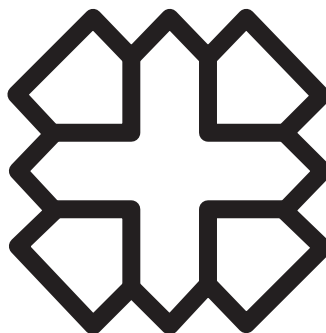
Secretary
William McCutchen, Ph.D.
Westport, Connecticut

SCIENTIFIC ADVISORY BOARD

Chairman
Karl E. Anderson, M.D.
University of Texas Medical Branch

D. Montgomery Bissell, M.D.
University of California

Joseph R. Bloomer, M.D.
University of Alabama



Herbert L. Bonkovsky, M.D.
Carolinas HealthCare System
Robert J. Desnick, M.D., Ph.D.
Mount Sinai School of Medicine

John H. Epstein, M.D.
University of California

Richard A. Galbraith, M.D., Ph.D.
University of Vermont

Micheline M. Mathews-Roth, M.D.
Harvard Medical School

John D. Phillips, Ph.D.
University of Utah School of Medicine

Neville Pimstone, M.D., Ph.D.
Los Angeles, California

Maureen B. Poh-Fitzpatrick, M.D.
Columbia University

Peter V. Tishler, M.D.
Harvard Medical School

AMERICAN PORPHYRIA FOUNDATION

BY ELECTRONIC DELIVERY

June 20, 2019

The Honorable 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: 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; Proposed Quality Reporting Requirements for Specific Providers; Medicare and Medicaid Promoting Interoperability Programs Proposed Requirements for Eligible Hospitals and Critical Access Hospitals

Dear Administrator Verma:

I am writing on behalf of the American Porphyria Foundation (APF) to once again draw your attention to the overwhelmingly pervasive difficulties patients experiencing acute porphyria attacks have in getting the prompt, adequate treatment they need. When Panhematin (hemin, for treating acute porphyria attacks) received the first-ever orphan drug approval in 1983, I could not have imagined that over 35 years later the APF would continue to hear countless stories from porphyria-diagnosed U.S. patients mirroring my pre-Panhematin battle with acute porphyria attacks progressing to life-threatening severity.

The APF has tried to navigate the MS-DRG reassignment process three times, and CMS has never disputed our assertion that growing numbers of acute porphyria patients are unable to get the Panhematin infusions they need in the inpatient hospital setting. The Agency's own data has consistently demonstrated the wide cost disparity between acute porphyria attacks and the remaining conditions within the payment grouping. Twice previously, CMS declined to make a change, but

promised to “monitor” the situation to assess the impact on patients. We have described the patient impact, worked with industry to present data to support that what we describe is, in fact, our reality, and enlisted the top experts treating porphyria patients to inform CMS on the care we should be receiving.

The nation’s foremost experts in disorders of inborn metabolism generally, and the porphyrias specifically, have generously taken time away from their research and clinical responsibilities to meet with or write to CMS. These experts have clearly conveyed to CMS that an inpatient stay related to an acute porphyria attack differs clinically, and in terms of resource use, from inpatient stays associated with the remaining diagnoses in MS-DRG 642. Despite the overwhelming weight of evidence suggesting that reimbursement for acute porphyria attacks has impeded, and will continue to impede access, CMS continues to maintain a status quo that perpetuates payment inadequacies.

- While CMS “monitored” access in response to our request for action, the gap between the standard of care and the treatment Medicare patients receive has widened;
- Each year, fewer and fewer hospitals offer the inpatient care that most of us need when experiencing an acute attack;
- CMS staff stated, both in our meeting and in last year’s final rule, that hospitals are not permitted to decline to administer Panhematin, and must absorb the financial loss for treating acute porphyria attacks according to the standard of care. Unfortunately, this requirement is useless unless providers have consequences and patients have a mechanism to resolve access hurdles in real-time;
- CMS acknowledged the access impediments and stated that it would examine potential mechanisms to address inpatient payment for rare diseases that, like the acute porphyrias, require orphan drugs. CMS has yet to take steps toward addressing this key shortcoming in the prospective payment system.

In an average week, the APF receives 4-5 calls (and often receives up to 10) from patients who know that they need to be admitted as inpatients and receive Panhematin infusions as soon as possible yet cannot find a hospital that will administer Panhematin in that setting. These patients are in excruciating pain and know that there is a treatment available that will stop their attack before it progresses further. These patients fear the humiliation they encounter when their description of pain triggers a lecture about hospital policies on drug-seeking behaviors. Acute porphyria patients do not go to the emergency room unless and until they absolutely must do so, and they are seeking treatment for their attack, not opioids. Opioid pain relief is necessary, but we know that it is not enough as it will not address the attack or halt its progression. Patients have described to me their encounters with hospitals, their frustrations with getting appropriate treatment, and being turned away from facilities offering a variety of excuses. The excerpts below are representative of the stories we have received from patients this year.

- “When I have an AIP attack, I experience debilitating pain that makes me unable to function. Yet, hospitals will not order the medication and treat my disease because they are not reimbursed. I am writing to request that you help change these so hospitals will be reimbursed by insurers, including Medicare/Medicaid for this treatment;” Female patient, California
- “If there were any delays [in receiving Panhematin] I experienced temporary paralysis which has left me with permanent Severe Peripheral Neuropathy. I have had to live on SSI and Medicaid

insurance was the only way I could be covered with insurance and necessary medical care.” Female patient, California

- “The chief medical officer talked to me and explained how much \$ they lose on panhematin, and they cannot give it to me anymore. If there’s anything you can do to help, I’d be very grateful.” Male patient, Arkansas
- “I was diagnosed 40 years ago and one of the first to receive hematin while it was being researched at the University of Minnesota from Dr. Klaus Pierach. . . . During an attack, I have severe nausea and vomiting, extreme pain, high blood pressure and very nasty migraine headaches. At this time, I can only get hematin at St. Francis Hospital in Tulsa. . . . Should St. Francis change their policy to not allow me inpatient access, as so many hospitals are, I am in trouble.” Female patient, Oklahoma
- “We currently have no Doctors that have shown enough concern to advocate for their patient and reasonable care and pain management until the medical issue resolved. I am devastated each day to know that by not taking my Panhematin, fluids and getting pain meds it spells an early death sentence to me. I am only 44. Please take some time to reinvest in those patients that truly need help in the form of proper care and pain management.” Female patient, Florida
- “When I have to go to the ER for help during an attack, I can guarantee waiting hours on end, sometimes into the next day to be seen while experiencing this excruciating attack that can lapse into seizures, comas, or even death if serious enough and not treated quickly and properly. Our hospitals are not educated in what Porphyria is or how to treat it and once we educate them on the only form of treatment that will help the attacks, Panhematin, they will not offer it to us.” Female patient, Kansas
- “When I have an AIP attack, I experience debilitating pain that makes me unable to function. Yet, hospitals will not order the medication and treat my disease because they are not reimbursed. I am writing to request that you help change these so hospitals will be reimbursed by insurers, including Medicare/Medicaid for this treatment.” Female patient, Tennessee
- “I was recently admitted to [redacted] in Chicago, IL for two months before receiving 5 doses of Panhematin. After being admitted a second time, I was not granted access to Panhematin. Medicaid refused to pay for it and the hospital refused to order it in fear that they would not be reimbursed for the treatment. I have now gone weeks without this life-saving therapy. Meanwhile, I am seriously ill and not well enough to get treatment on an outpatient basis.” Male patient, Illinois
- “After being admitted to [redacted], formerly known as [redacted], I was notified by my team of physicians that they would no longer be ordering Panhematin anymore. . . . The staff eventually discharged me without receiving any treatment.” Male patient, Tennessee
- “I experience another acute attack of the porphyria and upon admission I was told that I could not receive the Panhematin in the hospital because they said it was too expensive and not readily available . I cried because I didn’t know how I was going to get the treatment I needed. It took me a full year and multiple admissions to the hospital to find how to receive the Panhematin on outpatient basis.” Female patient,

- “Every minute counts, delay of treatment causes damaging components to the body’s organs and systems. Currently I have permanent damage to the intestinal, neuro, and starts of renal disease/failure. I need access to the only treatment for my condition to live a decent life and too be able to continue to help my parents out along with enjoying making memories with my children who I deeply love! I need your help along with the rest of the government to make this medication easily accessible. Female patient, Florida
- I was recently in the hospital with Acute-Intermittent-Porphyria. The pain was intense. I didn’t ask for pain meds but did ask for Panhematin. [redacted] in Decatur, TX refused to give it to me.” Female patient, Texas

When I relayed patient accounts of similar encounters to CMS during our discussion last year, I was encouraged by the clear outrage Mr. Don Thompson expressed. Mr. Thompson explained that these hospitals are acting in an unlawful manner and offered suggestions on how patients might help enforce Medicare requirements by reporting the violations through the appropriate channels. Anyone who has experienced an acute porphyria attack would doubt the ability of any patient to clearly, concisely, and effectively enforce Medicare requirements on their own behalf, particularly during an attack. Most of us just want to be able to receive appropriate treatment when we need it without having to threaten the providers we rely upon for care.

We hope that CMS, having been informed of the access difficulties patients experience, will act appropriately to ensure that those struggling with the excruciating pain of a porphyria attack can access real treatment in their communities rather than symptomatic treatment with opioids, fluids, and glucose. There is simply no reason for patients to suffer the pain and debilitating consequences of a prolonged porphyria attack when there is a clear standard of care.

The APF sincerely appreciates your attention. I hope that you will not hesitate to call should you need any additional information or have any questions.

Sincerely,



Desiree Lyon
Global Director

Kristen Wheeden
Executive Director