Responses to Questions for the Record

Subcommittee on Health
May 17, 2016 hearing
"The Obama Administration's Medicare Drug Experiment:
The Patient and Doctor Perspective"

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Questions Submitted by the Honorable Leonard Lance

As Chair of the Congressional Rare Disease Caucus, I am particularly concerned about those patients with rare diseases, a vulnerable population that already experience lengthy journeys to accurate diagnosis, only to be presented with limited therapeutic options – if any – for effective treatment.

These same at-risk patients have great difficulty locating providers who can appropriately treat their rare and complex disease. To equate their medical condition with one that has multiple therapies available puts their access to these providers at risk.

Further, as we look to encourage the adoption of personalized medicine, Congress has taken particular care to recognize that a "one-size-fits-all" approach does not respect the acute needs of rare disease patients and other unique populations.

1. **Ms. Boyle, can you elaborate on the struggles rare disease patients and their families must endure and the unique needs of this population that CMS must keep in mind?**

Representative Lance, the Immune Deficiency Foundation thanks you for your leadership as a champion of rare diseases issues. We greatly appreciate your recognition that a "one-size-fits-all" approach does not recognize the needs of individuals with rare diseases. It is imperative that the Centers for Medicare and Medicaid Services (CMS) keep in mind that any disruption or delay in access to treatment may cause serious health problems for patients.

Primary immunodeficiency, or PI, represents a group of more than 250 rare, chronic genetic diseases in which part of the body's immune system is missing or functions improperly, resulting in a decreased ability to fight off infection. Throughout their lives, people with PI are more susceptible to infections, endure frequent health problems, including a number of other comorbidities, and can develop serious and debilitating illnesses.

Approximately 250,000 people are diagnosed with PI in the U.S. Depending upon the type of PI, treatments can include prophylactic antibiotic therapy, bone marrow transplantation, enzyme replacement, interferon gamma and antifungals. Patients with PI who have a lack of and/or impaired antibody function require lifelong, lifesaving treatment with immunoglobulin replacement therapy (Ig therapy), partly replacing what the body should be making and protecting them from infection. Today, with early diagnosis and appropriate therapies, such as Ig, many patients diagnosed with PI can live healthy, productive lives.

Patients with rare diseases and their families not only face the challenges that come with a serious, chronic health condition, but also face high out-of-pocket costs, difficulty being correctly diagnosed, difficulty finding providers experienced to treat their particular condition, greater chance of infection and illness, challenges with coverage of their treatment, and missed work. Many times, these challenges are compounded for families because PI is a genetic disease and multiple family members may be affected.

There are several effective medical therapies available for patients with PI which optimize their health, improve their quality of life and allow them to be productive members of society. However, it is important to understand that the Ig that is given partly replaces what the body should be making, but it does not stimulate the patient's own immune system to make more Ig. Since Ig only replaces the missing end product, but does not correct the patient's defect in antibody production, Ig replacement is usually necessary at regular intervals for the patient's lifetime.

The IDF urges CMS to be aware that any disruption or delay in access to treatment may cause serious health consequences.

2. Have you heard of these concerns from the patients in your organization? Can you comment on the impact of any potential delay or interruption in treatment for these patients?

Yes, the Immune Deficiency Foundation (IDF) regularly hears from patients regarding their coverage problems or difficulty finding a provider for their regular infusions. In fact, our experience in this area was greatly intensified after the implementation of the Medicare Modernization Act of 2003 (MMA). At that time, our patient population experienced firsthand the impact of treatment delays and disruptions due to serious access problems.

Starting in 2005, patients with primary immunodeficiency (PI) saw significant reductions in reimbursement as a result of the MMA, which changed Part B drug reimbursement from the Average Wholesale Price (AWP) to Average Sales Price (ASP) plus 6%. Two studies by the Health and Human Services Office of Inspector General (OIG) (*http://oig.hhs.gov/oei/reports/oei-03-05-00404.pdf)* and the Assistant Secretary for Planning and Evaluation (ASPE) *(https://aspe.hhs.gov/sp/reports/2007/IGIV*) reported in 2007 the difficulties physicians and specialty pharmacies had obtaining immunoglobulin (Ig) products at the Medicare reimbursed price and the impact on patients' ability to obtain their infusions. The HHS OIG reported to Congress that, *"Sixty-one percent of responding physicians indicated that they had sent patients to hospitals for IVIG treatment because of their inability to acquire adequate amounts of IVIG or problems with Medicare payment. "*

Some of our patients had to go without treatment because they had difficulty identifying a provider who would continue to infuse them when they could no longer purchase IVIG at the Medicare reimbursed rate. The Office of the Inspector General's (OIG) April 2007 report, Intravenous Immune Globulin: Medicare Payment and Availability, found that that Medicare reimbursement for intravenous immunoglobulin (IVIG) was inadequate to cover the cost many providers must pay for the product. During the third quarter of 2006, 44% of IVIG sales to hospitals and 41% of sales to physicians by the three largest distributors occurred at prices above

Medicare payment amounts. Earlier in the year, after price increases by manufacturers at the beginning of the year, 77% of IVIG sales to hospitals and 96% of sales to physicians occurred at prices above Medicare payment amounts. The result was that our patients struggled to find providers or had to miss or delay treatments. Some had to travel great distances, including crossing state lines, to access care. This infusion is typically given over several hours, so when you combine the travel time each way, the infusion time, and the recovery time, it means a patient could regularly miss a couple days of work or other activity for a regular infusion and incur travel costs.

Following the reduction in Medicare reimbursement for IVIG in 2005, the IDF commissioned three national surveys to better quantify the effect on patient access to care for PI patients.

The IDF survey[i] found that substantial numbers of Medicare patients had their treatments postponed and/or reduced in frequency following the change to ASP+6%. More than 4 in 10 Medicare patients (41%) reported postponed treatments and one-quarter of all Medicare patients suffered multiple treatment postponements. Eighteen percent of Medicare patients indicated that the time interval between infusions had increased since the end of 2004/beginning of 2005.

In addition, those surveyed reported that as a result of postponed treatments and increases in intervals between treatments, 26% of PI patients on Medicare reported suffering negative health consequences, including more infections generally (21%), increased use of antibiotics (19%), bronchitis (14%), and pneumonia (7%).

The IDF urges CMS to ensure that any reimbursement changes do not result in delay or interruption in treatment.

The Agency fails to recognize the reality that, for some conditions, there is no appropriate alternative treatment other than an orphan drug. The Proposed Rule all but acknowledges its disproportionate impact on beneficiaries with rare disorders in its discussion of budget neutrality and acknowledgement that the Model would shift Part B drug payments from specialists (treating the majority of rare disorders) to primary care providers, without furthering CMS' stated goal of encouraging use of lower-cost treatment options.

3. Can you speak to the impact of having to switch physicians for the rare disease patients you represent?

Our patients' disorders are rare and complex, and there are a limited number of major medical centers and physician offices that know how to manage them. Patients with a serious, chronic condition develop a long-term relationship with their physician. It is very difficult for a patient to change physicians, especially when it is not their choice to do so.

The Immune Deficiency Foundation (IDF) estimates that the average length of time between onset of symptoms and diagnosis is between nine and 15 years. Once a patient has a correct diagnosis and physician to oversee their treatment, any disruptions in that continuity of care are difficult for the patient, but also can jeopardize his or her health.

As the IDF's patients have experienced, access is compromised when physicians cannot purchase drugs at the Medicare reimbursed rate. It's not about making money off the patient—it's about being able to sustain infusions for patients – especially those with chronic conditions that require treatment in regular intervals. For our patients, intravenous immunoglobulin (IVIG) is given every 3 to 4 weeks and more frequently for subcutaneous immunoglobulin (Ig). If providers can't break even, they don't provide the treatment to our patients.

In 2007, the HHS OIG reported that 61% of responding physicians indicated that they had sent patients to hospitals for IVIG treatment, largely because of their inability to purchase IVIG at prices below the Medicare payment amounts. In addition, OIG found that some physicians had stopped providing IVIG to Medicare beneficiaries altogether.

In 2007, IDF commissioned a national survey of immunologists, conducted with the American Academy of Asthma, Allergy and Immunology (AAAAI). Fifty-one percent (51%) of physicians reported having patients change their site of IVIG therapy because of reimbursement. Thirty-six percent (36%) of physicians treating PI patients with IVIG reported their IVIG-using patients have experienced additional or more severe health problems since the beginning of 2005 because of reductions in Medicare reimbursement. Nearly half of the doctors with IVIG-using PI patients believe current Medicare reimbursement rules for IVIG pose an extreme or serious risk to the health of their patients. Three-quarters of physicians were of the opinion that current reimbursement poses at least a moderate risk to the health of their PIDD patients.

Sending patients with compromised immune systems to the hospital for treatment is dangerous and actually more expensive for the Medicare program. From experience, the IDF knows that there are few hospitals in every state that have the capacity to treat and provide IVIG to patients with PI (Two examples: only 2 in Connecticut – Yale and Hartford; and only two in Maryland -- both in Baltimore) making it a geographical challenge for patients. In addition, not all sites of care are appropriate for all patients so access to the most appropriate site is crucial.

As a result of the Medicare Modernization Act of 2003 (MMA) cuts, IVIG in the physician's office was nearly eliminated because physicians could not afford to administer infusions. Even though Medicare covered home infusions, the reimbursement became so low that specialty pharmacies could not afford to provide the items and services necessary for IVIG in the home. Congress responded by passing the Medicare IVIG Access Act (P.L. 112-242) with overwhelming support (401-3 in the House; unanimously in the Senate). This demonstration is currently underway, and IDF anticipates it will lead to a permanent fix in the current Medicare home infusion benefit for IVIG. (See https://innovation.cms.gov/initiatives/IVIG/index.html.) Our fear is that the proposed Part B demonstration, which explicitly includes the current Medicare IVIG Access demonstration, will undercut the IVIG demo. Specialty pharmacies already complain that they are close to underwater now with ASP+6 and low payment for the items and services needed for infusions in the home.

It's important to ensure access to the most appropriate and medically indicated setting. Patients with rare diseases must not lose access to their physicians and sites of care.

[i] *Assessing the Impact of Changes in Reimbursement Regulations and Product Availability on Access to Intravenous Gammaglobulin Treatment Among Primary Immune Deficiency Patients, The Immune Deficiency Foundation, November 28, 2006*