Statement of Immune Deficiency Foundation

To

Department of Health and Human Services

October 20, 2011

Regarding Essential Health Benefits:

Protection of Patients with Rare and Chronic Diseases

The Immune Deficiency Foundation (IDF) is the national patient organization dedicated to improving the diagnosis, treatment and quality of life of persons with primary immunodeficiency diseases (PIDD) through advocacy, education and research.

Primary immunodeficiency diseases occur in persons born with an immune system that is either absent or hampered in its ability to function. While not contagious, these diseases are caused by hereditary or genetic defects and can affect anyone, regardless of age or sex. The World Health Organization recognizes more than 150 primary immunodeficiency diseases, which are a group of rare disorders. Some affect a single cell within the immune system; others may affect one or more components of the system. While some of the nearly 150 primary immunodeficiency diseases still resist effective treatment, biologic medications are extremely important long-term therapeutic agents for many of these life-threatening disorders, now and into the future.

Years ago, a diagnosis of PIDD meant extremely compromised lives, not just for the patients but for their families as well. Today, with early diagnosis and appropriate lifelong therapies, many patients with that same diagnosis can live healthy, productive lives. The majority of individuals diagnosed with a PIDD have antibody disorders, many of which require life-long treatment with immunoglobulin (IG) replacement therapy to replace missing or improperly functioning antibodies needed to fight infection. Without lifelong treatment with IG therapy, individuals with PIDD would be unable to fight off even minor infections, including the common cold.

IDF supports the recommendations of the National Health Council (NHC) regarding the Department of Health and Human Services’ (HHS’s) design of Essential Health Benefits (EHB) with several additional concepts that pertain to patients with rare and chronic diseases. In addition to this statement, IDF will also submit amendments to the regulatory language suggested by the NHC to HHS.

(1) In keeping with the title of the Institute of Medicine report “Essential Health Benefits—Balancing Coverage and Cost”, how can the Department best meet the dual goals of balancing the comprehensiveness of coverage included in essential health benefits and affordability?

The first objective of the essential health benefits (EHB) regulation should be to achieve a balance of cost and quality. And it should also protect those people who have rare and chronic diseases rather than
just those who need episodic health care. IDF strongly believes that while the Institute of Medicine report provided a starting framework in striking the balance between affordability and appropriate coverage, overall the report emphasized affordability too stringently. Because patients with rare and chronic diseases will be required to receive coverage or receive a penalty, the insurance coverage options must provide them with meaningful benefits, not just a cookie cutter solution inappropriate for a complex disease. This level of protection for patients involves the design of plans’ benefit structures to avoid discrimination, encourage equal access, and provide clear guidance for plan members. Three specific patient protections should be included in the EHB regulation to help achieve this goal.

Cost-Sharing Protections
First, although the ACA requires EHB to reflect an appropriate balance among the ten categories of health services, additional protections are necessary to accomplish broad cost-sharing protections. The Affordable Care Act (ACA) leaves much flexibility to states to ensure plan compliance. Therefore, HHS has an obligation to ensure that states are performing oversight activities as required. Further, patients need to understand potential costs prior to enrollment and renewal, including in-network and out-of-network cost differentials, co-pays and co-insurance.

As HHS reviews plan benefit designs and assesses access to care issues for enrollees, the agency may consider additional requirements on plans. For example, the Maryland Comprehensive Standard Health Benefit Plan specifies cost-sharing requirements for certain services and includes some service limits to offer an extra level of patient protection for enrollees in these plans. Specifically, the plan requires a $100 copayment for emergency room services; a $40 copayment for skilled nursing facility care; and service limits on outpatient rehabilitation services of 30 visits each for physical therapy, speech therapy, and occupational therapy.

Cost-sharing protections should include:
- Requirements for plans to disclose to all prospective and current members the deductible, co-payment and co-insurance amounts applicable to in-network and out-of-network covered services
- Oversight mechanisms to ensure that states are reviewing plan benefit design to determine that cost-sharing is neither unfair nor discriminatory

Continuity of Care Protections
Second, the EHB regulation should require plan cooperation and coordination when people switch enrollment between plans or between coverage (e.g., Medicaid and Exchange coverage). Patients, especially those with chronic conditions, may spend a considerable amount of time following plan rules working with their providers to tailor their treatment approach to best fit their needs and health status. Once people are stabilized on appropriate therapies and protocols, it is critical to minimize disruption to the often delicate balance of their treatment. For patients with PIDD, immunoglobulin replacement therapy is life-long and should not be interrupted unless medically justified. Although some plans will honor previous care pathways and protect the continuity of care for their new members, there are no requirements for them to do so. Additionally, plans will often require new members to re-establish the medical necessity of their current course of treatment in order to comply with the plan’s utilization management program and coverage policies. Such policies jeopardize the health and welfare of patients with rare and chronic diseases.

Continuity of care protections should include:
- Protections for patients switching enrollment between qualified health plans or to or from Medicaid, so patients do not have to re-establish the necessity of treatment protocols already in place.
- Navigator education programs must provide information about the potential implications of switching between plans
Non-Discriminatory Plan Design

Third, the EHB regulation should provide for oversight of plan benefit design to avoid discrimination caused by unfair utilization management (UM) techniques or other plan design elements. The ACA specifically permits health plans to employ commonly used UM techniques; but at the same time, it prohibits plans from designing benefits that are discriminatory. Health plans often require UM tools, including fail first, prior authorization, step therapy, and other approaches. Health plans sometimes require members to try less expensive services, therapies, or prescription drugs prior to receiving coverage for more expensive options. In many cases, people with chronic conditions use expensive therapies and are subject to these UM techniques. Currently, such tools are used to create obstacles and discourage treatment which literally can be dangerous and life-threatening to patients in the PIDD community as well as other rare diseases.

To ensure non-discriminatory utilization management, the regulation should include:

- Specific oversight mechanisms for states to use in reviewing plan UM policies to ensure practices are neither unfair nor discriminatory (especially to those with rare and chronic diseases);
- Requirements for plans to disclose to all prospective and current members all utilization management techniques as well as all limits on services;
- Federal monitoring programs to ensure appropriate checks are in place to guarantee that plans are meeting federal requirements; and
- Prohibitions against the use of UM techniques to block or delay care and treatment to the rare and chronic disease community.

Addressing these three priority areas (i.e., cost-sharing protections, continuity of care protections, and non-discrimination plan design) will help plans achieve balance between costs and quality for members to help them avoid issues accessing health care services from their plans.

**(2) How might the Department ensure that essential health benefits reflect an appropriate balance among the categories so that they are not unduly weighted toward any category?**

By providing all of the key benefit areas, the Secretary will have provided an “appropriate balance” among the ten categories. However, each insured individual will likely use those categories to best suit his or her needs. For individuals with rare and chronic diseases, it will be most important that those individuals have access to specialists and all prescription medications. For instance, patients with primary immunodeficiency diseases rely upon an early diagnosis (which usually requires prompt access to a specialist) and appropriate lifelong therapies (usually immunoglobulin to replace missing or improperly functioning antibodies needed to fight infection) to maintain healthy, productive lives.

**(3) What policy principles and criteria should be taken into account to prevent discrimination against individuals because of their age, disability status, or expected length of life as the Affordable Care Act requires?**

We call upon all policymakers engaged in the current health care reform process to ensure that the essential health benefits package fully addresses the needs of all Americans including the specialized needs of individuals with rare diseases. For individuals with these conditions, “one size does not fit all.”

**Access to Specialists**

Requirements for qualified health plans must ensure that minimum patient protections are met in every state’s Exchange plans. Individuals with rare diseases require services and care from members of the medical profession who have specialized knowledge of the diagnosis, treatment and management of their disorders. Qualified health plans utilizing a provider network must provide an adequate number of in-network providers in various specialties corresponding to the EHB categories of health services. Streamlined access to specialists is critical for people with rare diseases. Referral requirements or prior authorization polices should be waived to allow direct access to specialists for individuals with chronic
diseases requiring specialist care. Qualified health plans should also have a mechanism in place for individuals with extremely rare diseases to receive services out of network without incurring astronomical additional costs.

**Access to Therapies**

Individuals with rare diseases must have access to the full range of medically necessary treatments appropriate for their condition. Decisions regarding which treatments are most suitable must be reserved for the physician in consultation with the individual patient. Without appropriate treatment, individuals face detrimental health outcomes. Furthermore, payers risk facing unnecessary costs from potential complications that arise from any limitations placed on the full range of therapies.

**Access to all Sites of Care**

Patients with rare and chronic conditions must have access to the site of care that is determined by the patient and his/her physician. Because our patient populations have the need for life-long treatment and not episodic care, it is important to take into consideration the site of care that works best for the patient – whether that be in the hospital, hospital out-patient, a physician’s office or in the home. Restricting treatment sites is a barrier to access to care.

**Cost-Sharing**

It is vital for patients with rare diseases to choose a health insurance policy that will meet their unique needs. Therefore, plans must be required to disclose to all prospective and current members information about the deductible, co-payment and co-insurance amounts that are applicable to in-network and out-of-network covered services as well as any limitations on services. In addition, the Department should prohibit specialty tiers, and offer protections for high out-of-pocket costs on prescription drugs by providing tiering exceptions. States should be provided with oversight mechanisms allowing them to review plan benefit design ensuring that cost-sharing does not discriminate or unfairly target any patients or rare disease groups.

**Medical Necessity Determinations, Appeals, and Grievances Processes**

IDF strongly agrees with the following recommendations previously provided by the National Health Council (NHC):

- Requirements for plans to use medical necessity criteria should be objective, clinically valid, and compatible with generally accepted principles of care. Furthermore, plan denials, based on lack of medical necessity, should explain in clear language the criteria used to make the determination.
- Uniform exceptions and appeals processes and requirements for states to perform plan oversight must also be included in any regulation. Easy-to-access plan grievances processes and a system to track grievances and oversee plan responses to grievances filed.

**Utilization Management (UM) without Discrimination**

Health plans’ UM practices should not impose unfair nor discriminatory requirements for plans to disclose to all prospective and current members all utilization management techniques. States must be provided with specific oversight mechanisms allowing them to review plan UM policies and to guarantee that plans are meeting federal requirements.

**What models should HHS consider in developing essential health benefits?**

While the results of the Secretary of Labor’s survey of employer-sponsored health benefits have informed the development of the essential health benefits package, existing federal programs, such as the Federal Employee Health Benefits Plan (FEHBP) and Medicare, may be instructive in determining the potential approaches the Secretary may take to develop the benefits package as well as in identifying the items and services that could be included.
The FEHBP is the largest employer health insurance program in the United States, insuring eight million current and former federal employees and their families. FEHBP is a system of competing private health plans that is often viewed as a working model for providing decent, affordable coverage.

While there is no prescribed minimum benefit package for FEHBP plans, an often mentioned benchmark standard for adequate coverage is the Standard Option offered by Blue Cross Blue Shield under the FEHBP (BCBS-SO). The benefit package provided under BCBS-SO offers comprehensive coverage for hospital, medical, surgical, diagnostic, preventive, maternity, and emergency services; prescription drugs; mental health; treatment therapies; physical and rehabilitation therapy; and home health, skilled nursing, and hospice care.

A review of the services covered under the Medicare program shows that Medicare covers substantially the same services as BCBS-SO, barring age-specific services such as pediatric care. The categories described in ACA are insufficient in capturing the range of services that could be included in the essential health benefits package.

**We strongly disagree with the Institute of Medicine’s reliance upon small employer plans as the appropriate model for defining essential health benefits.** As the IOM acknowledged, the majority of Americans receive health insurance through large employers, not small employers. Given that the essential health benefits package is intended to provide a coverage baseline for all Americans, it is important that such a baseline is relative to what the majority of insured Americans already receive. The baseline should not become a “ceiling”.

The Department should adopt specific national uniform guidelines to provide greater clarity regarding the definition of essential health benefits. While insurer’s guidelines are quite specific, they are not uniform, sometimes do not cover necessary therapies (especially for patients with chronic conditions), can be changed without notice, and sometimes include specific provisions which hamper proper chronic disease management. For instance, several insurers’ guidelines do not cover all of the available immunoglobulin therapies in all settings for those with immunodeficiency diseases, forcing patients to switch immunoglobulin therapies and unnecessarily incur the likelihood of adverse events.

Based on unpublished data from the IDF 2008 survey, *Treatment Experiences and Preferences among Patients with Primary Immunodeficiency Diseases*, patients are at greater risk of adverse events when switched to a new product. Due to the seriousness of these reactions some patients have taken drastic steps to avoid having these problems, including 24% who refused a particular product and 15% who delayed their infusion due to concerns about product tolerability. In addition, some insurers require specific protocols which hamper proper chronic disease management, such as a hiatus from immunoglobulin therapy for individuals with primary immunodeficiency diseases to demonstrate the necessity of the treatment. During this hiatus, the patient is simply waiting for the immune system to fail to prove that the immunoglobulin therapy is indeed medically necessary.

Therefore, it would be most helpful if the Secretary adopted national guidelines that are developed by specialty medical organizations or others that deal most closely with patients with rare and chronic conditions and not rely upon those developed by insurers. By doing so, those guidelines would recognize actual rare, chronic conditions, not require periodic substantiation, be uniform, relatively stable, and not hamper proper chronic disease management. In addition, the guidelines should take into account the best specialty clinician to provide such care and not rely heavily on gatekeepers for those with complex chronic conditions.

Besides adopting national guidelines for care, the Secretary should examine multiple care measures, including those related to access (e.g., access to specialists, wait times for seeing various practitioners, ability to be seen as a new patient, etc.), quality of care (e.g., appropriate chronic disease management, various service sites for care), and patient-centered care (e.g., meeting the particular patient needs). In crafting such measures, rather than relying on the “typical” patient, HHS should ensure that they
examine specific subpopulations to ensure that there are no discriminatory practices regarding special populations, especially those who may have higher than average health care costs.

(5) What criteria should be used to update essential health benefits over time and what should the process be for their modification?

The Secretary is required to periodically review the benefits package and assess if enrollees are experiencing difficulty accessing services. Several states have adopted mandated benefit review laws, which may serve as models for the different approaches the Secretary can take. The primary review criteria used across states that have such a process include: cost impact (e.g., impact of the total cost of health care in the state and costs for stakeholders affected by the mandate); social impact (e.g., utilization, insurance coverage, demand, availability, and need); and medical efficacy (e.g., verification of the effectiveness of the service or treatment in the prevention or treatment of disease or disability). In addition, while we support the goal of having data-driven measures to drive the health care delivery process, we remain concerned about an over-reliance on such measures. For instance, an over-reliance on collecting and evaluating data on new interventions for purposes of updating the EHB on an annual basis could result in unintended consequences. It is conceivable that an insurer will refuse to cover a new treatment as not medically necessary under the EHB making it virtually impossible to generate the data necessary to demonstrate the value of the new intervention. Therefore, an open, transparent process will be critical to ensuring the patient perspective is incorporated in the development of measures used to evaluate the essential health benefits package.

IDF appreciates the opportunity to submit its suggestions. For patients with a primary immunodeficiency disease, the ability to obtain the treatment they need in the setting they need is paramount. With treatment, patients with PIDD can live normal, healthy and productive lives. Without treatment, these same patients look forward to constant infections, multiple trips to the ER, stays in the ICU and the development of severe disabilities over time. We believe that United States health policy should promote healthy and productive lives rather than ultimately disability and possibly premature death. Please take into consideration how patients with rare and chronic diseases will fare under the rules you will be developing.