August 9, 2011

The Honorable Patrick J. Toomey  
502 Hart Senate Office Building  
Washington, DC 20510-3806  

The Honorable Robert P. Casey, Jr.  
393 Russell Senate Office Building  
Washington, DC 20510-3805  

The Honorable Ron Wyden  
223 Dirksen Senate Office Building  
Washington, DC 20510-3703  

Dear Senators Toomey, Casey and Wyden,

The Immune Deficiency Foundation (IDF) expresses strong support for S. 1423 “Preserving Access to Orphan Drugs Act of 2011” which addresses the PPACA fee on orphan drugs. This bill will clarify that orphan drugs used solely for orphan diseases such as primary immunodeficiency diseases (PIDD) will be exempt from the PPACA fee. We believe the fee works against the statutory incentives to encourage research and development of new treatments for rare 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. There are more than 150 primary immunodeficiency diseases recognized by the National Institutes of Health and the World Health Organization. Patients with PIDD are part of the rare disease population that impacts the lives of 30 million men, women and children in the United States.

Over the years, the Federal government has gone to significant lengths to facilitate the availability of treatments for rare diseases or conditions. S.1423 will continue to ensure that key therapies are available for patients with rare diseases such as PIDD. It is important that Congress’ intent that no roadblocks are erected for needed research and development of pharmaceutical therapies for rare diseases is kept. Congressional support has been the mainstay of continued research for effective treatments of rare and chronic diseases. Without Congressional support orphan diseases would not be studied and effective treatments would not be developed. Individuals diagnosed with PIDD rely on orphan therapies such as the Immunoglobulin (Ig) replacement therapy.

Thank you for your leadership on this important issue.

Sincerely,

Lawrence A. LaMotte  
Director, Public Policy