

**TESTIMONY**  
**OF**  
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**IMMUNE DEFICIENCY FOUNDATION**

**BEFORE THE**  
**HOUSE APPROPRIATIONS SUBCOMMITTEE ON LABOR,**  
**HEALTH AND HUMAN SERVICES, EDUCATION AND**  
**RELATED AGENCIES**

**APRIL 14, 1999**

Congressman Porter and members of the subcommittee, thank you for the opportunity to testify today on behalf of the Immune Deficiency Foundation (IDF).

Primary immunodeficiencies are a group of genetic diseases that share in common an inability of the individual's immune system to combat infection. They affect an estimated 40,000 individuals, infants, children and adults and if not diagnosed early and treated properly lead to significant illness and early death.

The Immune Deficiency Foundation is the national organization dedicated to improving the lives of these individuals. I am here today to speak as a patient, but I am also a physician. My case is quite representative of a typical immune deficient patient. I was diagnosed with Common Variable Immunodeficiency 10 years ago, following years of repeated infections, which were unresponsive to antibiotics, and undiagnosed by numerous physicians who were colleagues of mine. This led to numerous unsuccessful surgeries resulting in permanent lung and sinus damage. Prior to my diagnosis, a day was considered successful if I had enough energy to get out of bed. Following appropriate diagnosis and treatment with intravenous immunoglobulin (IGIV) I have been able to return to my medical practice and have a new lease on life.

IDF works to improve the clinical awareness and treatment of immune deficiencies. Research has shown that early diagnosis and treatment leads to fewer long term health complications and a better quality of life. Since 1997, IDF has had a contract with the National Institutes of Allergy and Infectious Diseases (NIAID) to construct and maintain registries of 8 primary immunodeficiency diseases.

The goal of the registries is to assemble a comprehensive clinical picture of each disorder including estimates of the disease prevalence, clinical course and complications. This data will be an invaluable source for physicians doing basic research. As an example, the chronic granulomatous disease registry is being used by four different institutions to examine six different questions relating to various aspects of this disease. Further expansion of these registries is essential if we are to increase our understanding of primary immunodeficiency diseases and the immune system itself.

The majority of primary immune deficient patients receive IGIV antibody replacement therapy. IGIV is derived from pooled plasma and is administered intravenously for two to six hours for the life of the patient. I have been receiving this therapy since my diagnosis. In the past, when patients received this life saving treatment they were primarily concerned with the potential transmission of blood borne pathogens (i.e., HIV, HCV) and the considerable difficulties associated with

frequent infusions. However, since the fall of 1997 a new and greater concern arose, an inability to obtain IGIV.

Due to a confluence of circumstances, the U.S. marketplace has been unable to meet the needs of patients who are dependent on IGIV. Demand has simply exceeded supply. Moreover, despite a number of promises by both government and industry to increase production and expedite new product licensure, today we are in shorter supply than at any time in recent history. In 1998, the US shortfall was 5 million grams and industry currently estimates that only a nine day supply of IGIV is available. Imagine knowing that a life saving therapy exists for your child and living with the fear that it may not be available tomorrow.

The shortage has prompted several US manufacturers and numerous off-shore companies to attempt supply solutions. The US manufacturers want to introduce new products and technologies that will increase the yield of each donor pool, thereby increasing final product supply. Because the rest of the world is not experiencing a shortage, off-shore companies would like to import their IGIV products which have been in therapeutic use in their own countries for a number of years. Regulatory requirements dictate that these products be proven safe and effective in the US through clinical trials. Clinical efficacy in a study can take years to assess; time we don't have under the current health care crisis.

IDF has worked closely with FDA and industry to propose solutions, including a revised clinical trial protocol allowing for numerous trials to be conducted concurrently. In addition, NIAID has expressed interest in investigating the mechanisms of action in IGIV, correlating the antibodies present in the preparations to clinical disease outcomes. IDF's medical and scientific advisors endorse this research and encourage seizing the opportunity to answer these questions while patients are participating in new licensure studies and subject to numerous blood draws. We encourage the subcommittee to support this important research.

Finally, in spite of the IGIV shortage, IGIV usage continues to expand as long term treatment of other chronic disease states. The medical community is aware of a number of reported cases of unexplained central nervous system disorders experienced by immune deficient patients who receive ongoing IGIV therapy. Blood carries with it inherent risks which increase for life-long recipients. Although all of these products are virally inactivated, there are concerns about new and emerging pathogens and their ability to be transmitted through blood. Targeted surveillance of primary immunodeficient patients would allow for an early warning system should such a threat be present in the blood supply. Specifically, IDF would welcome a joint collaboration between the Centers for Disease Control and Prevention and the FDA

in conducting surveillance within the immune deficient patient population to evaluate the experience of the only long term and frequent recipients of IGIV. We would encourage the participation of NIAID in this important effort.

Mr. Chairman and members of the subcommittee, thank you again for the opportunity to testify today. In closing, I want to state IDF's strong support for a 15% increase in funding for NIAID in FY 2000. I would be happy to answer any questions that you may have.