

**LYNNE SZOTT  
IMMUNE DEFICIENCY FOUNDATION**

**TESTIMONY**

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

**MARCH 14, 2000**

Mr. Chairman thank you for the opportunity to testify today on behalf of the Immune Deficiency Foundation (IDF).

Primary immunodeficiencies are a group of genetic diseases marked by an inability of the immune system to combat infection. An estimated 40,000 individuals, infants, children and adults are affected with primary immunodeficiencies. And, without early diagnosis and treatment these diseases can lead to significant illness and premature death.

The Immune Deficiency Foundation is the national organization dedicated to improving the lives of these individuals. I am here today testifying because I am personally affected by primary immunodeficiencies. I am the mother of two immune deficient boys, and the President of the IDF Florida Chapter. I spend hours on the phone and in support group meetings talking to parents of newly diagnosed patients, who tell a story similar to mine. That story is that your infant or young child returns to the hospital with repeated, unexplained and often life threatening infections. In the case of my son Michael his small body was ravaged with a horrifying blood infection which spared no part of him and did not subside despite the prescribed treatment. Near death he was air lifted from our local hospital to a university teaching hospital 230 miles from our home. I remain grateful for the day a pediatric immunologist was called in to consult on his case and Michael was properly diagnosed with x-linked agammaglobulinemia, his brother Matthew, born two months later was also found to have x-linked agammaglobulinemia.

Terrified, my husband and I began educating ourselves about our son's disease. With two out of three of our small children requiring chronic care disease management, I became my sons health care advocate. Often I had to educate physicians about their condition. This experience encouraged me to return to school and earn a nursing degree.

As a parent ongoing research is a top priority, because for anyone stricken with a life threatening illness we hope that one day our children will be cured. 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. This is the type of research effort that allows us to further understand the comprehensive clinical course of these diseases, and collect a cohort of patients in the event new therapies are developed. Primary immunodeficiency research helps investigators unlock the mysteries of the normal immune system giving us insight into common diseases such as cancer. Because of this and other important work conducted by NIH through intra and extra mural grants, the IDF strongly supports a 15% increase in funding for NIH in FY 2001.

When my sons were diagnosed the doctor explained to me that the boys would be life long recipients of the blood product intravenous immune globulin (IGIV). The majority of immune deficient patients receive IGIV every three to four weeks for their lifetime. For my sons this therapy is the difference between life and death. Withholding IGIV from my children is equivalent to withholding insulin from a diabetic. However, there are concerns about the safety of the blood supply. At the time my sons became dependent on blood products, there was a barrage of media coverage regarding HIV/AIDS and blood borne transmissions. Although, IGIV has not been known to transmit HIV, there are known transmissions of Hepatitis C and several years of repeated recalls due to concerns about CJD. You can imagine as a parent that these are the concerns that rip you from sleep and make you pace the floor at night.

I continue to ask many questions regarding the long term health consequences of receiving a blood product whenever given the opportunity to discuss this with those considered experts in the field. The truth of the matter is that long term health status is not know because surveillance has yet to be conducted on this group of patients.

Mr. Chairman, IDF is grateful for your support of Committee recommendations in last year's appropriations bill encouraging CDC to initiate a national primary immune deficiency surveillance program. I am pleased to report that CDC, the Office of the Surgeon General and IDF's recently established National Health Surveillance Research Planning Committee are working together to establish a comprehensive surveillance program this year. We encourage the Subcommittee to continue to support this important initiative in FY2001.

Mr. Chairman, one final thought on surveillance, last year IDF successfully advocated for a change in the recommended childhood vaccine schedule, urging that the live polio vaccine no longer be administered to infants because it transmitted polio to immune deficient children. My eldest son received the live vaccine prior to his diagnosis, and although he did not develop polio, he has peripheral nerve damage and fine and gross motor difficulties that some physicians speculate relates to receiving the vaccine. A national health surveillance program in this patient population would answer many unknown questions and help to promote the safety of the blood supply by obtaining data on this sentinel group.

Mr. Chairman, thank you again for the opportunity to testify today and for your long-standing support of the primary immune deficiency community. IDF has enjoyed working with you and we wish you well in your retirement. I would be happy to answer any questions that you may have.

Lynne Szott serves as the President of the Florida Chapter of the Immune Deficiency Foundation (IDF). In addition, Mrs. Szott is a member of the Chapter Advisory Committee, the peer review committee of the national organization providing leadership and oversight of local IDF programs. She has been an active spokesperson for many years to benefit patients and families affected by primary immunodeficiencies. Mrs. Szott has two sons born with X-Linked Agammaglobulinemia. Their medical therapy includes bimonthly infusions of intravenous immunoglobulin replacement therapy (IGIV).

IGIV has been instrumental in helping the Szott family live a normal life, free from the fear of constant and recurring infections. Concerns over appropriate diagnosis and treatment and access to quality health care in an age of managed care plans as well as issues related to blood safety caused Mrs. Szott to become a proactive volunteer for the IDF. As a mother and nurse, Mrs. Szott has educated patients and health care providers regarding the diagnosis and treatment of primary immunodeficiencies.

Mrs. Szott has been outspoken on the issue of Inactivated Polio Vaccine (IPV) vs. The Oral (live) Polio Vaccine. The CDC's recommended change in the polio vaccine schedule was quickly incorporated into the professional education programs she directs through the Florida Chapter. The goal of the information provided is to assist local pediatricians in making early diagnosis of primary immunodeficiency diseases.

Mrs. Szott, her husband Rick and their three children reside in Port St. Lucie, Florida.

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**BY**

**SEVERE COMBINED IMMUNODEFICIENCY DISEASE  
COMMON VARIABLE IMMUNODEFICIENCY DISEASE**

**CHRONIC GRANULOMATOUS DISEASE**

**X-LINKED AGAMMAGLOBULINEMIA**

**WISKOTT-ALDRICH SYNDROME**

**LEUKOCYTE ADHESION DEFICIENCY**

**HYPER IGM SYNDROME**

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