



**IMMUNE DEFICIENCY FOUNDATION**

The National Organization Dedicated To Research And Education For The Primary Immune Deficiency Diseases

**IMMUNE DEFICIENCY FOUNDATION RELEASES NATIONAL  
STATISTICS ON THE TREATMENT OF PRIMARY IMMUNE DEFICIENCY DISEASES  
Results Show Average Time from Onset of Symptoms to Diagnosis a Staggering Nine Years**

**BALTIMORE** – Results from a nationwide benchmark report of patients treated for primary immune deficiency diseases (PIDD) released today at the Immune Deficiency Foundation (IDF) National Conference show that the genetic diseases, once thought to be a pediatric problem, now affect more adults than children, and that the time to diagnosis is still too long. The average time from the onset of symptoms in individuals to their diagnosis is nine years. In the interim, these patients may suffer repeated and serious infections and possibly irreversible damage to internal organs.

The Immune Deficiency Foundation (IDF), a non-profit organization serving primary immune deficient patients and their families, conducted an in-depth patient survey to provide a comprehensive portrait of this population with rare diseases. The report is the second national survey of individuals affected by PIDD and provides the best representation of the population currently available. The major health surveys conducted by the U.S. government – the National Health Interview Survey and the National Health and Nutrition Examination Survey – do not collect information on primary immune deficiency diseases. No comprehensive population survey has ever been undertaken by the federal government to estimate the prevalence or the population characteristics of PIDD in the U.S.

Primary immune deficiency diseases are disorders in which part of the body's immune system is missing or does not function properly. In contrast to secondary immunodeficiency diseases in which the immune system is compromised by factors outside the immune system, PIDD are caused by intrinsic or genetic defects in the immune system. In the U.S., as many as 50,000 people live with a clinically significant primary immune deficiency disease. The most widely used treatment method for these diseases is the periodic infusion of intravenous immunoglobulin (IGIV). Nearly 70 percent of primary immune deficient patients use IGIV to maintain their health.

“The widespread use of IGIV among patients diagnosed with primary immune deficiency has changed the way we think of the prognoses of these diseases by extending the lives of those affected,” said Dr. Jonathan Goldsmith, Vice President of Medical Affairs, of the Immune Deficiency Foundation. “The recurrent, persistent and unusual infections that once led to the death and disability of primary immune deficient patients are now effectively treated by this life-saving therapy.”

Changing Face of Patients Affected By Primary Immune Deficiency Diseases

Attributed in large part to widespread treatment, today's survey findings reveal a dramatically changing face of PIDD, highlighted by a marked shift from children to adults. Based on responses from 3,000 participants, the survey found that 77 percent of patients treated with IGIV are over the age of 18. In fact, fewer than a quarter of patients included in the survey are under the age of 18. This is in startling contrast to the diseases' progression 20 years ago when most patients, without access to life-saving therapies such as IGIV, did not usually survive into adulthood. Today, nearly one in ten patients is over the age of 65. Moreover, the delay in diagnosis indicates initial diagnoses are being made in many adolescent and young adults.

- more -

The report also shows that PIDD patients suffered serious impairment before treatment improved their health. “My son was diagnosed with a primary immune deficiency disease at a young age and has been able to live a normal life thanks to years of IGIV treatment,” said Kathy Crews, mother of an adolescent patient.

#### Ongoing Concerns over Safety

Despite progress, real concerns for this patient population remain. Many patients surveyed are concerned about the continued safety of IGIV therapy. Seventy five percent of respondents said safety was a real concern, including the theoretical risks associated with viral diseases known to be transmitted by blood such as hepatitis, HIV/AIDS, and West Nile Virus.

“People who suffer from primary immune deficiency diseases face multiple challenges in today’s public health climate,” said Dr. Goldsmith. “While many of us may feel a sort of detached fear of serious life-threatening viruses like SARS and the smallpox vaccination agent, these infectious agents are very real threats to primary immune deficiency disease patients.”

#### Treatment Access Concerns Remain

Because IGIV therapy has proven to be fundamental to the health of the PIDD community, continued availability of IGIV therapy causes concern. A majority of the users surveyed (54%) reported that they personally had difficulties getting regular IGIV infusions due to shortages. IGIV is usually considered a “maintenance” treatment, one that will be taken for life, putting added pressure on the continued need for adequate supplies. The health problems that resulted from IGIV shortages during the 1990s have not been forgotten by patients. While there has not been a shortage for several years because manufacturers have increased capacity and product yield, availability of supply continues to be top of mind with the patients surveyed.

In addition, because IGIV is considered a life-long treatment, it can be a considerable cost burden for patients and their families. The survey found that nearly 20% of patients who are no longer on therapy ended it because of cost concerns or insurance barriers, not for medically indicated reasons. Ten percent have had health insurance applications denied, and nine percent have had conditions excluded from their coverage. Moreover, after a number of years, the costs associated with ongoing treatment may cause patients with chronic illnesses to reach the individual financial caps enforced by most insurers and ultimately lose their benefits. The results can be economically devastating for the patients’ families and life threatening for the patient.

#### Summary

The findings from the survey provide useful information to the Immune Deficiency Foundation in determining patient concerns and needs. This information will help direct the IDF’s future programs including patient education, physician education, Capitol Hill advocacy efforts and continued diligence in addressing the safety and reliability of IGIV product.

“If the results of this important benchmark survey teach us anything, they underscore the fact that the diagnosis and treatment of primary immunodeficiency diseases has been greatly improved,” said Dr. Goldsmith. “And that we can look forward to even greater improvements in the next 20 years.”

This study was funded by an unrestricted educational grant from the Bayer Corporation. It was conducted by the national public opinion research firm of Schulman, Ronca & Bucuvalas, Inc.

- more -

(SRBI). The survey was authored by John Boyle, Ph.D., a partner at SRBI and a member of the Board of Trustees at the IDF. The full results of the survey presented at the IDF National Conference are available at [www.primaryimmune.org](http://www.primaryimmune.org) or by calling the IDF at (800) 296-4433.

###

CONTACT:

Pamela Mooring  
Manager, Marketing and Communications  
Immune Deficiency Foundation  
40 W. Chesapeake Avenue  
Towson, MD 21204  
410.321.6647 ext. 206  
410.321.9165 (f)