STATEMENT OF THE IMMUNE DEFICIENCY FOUNDATION

PRESENTED TO:

DEPARTMENT OF HEALTH AND HUMAN SERVICES

ADVISORY COMMITTEE ON BLOOD SAFETY AND AVAILABILITY

PRESENTED BY JERRY WINKELSTEIN, MD
CHAIRMAN, MEDICAL ADVISORY COMMITTEE

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Good afternoon - my name is Jerry Winkelstein. I am a clinical immunologist at the Johns Hopkins Hospital. I am also the volunteer Chairman of the Medical Advisory Committee of the Immune Deficiency Foundation.

The Primary Immunodeficiency Diseases are a group of 50 diseases characterized by functional defects that are intrinsic to the cells and tissues of the immune system. Most of the diseases - but not all - are genetically determined. They affect thousands of infants, children and adults. Patients with Primary Immunodeficiency Diseases usually present with recurrent and chronic infections - and many times with potentially fatal infections. Some of the more well know primary immunodeficiency diseases are X-linked agammaglobulinemia, common variable immunodeficiency (a disorder that usually affects adults), and severe combined immunodeficiency disease - a disorder popularized as the "boy in the bubble" disease.

Many of these disorders affect B-lymphocyte function and thereby affect those patients' abilities to produce immunoglobulins and functional antibodies. Intravenous gammaglobulin has become the mainstay of therapy for most of these patients. It is not an overstatement to state that gammaglobulin replacement therapy is critical to the health of these patients - it prevents the fatal disseminated bacterial and viral infections, such as sepsis and meningitis, to which these patients are susceptible - and also prevents the ongoing chronic lung disease which accumulates over the years from chronic bronchitis and recurrent pneumonias.

As you know, there has been a recent shortage of gammaglobulin which has directly impacted on the care of these patients. Many factors may have contributed to this shortage. On the supply side of the equation, product recalls, limited manufacturing facilities, and availability of plasma donors may have contributed. On the demand side of the equation, off label use, as well as a growing number of patients with primary immunodeficiency diseases may play a role.

For what ever reason or reasons, the consequences of this shortage are very significant to patients with primary immunodeficiency diseases. Lowering the dose - or stretching the dose out to an unacceptable interval - or skipping doses altogether - all put these patients at significant risk for life limiting or life threatening infections. I should say, that I personally have seen patients who - for one reason or another - have not received gammaglobulin, and then developed a serious infection and died.

Between December 15, 1997, and today - about six weeks elapsed time - the Immune Deficiency Foundation has fielded emergency requests from physicians, pharmacists and home care companies representing over 1,000 primary immune deficient patients who were unable to obtain IVIG. The IDF is working will all IVIG manufacturers on a daily basis to obtain product for our patients. Based on our assessment that this situation was imminent, the IDF on December 10, 1997 wrote the FDA supporting the recommendation of its TSE Advisory Committee recommending the release of IVIG lots in which albumin was used as an excipient in the manufacture of these lots and that the albumin was derived from lots in which a donor or donors were identified at higher risk for CJD. IDF further recommended that manufacturers immediately stop the practice of mixing albumin from other production lots in the manufacture of IVIG.

The IDF re-affirms this recommendation in light of the shortage confronting our patients and the medical consequences of no treatment, under-treatment, or delayed treatment in our patient population.

In addition, the Foundation, on behalf of its patients and the physicians who treat them, would like to make the following suggestions:

1. We would like the NIH to move as aggressively as possible to identify the agent that causes CJD. Until the agent is identified we will not have a clear path to follow.

2. We would like the FDA and industry to identify the precise causes of the IVIG shortage. FDA and industry must quantify the production and release of IVIG to the marketplace. Further, reliable data must be obtained on the current consumption of IVIG - who is using it for what purposes. Facts related to these issues bear on policy decisions regarding withdrawal, recall and quarantine.

As a physician treating primary immunodeficient patients of course I would like to know that the material I am prescribing meets the strictest safety standards possible. However, at the same time, I also need adequate supply of that material available for the treatment of my patients. The issue we are here to address today - and the issue impacting directly on the health and survival of my patients - is how to balance safety and supply. The National Institute of Health, the FDA, other oversight bodies such as this committee, and industry must insure that all scientific avenues are being aggressively pursued and that appropriate measures are being pursued to understand and relieve the apparent shortage of this critical therapy.