

**FOOD AND DRUG ADMINISTRATION
BLOOD PRODUCTS ADVISORY COMMITTEE MEETING**

**Statement of the Immune Deficiency Foundation
RE: IGIV Clinical Endpoints**

Miriam O'Day, Vice President

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The Immune Deficiency Foundation is a patient advocacy group dedicated to improving the lives of individuals affected with primary immunodeficiency disease. IDF has presented testimony and data documenting the depth of the IGIV shortage and its human consequences before BPAC on many occasions. In addressing the ongoing shortage, IDF has recommended various strategies, a number of which are aimed at rationing the available supply of IGIV based on medical necessity. In cooperation with FDA the agency has supported and endorsed prioritization protocols and emergency supply programs such as the IDF Safety Net Program.

Since the Fall of 1997 industry estimates have consistently projected that demand will continue to outstrip supply well into the foreseeable future. It is estimated that the current annual supply gap for IGIV is approximately five million grams. For this reason the Foundation has encouraged additional strategies such as expediting licensure of new IGIV products and processes to alleviate the shortage.

IDF and its medical advisors support the FDA's revised guidance on IGIV clinical trials. The immunology community and immune deficient patients believe that the recommended revisions for IGIV licensure are a significant step toward improving the supply of this life-saving therapeutic. The IDF commends the agency for adopting end-points which are measured using the standard of care in the practice of clinical immunology, therefore avoiding undue diagnostic burdens on patients participating in clinical trials. FDA policy revisions in IGIV licensure are an excellent representation of public and private collaboration, allowing physicians who treat immune deficient patients on a daily basis the opportunity to consult on the appropriate clinical trial design while ensuring that patient safety has not been compromised.

In cooperation with FDA, IDF is conducting a retrospective data collection project to determine the incidence of serious infection for patients with Common Variable Immunodeficiencies. The data obtained in this study in conjunction with the published literature will further assist in substantiating an historical control group of untreated patients.

Thank you for your ongoing efforts to resolve this crisis in health care for immune deficient patients.