

**DEPARTMENT OF HEALTH AND HUMAN SERVICES
ADVISORY COMMITTEE ON BLOOD SAFETY AND AVAILABILITY**

**TESTIMONY OF THE
IMMUNE DEFICIENCY FOUNDATION**

MONITORING THE SUPPLY OF PLASMA DERIVATIVES

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Good morning. My name is Jason Bablak and I am Vice President of the Immune Deficiency Foundation. IDF is the national organization dedicated to improving the lives of primary immune deficient patients through research and education. I would like to briefly address the issue of monitoring the supply and demand of plasma derivatives from the perspective of a group that has recently experienced a shortage of life-saving medicines.

The primary immune deficient community experienced a severe shortage of IVIG that began in December 1997 and although has somewhat lessened, continues today. In late 1997 and early 1998, our community was unprepared for this type of event, both in terms of its effect on individuals dependant on IVIG, and in ways the IDF could work to mitigate this effect. We had no forewarning of the oncoming shortage, no method for tracking IVIG supplies, and procedures in place to deal with supply emergencies. Since then, IDF, in cooperation with certain manufacturers, have identified several key actions that worked to reduce the negative impact of the shortage, and to better prepare us for future shortages. From these efforts, which I will briefly describe, we also identified several general themes for dealing with a shortage of life-saving therapies:

- 1) *Identify potential emergency sources of product for patients in need.*

When the IVIG shortage first surfaced, IDF received hundreds of calls each week from patients, physicians and pharmacists who could not find product. In order to serve our community better, we developed the Safety Net program, which provides an emergency supply of IVIG. Since January 1999, IDF has allocated a total of 422,350 grams of IVIG through Safety Net, which equates to approximately 14,000 patients doses.

- 2) *Encourage the development of prioritization protocols within the major medical centers and home healthcare companies so that during periods of severe shortage the limited supply is reserved for the most medically necessary uses.*
- 3) *Survey the population to gain an understanding of the severity of the shortage and its impact on patient health.*

IDF was able to provide detailed information on the extent of the shortage and its effect on our patient community through a series of patient and physician surveys undertaken during the height of the IVIG shortage. We learned that nearly half of the primary immune deficient patients were experiencing serious negative health effects. This data

allowed us to quantify the shortage, and also to document its negative impact on the public health. This brought the shortage to the attention of government officials at the national level and resulted in swifter actions to resolve the shortage.

4) *Monitor the supply*

The plasma fractionation industry, through its trade association, PPTA, developed a method to monitor the supply of plasma derivatives in the US. While not perfect, it at least provides a historical understanding of the volume of product available, and may also show trends in the supply line. IDF also developed an informal information network with distributors, group purchasing organizations and manufacturers to get “real time” product release data and projections for near term product releases.

5) *Identify additional sources of product*

IDF recognized that expanding the current manufacturing capacity for IVIG in the US was a long term goal, and that we needed additional supply from foreign manufactures and new generation IVIG products from currently licensed manufacturers offering higher yields in order to ease the supply issues in the near term. With this in mind, we began to seek out and develop relationships with interested companies in an effort to facilitate the testing and approval of new products in the US.

With this background in mind, I would like to take the next several minutes to answer the question posed by the Committee: How can shortages of plasma derivatives be identified and monitored? The IDF believes that an enhanced supply system integrated with a real-time demand model is necessary to improve our current understanding of the complex supply and demand of plasma derivatives such as IVIG. First, we need a more robust system of data collection that takes into account anticipated releases of product for each month in advance, as well as near-term projections looking out an additional two to three months. Secondly, we believe more communication regarding production and distribution issues from industry to the patient community is required. Finally, a system to monitor demand for IVIG must be developed to complement the supply data that is generated.

We applaud the efforts of industry and PPTA to make available its current distribution data set. We have been able to use this data to monitor the extent of a shortage, and it is also very useful

to gain a historical context of the volume of product in a given time period in relation to market situations during that same period. However, as can be seen in the recombinant factor VIII market, the current data is a trailing indicator only and is not a useful tool for predicting or even identifying a current product shortage. Our proposal is to enhance the current system in place with a new data point identifying the projected product releases for each month along with a longer-term projection of an additional month or two. While this data will not be as reliable as the historical data we currently have, it would be useful in making short term assumptions, especially when put in contest of current market and regulatory situations. It is clear that the manufacturers have this data individually and use it in their own calculations. In an industry that is constantly in short supply of its life-saving products, it seems only fair that we develop a method to communicate this information to those that depend on these products so that they may make adjustments when necessary.

The second tier of our proposal is that the manufacturers provide better communication of current operating conditions with the consumers. While we understand that each company desires to put its best face forward with its ultimate customer, at times it seems that we are the last to know of potential issues that may affect supply. We would encourage each company develop an early warning system, so that when potential issues do arise, a clear line of communication between the consumers and the company exists and can be used to share these developments. On the occasions when companies have come to us with product related issues, the IDF has been able to both provide

We understand that legal issues may exist that would prevent or discourage the type of predictive system identified in our proposal. In fact, it may require a third party outside of industry with a separate funding source, or another solution that is not apparent today. IDF stands ready to help in this process, and we offer our participation on a joint industry/consumer/government task force to identify potential solutions.

The final aspect of the plan is that IDF is defining a demand model for IVIG and is launching an effort to predict current and future demand for IVIG. Despite what we believe to be a general reduction in demand for IVIG during the past two years, the marketplace began to tighten again during the first quarter of 2001. Supporting this belief is our experience with the Safety Net program:

- Backorders representing approximately 80 patients
- Current inventory levels approximately 50% of normal
- New hospital customers, typically served through GPOs

In addition to this data, several manufacturers are having difficulty releasing product, and one brand had switched distribution channels and brand names resulting in significant market confusion. To put it metaphorically, we are skating on thin ice, and we do not yet know if the ice is getting thicker or beginning to break apart. Because of the sense of jeopardy that results from this lack of knowledge, the IDF is undertaking a study to monitor demand for IVIG across all indications. We believe that this data will fill the gap in the current understanding of the IVIG market, and will better able us to identify and monitor IVIG shortages.

Thank you for this opportunity to present our experience with severe product shortages and our recommendations on methods to provide a better system to monitor IVIG supply and demand. I would be happy to answer any questions the committee may have.