PPTA Statement on
PPTA's North America Data Program: A System to Monitor Availability of Life-Saving Plasma Protein Therapies

FDA Drug Shortage Workshop
September 26, 2011

Good morning. My name is Jan Bult. I am President & CEO of the Plasma Protein Therapeutics Association (PPTA). PPTA is the international trade association for the world's major producers of plasma-derived and recombinant analog therapies.

History of PPTA Data Program

PPTA's North American data program was developed in response to a shortage of intravenous immune globulin (IVIG) products in the late 1990s. For many users of these products, their health depends on regular IVIG infusions. They demanded information on the scope, likely duration, and causes of the shortage. The shortage was also the subject of Congressional hearings and substantial media attention.

To address this situation, as well as potential future shortages of plasma protein therapies, the Advisory Committee on Blood Safety and Availability (ACBSA) made a specific recommendation to FDA regarding an industry-wide supply data reporting program. The key elements were that: (1) the industry trade association – PPTA – should be directly involved in the program; (2) the program should involve collection *and dissemination* of standardized information; (3) the information reported should be quite detailed; and (4) the data should be reported monthly. By the Spring of 1998, FDA. PPTA, and manufacturers of the therapies – including both PPTA members and non-members – had all complied with ACBSA's recommendation.

Operational Aspects of the Program

PPTA's data program provides monthly, aggregate data on the supply of certain plasma protein therapies, including Ig, albumin, and hemophilia clotting factors. This information is reported directly to manufacturers that contribute data, FDA, and HHS. The information is also reported publicly, via PPTA's Web site, where it is available to patient advocacy groups, healthcare providers, and individual patients.

Antitrust compliance has been a priority since the program's inception, and it incorporates many competitive safeguards. These safeguards include the use of an independent, third party vendor to collect the individual company data. As a result of this firewall, individual company data is *not* made available to PPTA or to any manufacturer of plasma protein therapies. Both PPTA and manufacturer personnel receive aggregate, industry-wide data only.

Providing the data *only* to industry and regulators, however, is not enough. Patients also require access. In addition to the substantial benefit of providing peace of mind with respect to product supply, the PPTA data program empowers patients, and their physicians, to make better-informed decisions regarding treatment.

Benefits to Patients and Regulators

PPTA's data program has proven to be very helpful in times of real shortage. We experienced this with immune globulins in the late 1990s and with recombinant Factor VIII around 2000. For

example, the PPTA data helped many hemophilia patients make decisions regarding the timing of elective surgery.

The PPTA program is also very helpful when there are unsubstantiated rumors regarding shortages. In these situations, PPTA has been able to provide factual data to eliminate any potential concern. For example, in 2005-2006, the data was useful in distinguishing a mere supply disruption – resulting from changes in federal reimbursement policy that forced patient site-of-service changes and related IVIG access problems – from a true product shortage.

One thing we have learned is that, for a supply data reporting system to provide value to patients and regulators as part of a shortage-response framework, industry must make a sustained commitment. Implementing a reporting program only when there is a perceived shortage, and discontinuing it when it appears that supply is sufficient, will not work. In order to be there when needed, the system must be maintained continuously.

As a final note, collecting, aggregating, and reporting supply data takes time and expertise. In other words, it costs money. A data program must also be run in an antitrust compliant manner, which requires ongoing legal input and imposes another layer of expense. As we all know, FDA is already operating under substantial resource constraints and is not in a position to manage shortage-related early warning systems for the multitude of drug products it currently oversees on its own. The PPTA data program is an example of industry stepping up to lighten this burden.

Conclusion

PPTA member companies are committed to providing safe and efficacious plasma protein therapies to patients who need them. Part of that commitment is ensuring that there is an adequate supply of therapies to meet patients' needs. PPTA's North American data program has been a key component of this effort for over a decade. The program is an example of highly successful FDA-industry collaboration, and we hope that it can serve as a model to others