Plasma Protein Therapeutics Association

VIA HAND-DELIVERY

April 4, 2011

The Honorable Assemblyman William W. Monning
Chair, Assembly Committee on Health
State Capitol
Room 6005
Sacramento, CA 95814

RE: Letter of Support for AB 389/Mitchell (Pavley)
Standards of Service for Clotting Factor for Home Use

Dear Chairman Monning:

The Plasma Protein Therapeutics Association (PPTA) extends its strong support for AB 389, Standards of Service for Clotting Factor for Home Use, which will be heard in your committee on April 5, 2011. AB 389 would set treatment standards for persons with hemophilia and other bleeding disorders. People with bleeding disorders require access to high quality care in order to live long, productive lives. This bill would ensure that residents of California affected by bleeding disorders have access to their medically appropriate care.

PPTA represents the world's leading manufacturers of plasma-derived and recombinant biological therapies, collectively known as plasma protein therapies, and the collectors of source plasma. These critical therapies are infused or injected by more than 1 million people worldwide to treat a variety of rare, life-threatening diseases and serious medical disorders. PPTA members produce in excess of 80 percent of the plasma protein therapies used in the United States today and more than 60 percent worldwide.

Clotting factor, a lifesaving plasma protein therapy, is used every day to treat people with hemophilia, a blood clotting disorder that causes painful internal bleeding and debilitating joint damage. Delayed access to clotting factor can cause painful and crippling injury to the joints and organs of someone living with hemophilia. Such complications often lead to increased costs for hospital, skilled nursing and other specialty services. This bill would ensure that Californians will have timely access to their medically appropriate therapy.

An individual with hemophilia should have access to the full range of FDA-licensed clotting factor concentrates from the most medically appropriate provider, who is specialized in the treatment of their specific bleeding disorder. In fact, the U.S. Food and Drug Administration (FDA) has approved the various clotting factor therapies [Factors VII, VIII, IX and X and von Willebrand Disease] for distinct clinical indications. The therapies are neither clinically nor therapeutically interchangeable. In addition, some therapies are derived from human plasma, while others are made utilizing recombinant DNA technology, created from genetically modified cell lines.

Moreover, the Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation (NHF)—a leading patient organization for persons with bleeding disorders in the

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United States--has stated in its Guideline #159:

> *"Clotting factor therapies are neither pharmacologically nor therapeutically equivalent and vary based upon purity, half-life, recovery, method of manufacture, viral removal and inactivation processes, potential immunogenicity, and other attributes. The characteristics of each product and the resultant product choice for an individual patient require a complex decision-making process with the ultimate product being agreed upon by the patient and their respective healthcare provider. It is critical that the bleeding disorders community has access to a diverse range of therapies, and that prescriptions for specific clotting factor concentrates are respected and reimbursed."*[1]

AB 389 would ensure that individuals with bleeding disorders have unrestricted access to the full range of clotting factor therapies and ancillary infusion equipment and supplies. This bill would also make certain that all FDA-approved clotting factors are included in an insurer's formulary. These standards are essential for optimal treatment, because plasma protein therapies are distinct sole source products that have no generic biological equivalents and are not interchangeable. Furthermore, individual patients will react differently to therapies depending upon their unique health care needs.

AB 389 would also allow individuals to select providers that are familiar with the treatment of bleeding disorders. For example, many patients self-infuse clotting factor in their homes. The type(s) of home supportive services that are required is a decision best made by the patient in consultation with his or her physician. Patients need options when selecting such services to ensure that they will receive the highest possible level of service and care.

We thank you for your leadership in hearing this bill. If you should have any questions, comments, or concerns, please let me know.

Best Regards,

Bill Speir
Director, State Affairs

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[1] MASASC Recommendation #159 (last visited August 14, 2008), available at http://www.hemophilia.org/NHFWeb/MainPgs/MainNHF.aspx?menuid=57&contentid=179

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