



June 15, 2010

VIA E-MAIL

Reference No.: SASC10046

The Honorable Representative Beverly Earle  
NC House of Representatives  
300 N. Salisbury Street, Room 634  
Raleigh, NC 27603-5925

Dear Chairman Earle:

We are writing to you with our concerns that Section 10.23 of the Appropriations Act (SB 897) will negatively impact individuals with hemophilia and primary immune deficiency. These are rare, chronic conditions that require patients to have access to their medically appropriate therapy in order to live normal, healthy lives. Section 10.23 directs the Department of Health and Human Services (DHHS) to implement controls on specialty drug providers that distribute therapies to Medicaid recipients with hemophilia and primary immune deficiency. While we agree that controls are a necessary component of Medicaid administration, we become concerned when those controls negatively impact patient care and service.

The Plasma Protein Therapeutics Association (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 conditions including hemophilia and primary immune deficiency. 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.

Plasma protein therapies are not interchangeable. Individuals who rely on plasma protein therapies should have access to the full range of FDA licensed therapies from the most medically appropriate provider. Every product is unique and, because all products are all biologics, each therapy reacts differently for each patient. It is essential that the selection of product be made collaboratively with the patient and healthcare provider in order to achieve the best possible health outcome for the patient. Therefore, it is critical that individuals have access to a diverse range of therapies.

This is a position supported by the Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation, a leading patient organization for persons with bleeding disorders in the United States. MASAC has stated that, "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.”<sup>1</sup>

The language passed by the Senate states:

**SECTION 10.23.** The Department of Health and Human Services shall create a specialty drug provider network that requires best practices, prevents overutilization, and allows for drug reimbursement rate negotiations for hemophilia, hepatitis C, and intravenous immunoglobulin (IVIG) drugs.

The language passed by the House states:

**SECTION 10.23.** The Department of Health and Human Services shall work with providers to develop ways to reduce expenditures for specialty drugs, maintain best practices, prevent overutilization, and allow for drug reimbursement rate negotiations for hemophilia, hepatitis C, and intravenous immunoglobulin (IVIG) drugs.

Both of these provisions target individuals with hemophilia and primary immune deficiency. There are very few individuals in North Carolina that receive clotting factor through your state’s Medicaid agency. There even fewer individuals with primary immune deficiency who rely on IVIG provided by North Carolina Medicaid.

We wonder why the North Carolina General Assembly is targeting therapies that treat individuals with rare, chronic conditions that make up such a small percentage of your Medicaid population when reviewing your specialty drug providers? Like all components of the Medicaid budget, specialty pharmacy should be reviewed to ensure the North Carolina Medicaid program is providing recipients with quality care and service. Therefore, we would like to recommend the following language:

**Section 10.23.** *The Department of Health and Human Services shall work with specialty drug providers, manufacturers of specialty drugs, Medicaid recipients that are prescribed specialty drugs, and the medical professionals that treat them to develop ways to ensure that best practices and the prevention of overutilization are maintained in the delivery and utilization of specialty drugs.*

This would make clear to DHHS that they are to ensure best practices and limit overutilization in the distribution of all specialty drugs and rely on those steps alone to generate savings in the Medicaid program. It would also confirm for North Carolina residents that the General Assembly does not want DHHS to balance their Medicaid budget by limiting patient access to medically appropriate therapies, nor are they targeting care provided to a small population of individuals who live each day with rare, chronic conditions.

---

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

I appreciate your consideration of this matter, and would welcome any questions or comments you may have. I may be reached at [bspeir@pptaglobal.org](mailto:bspeir@pptaglobal.org) or 443-994-0900.

Best Regards,



Bill Speir  
Assistant Director, State Affairs

**cc:**

Rep. Bob England  
Rep. Verla Insko  
Rep. Jean Farmer-Butterfield  
Rep. William Brisson  
Rep. Randy Stewart  
Rep. Jennifer Weiss  
Rep. Jeff Barnhart

Ms. Susan Cowell  
North Carolina Hemophilia Foundation

Mr. Larry LaMotte  
Immune Deficiency Foundation

Ms. Ruthlyn Noel  
National Hemophila Foundation

Ms. Kisa Carter  
Hemophilia Federation of America

Mr. David Cavanaugh, Committee of Ten Thousand