

## **Implementation of a Step Therapy Protocol for Anti-hemophilic Medications Could be Life Threatening and Will Result in Higher Treatment Costs**

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### **Summary**

The member companies of the Plasma Protein Therapeutics Association (PPTA) recognize the need to control escalating prescription drug costs in State Medicaid programs. However, subjecting individuals with hemophilia to a step therapy protocol will result in less than optimal treatment outcomes, and could result in death. In addition, implementation of a “fail-first” step therapy protocol for life-saving blood clotting factor therapies will result in significantly increased expenditures within State Medicaid programs. **The member companies of PPTA urge State Drug Utilization Review Boards not to consider and not to recommend implementation of a step therapy protocol for life-saving anti-hemophilic medications.**

### **Background**

In 2003, the plasma protein therapeutics industry and the Nevada hemophilia community worked successfully for the inclusion of an exemption from the Medicaid preferred drug list (PDL) and prior authorization (PA) procedures for life-saving anti-hemophilic medications in Assembly Bill 384. It was the understanding of the industry and the hemophilia community that the PDL/PA exemption contained in AB 384 (and codified at NRS 422.4025) would be applicable to any utilization control, such as step therapy, that would employ a prior authorization process.

However, in August 2004, the Nevada Attorney General issued an opinion that stated that the Nevada Drug Utilization Review (DUR) Board could implement the step therapy requirements contained in AB 384 for the classes of drugs that are excluded from the PDL/PA. The Nevada DUR Board is now in the process of implementing the step therapy protocols of AB 384. However, the DUR Board retains the authority to determine the classes of therapies for which it will recommend that the Department of Human Resources implement step therapy.

### **About PPTA**

PPTA is the trade association and standards setting organization for the world’s leading producers of plasma-derived and recombinant analog therapies, including blood clotting factor therapies. These therapies are used by millions of people worldwide to treat a variety of diseases and serious medical conditions, including hemophilia and other life-threatening bleeding disorders.

## **Key Points**

PPTA opposes the implementation of a step therapy protocol for life-saving blood clotting factor therapies for individuals with hemophilia in State Medicaid programs for the following reasons:

- 1) **Physicians and their patients should decide the most appropriate initial therapy** - The sanctity of the physician/patient relationship should be maintained. Physicians with training in the treatment of hemophilia are uniquely qualified to determine the most appropriate product for each individual with hemophilia. Patients should not be required to “fail-first” on a specific product, or series of products, before having access to the blood clotting factor product that they, and their physician, have determined is most appropriate for their condition.
- 2) **“Step Therapy” is a medical term, not a payment term** – Step therapy is a term used in medicine to explain a protocol of using drugs of increasing strength. Such a protocol may be appropriate when drugs are recognized as therapeutically equivalent. However, since blood clotting factor drugs are not therapeutically equivalent or interchangeable, “step therapy” is not appropriate for this class of drugs. In addition, physician’s and their patients, not payers and their pharmacy benefit managers (PBMs) should be making medical decisions about the most appropriate therapy for a particular patient.
- 3) **Blood clotting factor therapies are neither pharmaceutically, nor therapeutically, interchangeable-** Each of the plasma derived and recombinant blood clotting factor therapies currently available to individuals with hemophilia is a unique, sole source product. The federal Food and Drug Administration (FDA) has approved each therapy for distinct clinical indications. Some of the blood clotting factor products currently available are stabilized with ingredients or use diluents that may not be appropriate for certain individuals. Patients should not be required to “fail-first” on a product to which they may have sensitivity prior to having access to the therapy of their and their physician’s choice. There are potentially dangerous health consequences from assuming that blood clotting factor therapies may be substituted at will.
- 4) **Allowing immediate access to most appropriate blood clotting factor product will result in better treatment outcomes and lower treatment costs–** If an individual with hemophilia is able to immediately access the blood clotting factor product of their physician’s choice, they will be able to achieve the most optimal treatment outcome as soon as possible. Increased hospitalizations will occur as factor replacement falls below recommended levels when individuals are forced to use a factor product that does not provide them with the best factor recovery levels. A step therapy protocol that requires that an individual with hemophilia “fail-first” on a specific therapy, or series of therapies, will result in

wasted Medicaid expenditures on therapies that do not achieve the most optimal treatment outcome for the patient.

- 5) **Different therapies may require different dosages to obtain the same therapeutic effect and may require verification via coagulation studies** – An individual with hemophilia may require significantly different dosages of different therapies to obtain the same therapeutic effect. Because of this, each time an individual with hemophilia changes therapies at the direction of a payer or pharmacy benefit manager, they must visit the physician in order to determine the proper dosage, administration schedule and other medical considerations. In addition, additional coagulation tests may be needed in order to verify that the therapeutic effect is being obtained. These coagulation studies must be performed in highly specialized coagulation laboratories.
- 6) **Possibility of inhibitor formation may result in higher Medicaid costs** – When an individual with hemophilia is forced to switch blood clotting factor therapies, there may be a higher level of inhibitor formation. Once an inhibitor has been formed, the inhibitor must be overcome with increasing levels of blood clotting factor or treated with immune tolerance. Both of these treatment options to overcome an inhibitor increase treatment costs exponentially.

### **Conclusion**

Blood clotting factor therapies constitute less than 1 percent of total Medicaid spending nationwide. While PPTA understands the need to control escalating prescription drug costs in state Medicaid programs, anti-hemophilic medications do not drive those costs. Life-saving blood clotting factor therapies treat a unique, life-threatening genetic condition. Blood clotting factor therapies do not have generic equivalents that can be substituted under a step therapy system. Lack of timely access to the most appropriate blood clotting factor therapy can lead to less optimal treatment outcomes, painful or debilitating injury, or even death.

**The member companies of PPTA urge State Drug Utilization Review Boards not to implement a step therapy protocol for life-saving anti-hemophilic medications.**