FDA Approves New Use Of Drug To Help Thousands Of Children

(NAPSA)—A recently approved use of factor for hemophilia A treatment may help many of the thousands of children living with this rare but serious disease.

Hemophilia A, also known as factor VIII deficiency or classic hemophilia, is largely an inherited bleeding disorder in which one of the proteins needed to form blood clots in the body is missing or reduced. The condition is characterized by prolonged or spontaneous bleeding, especially into the muscles, joints or internal organs.

Hemophilia A is the most common type of hemophilia and affects people of all races and backgrounds; it almost always affects boys, while girls carry the gene. Approximately one in 5,000 males born in the United States has hemophilia A.

Until now, treatments for hemophilia A have been approved for use episodically, prior to surgery and for short-term prevention of bleeding. But, based on the findings of a recent study, the FDA has now also approved Kogenate® FS, antihemophilic factor (recombinant), for use in a prophylactic way in children with hemophilia A and no pre-existing joint damage to reduce the frequency of bleeding episodes and risk of joint damage.

With this FDA approval, Kogenate FS, manufactured by Bayer HealthCare LLC, is the first and only factor VIII treatment determined to be safe and effective for routine prophylaxis, a treatment regimen recommended by the National Hemophilia Foundation's Medical and Scientific Advisory Council (MASAC).

A newly approved approach to treatment can help children with a serious disorder.

Prasad Mathew, M.D., director of the Hemophilia Program at the University of New Mexico and co-author of the Joint Outcomes Study, said, "The approval of this use of Kogenate FS is an important development in the care of patients with hemophilia A, especially young children. This newly approved use can help reduce the frequency of bleeding episodes both overall and into joints, and thus reduce the risk of joint damage in patients from infancy to age 16 who have hemophilia A and no pre-existing joint damage."

The clinical study on which the approval was based was published in The New England Journal of Medicine[1] and found:

- Ninety-three percent of participants in the routine prophylaxis group had no joint damage as measured by magnetic resonance imaging (MRI) compared to 55 percent in the episodic group. The difference between the two groups was significant when using MRI but not when using x-ray alone.
- The prophylaxis group had an 81.5 percent reduction in annual bleeding frequency compared to the episodic group.

Overall, eight of 64 patients developed any level of inhibitors over the 5½-year study. Only two patients developed high-titer inhibitors and were withdrawn from the study. The most common adverse events were related to central venous access, such as catheterization and catheter removal, central line infection and pyrexia.

"The clinical study results show that administering the drug as indicated in the study to reduce the frequency of bleeding into the joints was more beneficial in reducing joint damage," explained Dr. Mathew.

For more information, visit www.kogenatefs.com/info.

About Kogenate® FS

INDICATIONS

Kogenate® FS, antihemophilic factor (recombinant), *is a recombinant factor VIII treatment indicated for the control and prevention of bleeding episodes and perioperative management in adults and children (0-16 years) with hemophilia A. Kogenate® FS is also indicated for routine prophylaxis to reduce the frequency of bleeding episodes and the risk of joint damage in children with hemophilia A with no pre-existing joint damage.*

IMPORTANT SAFETY INFORMATION

The most serious adverse reactions are systemic hypersensitivity reactions and the development of high-titer inhibitors necessitating alternative treatments to AHF. The most common adverse reactions observed in clinical trials were inhibitor formation in previously untreated or minimally treated patients, skin-associated hypersensitivity reactions, infusion site reactions, and central venous access device (CVAD) line-associated infections.

Kogenate® FS *is contraindicated in patients who have manifested life-threatening immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including mouse or hamster proteins.*

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

Please see the full prescribing information for important risk and use information at www.kogenatefs.com.

[1]Manco-Johnson MJ, Abshire TC, Shapiro AD, et al. Prophylaxis versus episodic treatment to prevent joint disease in boys with severe hemophilia. N Engl J Med. 2007;357:535-44.