



December 1, 2014

## **Baxter Submits Application For US FDA Approval Of BAX 855, Extended Half-Life Recombinant FVIII Based On ADVATE For Hemophilia A**

### **Twice-Weekly Prophylactic Regimen Resulted in 95% Reduction in Median Annualized Bleed Rate Compared to On-Demand Treatment**

SAN FRANCISCO, Dec. 1, 2014 /PRNewswire/ -- Nektar Therapeutics (NASDAQ:NKTR) reported that partner Baxter International Inc. today announced that the company has submitted a biologics license application (BLA) to the United States Food and Drug Administration (FDA) for the approval of BAX 855, an investigational, extended half-life recombinant factor VIII (rFVIII) treatment for hemophilia A based on ADVATE [Antihemophilic Factor (Recombinant)].

"The evidence from our late-stage controlled trial showcases that BAX 855 could become an important new treatment option for people with hemophilia A, expanding on more than a decade of experience with ADVATE," said John Orloff, M.D., vice president and global head of research and development for Baxter BioScience. "BAX 855 represents our continued commitment to developing valuable new options for the hemophilia community that address the needs of patients around the world."

The submission is based on positive results from a prospective, global, multi-center, open-label, two-arm Phase 3 study of 137 previously treated patients (PTP). The results demonstrated that BAX 855 met its primary endpoint in the control and prevention of bleeding episodes and routine prophylaxis for patients who were 12 years or older. Patients in a twice-weekly prophylaxis arm experienced a 95 percent reduction in median ABR as compared to those in the on-demand arm (1.9 vs. 41.5, respectively). BAX 855 was also effective in treating bleeding episodes, 96 percent of which were controlled with one or two infusions. No patients developed inhibitors to BAX 855 and no treatment-related serious adverse events, including hypersensitivity, were reported. The most common (three patients) product-related adverse reaction was headache.

Baxter expects to present additional data from the BAX 855 pivotal trial in the coming months. The continuation study remains ongoing for patients who completed the pivotal trial and the Phase 3 study among previously treated patients under the age of 12 with severe hemophilia A is progressing. Once the pediatric study is complete, Baxter expects to file for marketing authorization with the European Medicines Agency in 2016. Upon approval, BAX 855 is expected to be produced at the company's Singapore manufacturing facility, one of several sites currently approved for ADVATE production.

BAX 855 is based on ADVATE, a full-length FVIII molecule with more than 10 years of real-world experience. Through a collaboration with Nektar Therapeutics (NASDAQ: NKTR), BAX 855 leverages proprietary PEGylation technology designed to extend the duration of activity of proteins in the body. This proprietary technology has been used for over 10 years in a number of approved medicines that treat chronic or serious conditions.

#### **About ADVATE**

ADVATE is a recombinant antihemophilic factor indicated for use in children and adults with hemophilia A (congenital factor VIII deficiency or classic hemophilia) for:

- Control and prevention of bleeding episodes.
- Perioperative management.
- Routine prophylaxis to prevent or reduce the frequency of bleeding episodes.

ADVATE is not indicated for the treatment of von Willebrand disease.

ADVATE has a demonstrated efficacy and safety profile. ADVATE is a full-length (derived from the complete FVIII gene) recombinant FVIII product that is processed without any blood-based additives. Because no blood-derived components are added at any stage of the manufacturing process, the potential risk of transmitting pathogens that may be carried in blood-based additives is eliminated. There have been no confirmed reports of transmission of HIV, HBV or HCV with rFVIII treatments.

ADVATE is the world's most prescribed FVIII treatment. It is currently approved in 64 countries worldwide, including the United States, Canada, 28 countries in the European Union, Algeria, Argentina, Australia, Brazil, Chile, China, Colombia, Ecuador,

Hong Kong, Iceland, Iraq, Israel, Japan, Kuwait, Macau, Malaysia, Mexico, Morocco, New Zealand, Norway, Panama, Puerto Rico, Russia, Saudi Arabia, Serbia, Singapore, South Korea, Suriname, Switzerland, Taiwan, Tunisia, Turkey, Ukraine, Uruguay, and Venezuela.

## **Detailed Important Risk Information for ADVATE [Antihemophilic Factor (Recombinant)]**

### **CONTRAINDICATIONS**

ADVATE is contraindicated in patients who have life-threatening hypersensitivity reactions, including anaphylaxis, to mouse or hamster protein or other constituents of the product.

### **WARNINGS & PRECAUTIONS**

#### **Hypersensitivity Reactions**

Allergic-type hypersensitivity reactions, including anaphylaxis, have been reported with ADVATE. Symptoms include dizziness, paresthesia, rash, flushing, facial swelling, urticaria, dyspnea, and pruritus.

Discontinue ADVATE if hypersensitivity symptoms occur and administer appropriate emergency treatment.

#### **Neutralizing Antibodies**

Neutralizing antibodies (inhibitors) have been reported following administration of ADVATE predominantly in previously untreated patients (PUPs) and previously minimally treated patients (MTPs). Monitor all patients for the development of factor VIII inhibitors by appropriate clinical observation and laboratory testing. If expected plasma factor VIII activity levels are not attained, or if bleeding is not controlled with an expected dose, perform an assay that measures factor VIII inhibitor concentration.

### **ADVERSE REACTIONS**

The serious adverse reactions seen with ADVATE are hypersensitivity reactions and the development of high-titer inhibitors necessitating alternative treatments to factor VIII. The most common adverse reactions observed in clinical trials (frequency  $\geq 10\%$  of subjects) were pyrexia, headache, cough, nasopharyngitis, vomiting, arthralgia, and limb injury. **Please see full prescribing information for ADVATE at:**

[http://www.baxter.com/downloads/healthcare\\_professionals/products/ADVATE\\_PI.pdf](http://www.baxter.com/downloads/healthcare_professionals/products/ADVATE_PI.pdf)

#### **About Baxter in Hemophilia**

Baxter has more than 60 years experience in hemophilia and has introduced a number of therapeutic firsts for hemophilia patients. Baxter has the broadest portfolio of hemophilia treatments in the industry and is able to meet individual therapy choices, providing a range of options at each treatment stage. The company's work focuses on optimizing hemophilia care and improving the lives of people worldwide living with bleeding disorders.

#### **About Baxter International Inc.**

Baxter International Inc., through its subsidiaries, develops, manufactures and markets products that save and sustain the lives of people with hemophilia, immune disorders, cancer, infectious diseases, kidney disease, trauma and other chronic and acute medical conditions. As a global, diversified healthcare company, Baxter applies a unique combination of expertise in medical devices, pharmaceuticals and biotechnology to create products that advance patient care worldwide.

*This release includes forward-looking statements concerning BAX 855, including expectations with regard to clinical studies and related data, regulatory filings, future manufacturing plans and its potential impact to patients. The statements are based on assumptions about many important factors, including the following, which could cause actual results to differ materially from those in the forward-looking statements: satisfaction of regulatory and other requirements; actions of regulatory bodies and other governmental authorities; additional clinical results; changes in laws and regulations; product quality or supply or patient safety issues; and other risks identified in Baxter's most recent filing on Form 10-K and other SEC filings, all of which are available on Baxter's website. Baxter does not undertake to update its forward-looking statements.*

#### **Media Contact**

Brian Kyhos  
(224) 948-5353, [media@baxter.com](mailto:media@baxter.com)

**Investor Contact**

Mary Kay Ladone, (224) 948-3371

Clare Trachtman, (224) 948-3085

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/baxter-submits-application-for-us-fda-approval-of-bax-855-extended-half-life-recombinant-fviii-based-on-advate-for-hemophilia-a-300002465.html>

SOURCE Baxter International Inc.

News Provided by Acquire Media