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Baxalta to Advance Care for Hemophilia A patients with FDA Approval of ADYNOVATE, a Simple, Twice-weekly Treatment to Reduce Bleeds

- ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated] is built on ADVATE [Antihemophilic Factor (Recombinant)], a well-established treatment for hemophilia A patients with more than 12 years of patient experience
- Proprietary pegylation technology (1) to extend the time between treatments and offer simple, twice-weekly dosing schedule compared to conventional 3-4 doses weekly
- ADYNOVATE prophylaxis resulted in 95 percent fewer bleeds compared to on-demand treatment in the pivotal study; nearly 40 percent of treated patients receiving ADYNOVATE prophylaxis experienced zero bleeds (2)

BANNOCKBURN, Ill., Nov. 16, 2015 /PRNewswire/ -- Baxalta Incorporated (NYSE: BXLT), a global biopharmaceutical leader dedicated to delivering transformative therapies to patients with orphan diseases and underserved conditions, announced today that the U.S. Food and Drug Administration (FDA) has approved ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated], an extended circulating half-life recombinant Factor VIII (rFVIII) treatment for hemophilia A. ADYNOVATE is built on the full-length ADVATE [Antihemophilic Factor (Recombinant)] molecule, a leading treatment for hemophilia A with more than 12 years of real-world patient experience.

"People with hemophilia want to minimize their bleeds and not let their hemophilia dictate their lives; having options empowers patients to make the best decisions for their personal care," said Val Bias, chief executive officer, National Hemophilia Foundation. "We applaud Baxalta for continuing to innovate for the care of hemophilia."

"ADYNOVATE represents Baxalta's commitment to meeting the needs of hemophilia A patients who want bleed reduction with a dosing schedule that better fits their personal needs," said Ludwig Hantson, chief executive officer and president, Baxalta. "As the first new product approved under the Baxalta name, ADYNOVATE represents a major milestone in achieving our goal of 20 product launches by 2020 and continuing to expand our world-leading hemophilia treatment portfolio."

In the pivotal phase 3 clinical trial, which served as the foundation for the approval, ADYNOVATE demonstrated efficacy in treating hemophilia patients through routine prophylaxis as well as for on-demand bleeding episodes. Patients, 12 to 65 years of age, in the prospective, global, multi-center, open label, non-randomized study were assigned to either twice weekly prophylaxis (40-50 IU/kg, n=120) or on-demand treatment (10-60 IU/kg, n=17) with ADYNOVATE. The study found that previously-treated patients in a twice-weekly prophylaxis arm had 95 percent fewer annual bleeds compared to those treated on-demand [median annual bleed rate (ABR) 1.9 vs. 41.5, respectively]. During the study, 38 percent (n=120) of prophylaxis-treated patients experienced zero bleeds. Moreover, 57 percent of patients experienced zero joint bleeds based on six months of prophylaxis with ADYNOVATE.[2]

Nearly all (98 percent) of patients on prophylaxis with ADYNOVATE did not have a dose adjustment in the study. Nearly all (96 percent) bleeding episodes (n=591) were controlled with one or two infusions of ADYNOVATE. No patients developed inhibitors to the treatment; the most common adverse reactions (≥1 percent of subjects) were headache and nausea.[2]

ADYNOVATE will be available in the United States in the coming weeks. Baxalta continues to invest in ADYNOVATE to expand the product's value for more patients worldwide. Currently, studies are ongoing in previously treated patients (PTPs) with severe hemophilia A undergoing surgery and in pediatric PTPs under the age of 12 with severe hemophilia A. Additionally, Baxalta will initiate a study in previously-untreated patients (PUPs) with severe hemophilia A. Baxalta has filed for regulatory approval of the treatment in Japan and following completion of the pediatric study, expects to file for marketing authorization in Europe.

Hemophilia A is a challenging chronic disease; treatment regimens require regular infusions to reduce the risk of bleeding. Working closely with their health professionals, many patients continue to seek treatment options that can be better personalized to fit their needs, providing both effective bleed protection and simplified dosing schedules. Today, the disease affects approximately 16,000 people in the United States and more than 400,000 people worldwide. With an estimated 75 percent of people with hemophilia undiagnosed and untreated or undertreated globally,[3] Baxalta continues to innovate in order

to address some of the greatest challenges associated with hematologic disorders, including hemophilia.

Through a collaboration with Nektar Therapeutics (NASDAQ: NKTR), ADYNOVATE leverages proprietary pegylation technology designed to prolong the amount of FVIII available for use in the body. The technology was selected because it maintains the integrity of the parent molecule (ADVATE) and reduces the speed at which the body clears ADYNOVATE, resulting in increased circulating half-life. This proprietary technology has been used for more than 15 years in a number of approved medicines that treat chronic or serious conditions. In addition to the patents relating to pegylated FVIII, Baxalta has an exclusive license in the field of hemophilia to certain other patents relating to pegylated FVIII proteins. These patents protect Baxalta's pipeline of extended circulating half-life FVIII products, including ADYNOVATE.

About ADYNOVATE

ADYNOVATE, Antihemophilic Factor (Recombinant), PEGylated, is a human antihemophilic factor indicated in adolescent and adult patients (12 years and older) with hemophilia A (congenital factor VIII deficiency) for:

- On-demand treatment and control of bleeding episodes
- Routine prophylaxis to reduce the frequency of bleeding episodes

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

DETAILED IMPORTANT RISK INFORMATION
CONTRAINDICATIONS

ADYNOVATE is contraindicated in patients who have had prior anaphylactic reaction to ADYNOVATE, to the parent molecule (ADVATE), mouse or hamster protein, or excipients of ADYNOVATE (e.g. Tris, mannitol, trehalose, glutathione, and/or polysorbate 80).

WARNINGS & PRECAUTIONS

Hypersensitivity Reactions

Hypersensitivity reactions are possible with ADYNOVATE. Allergic-type hypersensitivity reactions, including anaphylaxis, have been reported with other recombinant antihemophilic factor VIII products, including the parent molecule, ADVATE. Early signs of hypersensitivity reactions that can progress to anaphylaxis may include angioedema, chest tightness, dyspnea, wheezing, urticaria, and pruritus. Immediately discontinue administration and initiate appropriate treatment if hypersensitivity reactions occur.

Neutralizing Antibodies

Formation of neutralizing antibodies (inhibitors) to factor VIII can occur following administration of ADYNOVATE. Monitor patients regularly for the development of factor VIII inhibitors by appropriate clinical observations and laboratory tests. Perform an assay that measures factor VIII inhibitor concentration if the plasma factor VIII level fails to increase as expected, or if bleeding is not controlled with expected dose.

ADVERSE REACTIONS

Common adverse reactions (≥1% of subjects) reported in the clinical studies were headache and nausea.

For Full Prescribing Information, visit http://baxalta.com/assets/documents/ADYNOVATE_PI.pdf.

About ADVATE

ADVATE [Antihemophilic Factor (Recombinant)] is a recombinant antihemophilic factor indicated for use in children and adults with hemophilia A (congenital factor VIII deficiency) 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 for the treatment of hemophilia A. 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 virtually 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, pruritus, and vomiting.

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

Serious adverse reactions seen with ADVATE are hypersensitivity reactions, including anaphylaxis, and the development of high-titer inhibitors necessitating alternative treatments to factor VIII.

The most common adverse reactions observed in clinical trials (frequency ≥5% of subjects) were pyrexia, headache, cough, nasopharyngitis, arthralgia, vomiting, upper respiratory tract infection, limb injury, nasal congestion, and diarrhea.

Please see full prescribing information for ADVATE at: www.advate.com /assets/pdf/advate_iri_pi.pdf.

About Baxalta

Baxalta Incorporated (NYSE: BXLT) is a $6 billion global biopharmaceutical leader developing, manufacturing and commercializing therapies for orphan diseases and underserved conditions in hematology, oncology and immunology. Driven by passion to make a meaningful impact on patients' lives, Baxalta's broad and diverse pipeline includes biologics with novel mechanisms and advanced technology platforms such as gene therapy. The Baxalta Global Innovation and R&D Center is located in Cambridge, Massachusetts. Launched in 2015 following separation from Baxter International Inc, Baxalta's heritage in biopharmaceuticals spans decades. Baxalta's therapies are available in more than 100 countries and it has advanced biological manufacturing operations across 12 facilities, including state-of-the-art recombinant production and plasma fractionation. Headquartered in Northern Illinois, Baxalta employs 16,000 employees worldwide.

Forward-Looking Statements

This release includes forward-looking statements concerning ADYNOVATE, including expectations with regard to clinical trials, future regulatory actions, expected launch plans and potential impact on patients. Such statements are made of the date that they were first issued and are based on current expectations, beliefs and assumptions of management. Forward-looking statements are subject to a number of risks and uncertainties, many of which involve factors or circumstances that are beyond

Baxalta's control and which could cause actual results to differ materially from those in the forward-looking statements, including the following: clinical trial results; satisfaction of regulatory and other requirements; actions of regulatory bodies and other governmental authorities; changes in laws and regulations; product quality, manufacturing or supply issues; patient safety issues; and other risks identified in Baxalta's Registration Statement on Form 10 and other Securities and Exchange Commission filings, all of which are available on Baxalta's website. Baxalta expressly disclaims any intent or obligation to update these forward-looking statements except as required by law.

References:

1. Proprietary pegylation technology exclusively licensed from Nektar Therapeutics.
2. Konkle, Barbara, et al. Pegylated, full-length, recombinant factor VIII for prophylactic and on- demand treatment of severe hemophilia A. Blood. July 2015.
3. National Hemophilia Foundation. Fast Facts. Accessed August 16, 2015. https://www.hemophilia.org/About-Us/Fast-Facts.

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