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Baxalta Expands Global Reach of ADYNOVATE for Hemophilia A Patients with Approval in Japan

- **ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated] approved for treatment of patients 12 years and older with hemophilia A in Japan**
- **Built on ADVATE [Antihemophilic Factor (Recombinant)], the world's most widely used FVIII treatment, ADYNOVATE offers a simple, twice-weekly dosing schedule**
- **Approval expands access of ADYNOVATE to the nearly 5,000 people in Japan with hemophilia A[1]**

BANNOCKBURN, Ill.--(BUSINESS WIRE)-- 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 Ministry of Health, Labour and Welfare in Japan approved ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated], an extended circulating half-life recombinant Factor VIII (rFVIII) treatment, for patients 12 years and older with hemophilia A.

ADYNOVATE is built on ADVATE [Antihemophilic Factor (Recombinant)], the world's most widely used FVIII treatment for hemophilia A with more than 12 years of patient experience. ADYNOVATE leverages proprietary pegylation technology[2] designed to extend the time FVIII is available in the body. The technology reduces the speed at which ADYNOVATE is cleared from the blood, resulting in increased circulating half-life.

"This approval provides people with hemophilia A in Japan with a new innovative treatment option that's built on the proven science of factor replacement and more specifically, the world's most widely used FVIII treatment for hemophilia A," said Brian Goff, executive vice president and president, Hematology, Baxalta. "We remain dedicated to continually expanding the reach of ADYNOVATE for patients around the world."

The approval was based on positive results of a Phase 3, prospective, global, multi-center, open label, non-randomized study in patients 12 to 65 years of age with hemophilia A. The study found that previously-treated patients in a twice-weekly prophylaxis arm had a 95 percent reduction in the median overall annualized bleed rate (ABR) compared to those treated on-demand [1.9 vs. 41.5, respectively]. During the study, nearly 40 percent (n=120) of prophylaxis-treated patients experienced zero bleeds. No patients developed inhibitors to the treatment; the most common adverse reactions (≥1 percent of subjects) were headache and nausea.[3]

Japanese patients (n=11) participated in the pivotal clinical trial where ADYNOVATE demonstrated efficacy in treating hemophilia patients through routine prophylaxis as well as for on-demand bleeding episodes.[3]

Following approval from the Ministry of Health, Labour and Welfare on March 28, the marketing authorization was transferred from Baxter Limited to Baxalta Japan Limited. In the United States, ADYNOVATE is currently licensed for use in adult and adolescent patients (12 years and older) and is under regulatory review for use in pediatric and surgical settings. ADYNOVATE is also under regulatory review in Canada, Switzerland and Europe.

About ADYNOVATE in the United States

ADYNOVATE is built on the full-length ADVATE molecule, a leading treatment for hemophilia A that has been used by patients worldwide for more than 12 years. Through a collaboration with Nektar Therapeutics (NASDAQ: NKTR), ADYNOVATE leverages proprietary pegylation technology designed to extend the time FVIII is available in the body. The technology reduces the speed at which ADYNOVATE is cleared from the blood, 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.

Indications:

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 in the United States

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 widely used FVIII treatment. It is currently approved in 67 countries worldwide, including the United States, Canada, 28 countries in the European Union, Algeria, Argentina, Australia, Brazil, Brunei, Chile, China, Colombia, Ecuador, Hong Kong, Iceland, India, Iraq, Israel, Japan, Kuwait, Macau, Malaysia, Mexico, Morocco, New Zealand, Norway, Panama, Puerto Rico, Qatar, Russia, Saudi Arabia, Serbia, Singapore, South Korea, Suriname, Switzerland, Taiwan, Tunisia, Turkey, Ukraine, Uruguay, and Venezuela.

Indications:

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.

Detailed Important Risk Information

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.

For full prescribing information for ADVATE, visit: http://www.baxalta.com/assets/documents/ADVATE_PI.pdf.

About Baxalta

Baxalta Incorporated (NYSE: BXLT) is a global biopharmaceutical leader developing, manufacturing and commercializing therapies for orphan diseases and underserved conditions in hematology, immunology and oncology. 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. 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, with its Global Innovation Center in Cambridge, Mass., Baxalta employs 17,000 employees worldwide.

Forward-Looking Statements

This release includes forward-looking statements concerning ADYNOVATE, including expectations with regard to regulatory filings. 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: 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 filings with the Securities and Exchange Commission, 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. Report on the Annual Global Survey 2014. World Federation of Hemophilia. October 2015.

2. Proprietary pegylation technology exclusively licensed from Nektar Therapeutics.

3. Konkle, Barbara, et al. Pegylated, full-length, recombinant factor VIII for prophylactic and on- demand treatment of severe hemophilia A. Blood. July 2015.

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