Baxalta Announces the FDA Approval of ADYNOVATE: A Hemophilia A Treatment That Delivers Proven Prophylaxis with a Twice-Weekly Dosing Schedule

Baxalta is pleased to announce the approval of ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated], a new treatment option for adolescents and adults (12 years and older) with hemophilia A. ADYNOVATE can be used on-demand to control bleeding, or used regularly (prophylaxis) to reduce the frequency of bleeding episodes. ADYNOVATE is not indicated for the treatment of von Willebrand disease.1

ADYNOVATE is the first product to be launched by Baxalta, formerly Baxter BioScience, a company with over 60 years of experience in hemophilia. ADYNOVATE is built on the full-length ADVATE [Antihemophilic Factor (Recombinant)] molecule, and uses an established technology* to extend the circulating half-life by 40% to 50%. This allows for proven prophylaxis with a simple, twice-weekly dosing schedule (infusions on the same 2 days every week).1

In the pivotal study, ADYNOVATE prophylaxis delivered a 95% reduction in median overall annualized bleed rate (ABR) compared with on-demand treatment [Median total ABR: 1.9 (IQR, 0.0; 5.9) reduced from 41.5 (IQR, 31.7; 51.1) for on-demand. Mean total ABR: 4.7 (standard deviation [SD], 8.6) reduced from 40.8 (SD, 16.3) for on-demand]. Approximately 2 out of every 5 patients achieved 0 bleeding episodes [38% of patients in the treated population† (n=120) achieved zero bleeding episodes. 40% of patients in the per-protocol population‡ (n=101) achieved zero bleeding episodes], and the median ABR was 0 for both joint and spontaneous bleeds [Mean ABR for joint bleeds: 2.9 (SD, 8.0) reduced from 34.7 (SD, 15.1) for on-demand. Mean ABR for spontaneous bleeds: 2.9 (SD, 7.1) reduced from 26.0 (SD, 19.6) for on-demand]1

Zero people developed inhibitors to factor VIII in ADYNOVATE clinical studies. Common adverse reactions (≥1% of subjects) reported in clinical studies were headache and nausea.1

*Proprietary PEGylation Technology exclusively licensed from Nektar Therapeutics.
†Treated population=subjects assigned to routine prophylaxis or on-demand treatment with ADYNOVATE and who received at least one dose of the product.
‡Per-protocol population=all subjects who were assigned to the prophylactic or the on-demand treatment regimen, treated with their originally assigned dose for the entire duration of study participation and who fulfilled the protocol compliance requirements.
About ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated]

Indication
ADYNOVATE is used on-demand to control bleeding in patients 12 years of age and older with hemophilia A. ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION
You should not use ADYNOVATE if you:
- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE [Antihemophilic Factor (Recombinant)]

Tell your healthcare provider if you are pregnant or breastfeeding because ADYNOVATE may not be right for you.

You should tell your healthcare provider if you:
- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

Your body may form inhibitors to Factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADYNOVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to Factor VIII.

You can have an allergic reaction to ADYNOVATE.
Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

The common side effects of ADYNOVATE are headache and nausea. Tell your healthcare provider about any side effects that bother you or do not go away.

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.

To see the full Prescribing Information go to www.ADYNOVATE.com.
About ADVATE [Antihemophilic Factor (Recombinant)]

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.

Indications
ADVATE is a medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called “classic” hemophilia).

ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A.

Your healthcare provider may give you ADVATE when you have surgery.

ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION

You should not use ADVATE if you:

- Are allergic to mice or hamsters.
- Are allergic to any ingredients in ADVATE.

Tell your healthcare provider if you are pregnant or breastfeeding because ADVATE may not be right for you.

You should tell your healthcare provider if you:

- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Have been told that you have inhibitors to factor VIII (because ADVATE may not work for you).

Your body may form inhibitors to factor VIII. An inhibitor is part of the body’s normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

You can have an allergic reaction to ADVATE.
Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

Side effects that have been reported with ADVATE include: cough, headache, joint swelling/aching, sore throat, fever, itching, dizziness, hematoma, abdominal pain, hot flashes, swelling of legs, diarrhea, chills, runny nose/congestion, nausea/vomiting, sweating, and rash.

Tell your healthcare provider about any side effects that bother you or do not go away or if your bleeding does not stop after taking ADVATE.

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.

To see the full Prescribing Information go to www.ADVATE.com.

About Baxalta
Baxalta Incorporated (NYSE: BXLT) is a $6 billion global biopharmaceutical leader developing, manufacturing, and commercializing treatments for orphan diseases and underserved conditions in hematology, oncology, and immunology. 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 treatments 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 Web site. Baxalta expressly disclaims any intent or obligation to update these forward-looking statements except as required by law.
References:
1. ADYNOVATE Prescribing Information. Westlake Village, CA: Baxalta US Inc.