



FOR IMMEDIATE RELEASE

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**BAXTER PROVIDES PROGRESS UPDATE ON GENE THERAPY PROGRAM,  
INCLUDING PHASE I/II CLINICAL TRIAL OF BAX 335, INVESTIGATIONAL GENE  
THERAPY TREATMENT FOR HEMOPHILIA B**

**DEERFIELD, III., FEBRUARY 12, 2015** – Baxter International Inc. (NYSE:BAX) today provided an update on its gene therapy program, including progress on the Phase I/II open-label clinical trial assessing the safety and optimal dosing level of BAX 335, an investigational factor IX (FIX) gene therapy treatment for hemophilia B, during a sponsored symposium at the 8<sup>th</sup> Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) in Helsinki, Finland.

The trial is assessing the safety of ascending doses of BAX 335 to determine the optimal single dose in up to 16 adult patients with hemophilia B at treatment centers in the United States. The primary endpoint is the safety of a single dose of BAX 335 administered intravenously. Secondary endpoints include evaluation of the optimal dose to achieve stable therapeutic plasma FIX activity, as well as pharmacokinetics and immune response to treatment.

As of the end of 2014, a total of six patients in three dosing cohorts have been treated in the trial with evidence of a dose-related response. No patients have developed FIX

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inhibitors to date. In the two highest dose cohorts, FIX activity levels around 10 percent or above have been observed in two patients, who also experienced no bleeding events. One of these patients showed elevated levels of liver enzymes indicative of an immune response, which is being treated with oral corticosteroids, per protocol. Immune responses have been reported in previous studies with gene therapy technology. Additional patients are being screened and more information on the trial is available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov), by using Identifier #01687608.

“We continue to make steady progress in advancing our hemophilia B program with this technology and look forward to better understanding the applicability of this technology platform in hemophilia A patients as well,” said John Orloff, MD, vice president and global head of research and development at Baxter BioScience. “With the potential to redefine the treatment of hemophilia, this gene therapy technology is a central part of our R&D focus as we prepare to become an independent company this year,”

Patients with hemophilia B lack the ability to produce clotting factor IX and are treated with plasma-derived or recombinant factor IX today. BAX 335 is designed to provide a mechanism for the patient’s own liver to begin producing factor IX over an extended period following a single dose of the genetically engineered treatment.

In April 2014, Baxter announced the acquisition of Chatham Therapeutics, LLC, an affiliate of Asklepios BioPharmaceutical, Inc. (AskBio), and its developmental gene therapy programs. Chatham’s Biological Nano Particles (BNP), an advanced recombinant adeno-associated virus- (rAAV-) based gene therapy technology, has shown potential therapeutic

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benefit in early studies. In addition to the research in hemophilia B, Baxter is also advancing plans to evaluate the gene therapy technology in the treatment of hemophilia A.

**About Hemophilia B**

Hemophilia B is the second most common type of hemophilia (also known as Christmas disease) and is the result of insufficient amounts of clotting factor IX, a naturally occurring protein in blood that controls bleeding. Approximately 26,000 people worldwide, including more than 4,000 in the U.S., have been diagnosed with hemophilia B. Hemophilia B is often a debilitating, chronic disease with complications that include bleeding episodes, hemophilic arthropathy (bleeding into a joint) and hospitalization.

**About Baxter in Hemophilia**

Baxter has more than 60 years of scientific experience in supporting the treatment needs of patients with hemophilia and has introduced a number of therapeutic firsts. Baxter has the broadest portfolio of hemophilia treatments and is able to meet individualized patient therapeutic needs by 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.

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**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 a Phase I/II open-label clinical trial of BAX 335, as well as plans to separate Baxter's biopharmaceutical and medical products businesses and related research and development strategies. These 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: clinical trial results; satisfaction of regulatory and other requirements; actions of regulatory bodies and other governmental authorities; changes in laws and regulations; product quality 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.*

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