



## **U.S. FDA Approves CSL Behring's IDELVION® The First and Only Hemophilia B Therapy with Up to 14-day Dosing Intervals**

- *In clinical trials, **IDELVION** maintained factor IX activity levels above 5 percent over 14 days, resulting in a median annualized spontaneous bleeding rate (AsBR) of 0.00*
- ***IDELVION**, CSL Behring's long-acting recombinant albumin fusion protein, delivers on the company's promise to develop and provide innovative specialty biotherapies that help people with serious medical conditions live full lives*

**KING OF PRUSSIA, Pa. – March 4, 2016 –** CSL Behring announced today that the U.S. Food and Drug Administration (FDA) has approved IDELVION® [Coagulation Factor IX (Recombinant), Albumin Fusion Protein], its novel, long-acting albumin fusion protein linking recombinant coagulation factor IX with recombinant albumin for the treatment of hemophilia B. IDELVION is the first and only factor IX therapy that delivers high-level protection with up to 14-day dosing in appropriate patients. This dosing interval has been achieved while maintaining high levels of factor activity, above 5 percent over 14 days at 75 IU/kg. This reduces the monthly number of units needed for prophylaxis therapy.

**IDELVION** is indicated in children and adults with hemophilia B for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; on-demand control and prevention of bleeding episodes; and the perioperative management of bleeding (around the time of surgery). Appropriate patients 12 and older can go up to 14 days between infusions. **IDELVION** is expected to be available later this month.

Hemophilia B is a congenital bleeding disorder characterized by deficient or defective factor IX; nearly all affected patients are male. People with hemophilia B may experience prolonged or spontaneous bleeding, especially into the muscles, joints, or internal organs. According to U.S. Centers for Disease Control and Prevention, the condition affects approximately one in 25,000 male births.

“The approval of this long-acting recombinant factor IX therapy for hemophilia B is vital, as physicians need more options to help their patients effectively and safely manage their bleeding disorder,” said Elena Santagostino, M.D., Ph.D., Professor in the Medical School of Clinical and Experimental Hematology at the University of Milan/IRCCS Maggiore Hospital, and lead investigator of the PROLONG-9FP clinical development program. “As the only recombinant factor IX therapy offering up to 14-day dosing, **IDELVION** helps patients maintain factor IX activity levels over a long period of time. This provides them with greater freedom from frequent infusions which is an important attribute for patients who require a prophylactic regimen but don’t want treatment to disrupt their active lives.”

The approval of **IDELVION** is based on results from the PROLONG-9FP clinical development program. PROLONG-9FP includes Phase I through Phase III open-label, multicenter studies evaluating the safety and efficacy of **IDELVION** in children and adults (ages 1 to 61 years) with hemophilia B (factor IX levels  $\leq$  2%).

“**IDELVION** has the potential to significantly impact the treatment of hemophilia B as it maintains factor IX activity levels above 5 percent over a prolonged period of time. This provides excellent bleeding control,” said Dr. Andrew Cuthbertson, Chief Scientific Officer and R&D Director, CSL Limited. “**IDELVION** is the first product from our innovative recombinant factor development program to receive FDA approval. We are proud to add this new therapy to our growing portfolio of bleeding disorder products, and are particularly excited about the positive impact treatment with **IDELVION** can have on the well-being of patients with hemophilia B.”

### **About PROLONG-9FP**

The data from PROLONG-9FP showed median annualized spontaneous bleeding rates (AsBR) of zero and factor IX activity levels above 5 percent in patients using **IDELVION**<sup>®</sup> prophylactically. According to the World Federation of Hemophilia, patients with factor IX activity levels above 5 percent (and below 50 percent) are considered to have mild hemophilia. This result was achieved for both 14-day dosing and 7-day dosing. The data for on-demand therapy showed that 94 percent of bleeds were controlled with one infusion, while 99 percent were controlled with one or two infusions. The most common adverse reaction in clinical trials was headache. Data from

PROLONG-9FP were recently published in the American Society of Hematology's publication [Blood](#).

## **About IDELVION®**

CSL Behring engineered IDELVION® to extend the half-life of recombinant factor IX through fusion with recombinant albumin. CSL Behring selected recombinant albumin as its fusion partner for its coagulation factor proteins due to its long physiological half-life.

IDELVION® is approved in Canada. The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) recently recommended granting marketing authorization for **IDELVION** in the European Union. Regulatory agencies in Australia, Switzerland and Japan are also currently reviewing CSL Behring's license applications for **IDELVION**.

For more information about **IDELVION**, including full prescribing information, please visit <http://www.idelvion.com/idelvion-prescribing-information>. For more information about CSL Behring's recombinant products in development to treat hemophilia, visit <http://www.cslbehring.com/products/bleeding-disorders/novel-recombinant-hemophilia-treatments>.

## **Important Safety Information**

IDELVION®, Coagulation Factor IX (Recombinant), Albumin Fusion Protein (rFIX-FP), is indicated in children and adults with hemophilia B (congenital factor IX deficiency) for:

- On-demand control and prevention of bleeding episodes
- Perioperative management of bleeding
- Routine prophylaxis to prevent or reduce the frequency of bleeding episodes

**IDELVION** is not indicated for induction of immune tolerance in patients with hemophilia B.

**IDELVION** is contraindicated in patients who have had life-threatening hypersensitivity to the product or its components, including hamster proteins.

**IDELVION** is for intravenous use only. **IDELVION** can be self-administered or administered by a caregiver with training and approval from a healthcare provider or hemophilia treatment center. Higher dose per kilogram body weight or more frequent dosing may be needed for pediatric patients.

Hypersensitivity reactions, including anaphylaxis, are possible. Advise patients who self-administer to immediately report symptoms of hypersensitivity, including angioedema, chest tightness, hypotension, generalized urticaria, wheezing, and dyspnea. If symptoms occur, discontinue **IDELVION** and administer appropriate treatment.

Development of neutralizing antibodies (inhibitors) to **IDELVION** may occur. If expected factor IX activity plasma levels are not attained or bleeding is not controlled with appropriate dose, perform an assay to measure factor IX inhibitor concentration. Factor IX activity assay results may vary with the type of activated partial thromboplastin time reagent used.

Thromboembolism (e.g., pulmonary embolism, venous thrombosis, and arterial thrombosis) can occur when using factor IX-containing products. In addition, nephrotic syndrome has been reported following immune tolerance induction in hemophilia B patients with factor IX inhibitors and allergic reactions to factor IX.

The most common adverse reaction (incidence  $\geq 1\%$ ) reported in clinical trials was headache.

## **About CSL Behring**

CSL Behring is a global biotherapeutics leader which is driven by its promise to save lives. Focused on serving patients' needs by using the latest technologies, we develop and deliver innovative therapies that are used to treat coagulation disorders, primary immune deficiencies, hereditary angioedema, inherited respiratory disease, and neurological disorders. The company's products are also used in cardiac surgery, organ transplantation, burn treatment and to prevent hemolytic disease of the newborn.

CSL Behring operates one of the world's largest plasma collection networks, CSL Plasma. The parent company, [CSL Limited](#) (ASX:CSL), headquartered in Melbourne, Australia, employs more than 16,000 people with operations in more than 30 countries. For more information visit [www.cslbehring.com](http://www.cslbehring.com) and follow us on [www.Twitter.com/CSLBehring](http://www.Twitter.com/CSLBehring).

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