Haegarda® (C1 esterase inhibitor [human]) – New orphan drug approval

- On June 22, 2017, the FDA announced the approval of CSL Behring's Haegarda (C1 esterase inhibitor [C1-INH] [human]) for subcutaneous injection, indicated for routine prophylaxis to prevent hereditary angioedema (HAE) attacks in adolescent and adult patients.

- HAE is caused by having insufficient amounts of a plasma protein called C1-INH. People with HAE can develop rapid swelling of the hands, feet, limbs, face, intestinal tract or airway. These attacks of swelling can occur spontaneously, or can be triggered by stress, surgery or infection. HAE affects approximately 6,000 to 10,000 people in the U.S.

- Haegarda is a human plasma-derived, purified, pasteurized, lyophilized (freeze-dried) concentrate prepared from large pools of human plasma from U.S. donors. It is the first subcutaneous therapy approved for the prevention of HAE.

- The efficacy and safety of Haegarda for routine prophylaxis to prevent HAE attacks in adolescent and adult patients was demonstrated in a clinical trial of 90 patients treated for 16 weeks with Haegarda 40 international units (IU)/kg or 60 IU/kg or placebo.

    — The mean number of HAE attacks per month decreased significantly more in the active treatment groups vs. the placebo groups (Haegarda 40 IU/kg: 1.2 vs. 3.6 with placebo; $p < 0.001$ and Haegarda 60 IU/kg: 0.5 vs. 4.0 with placebo; $p < 0.001$).
    — The mean number of uses of rescue medication per month was also significantly decreased in the Haegarda groups vs. the placebo groups (Haegarda 40 IU/kg: 1.1 vs. 5.6 with placebo and Haegarda 60 IU/kg: 0.3 vs. 3.9 with placebo).

- Haegarda is contraindicated in individuals who have experienced life-threatening hypersensitivity reactions, including anaphylaxis, to C1-INH preparations or its excipients.

- Warnings and precautions of Haegarda include hypersensitivity, thromboembolic events, and transmissible infectious agents.

- The most common adverse reactions ($\geq$ 4%) with Haegarda use were injection site reaction, hypersensitivity, nasopharyngitis, and dizziness.

- The recommended dose of Haegarda is 60 IU/kg body weight by subcutaneous injection twice weekly (every 3 or 4 days).

- CSL Behring plans to launch Haegarda in the fourth quarter of 2017. Haegarda will be available as a white lyophilized powder supplied in single-use vials containing 2,000 or 3,000 IU of C1-INH.