Berinert® (C1 esterase inhibitor [human]) – Expanded Indication

- On July 18, 2016, CSL Behring announced the FDA approval of Berinert (C1 esterase inhibitor [human]) injection, for the treatment of acute abdominal, facial, or laryngeal hereditary angioedema (HAE) attacks in adult and pediatric patients.
    - Previously, Berinert was only approved for use in adults and adolescents.
    - The safety and efficacy of Berinert for prophylactic therapy have not been established.
- HAE is a rare, but serious, genetic disorder affecting 1 in 10,000 to 50,000 individuals.
    - Attacks often start in childhood and become more severe over time.
    - HAE attacks may cause swelling and edema, affecting specific body parts, including the abdomen, face, and throat.
- The expanded indication for Berinert was approved based on placebo-controlled and open-label extension studies in 12 pediatric patients (ages 10 – 16 years old) with HAE. Berinert was also evaluated in 18 pediatric patients (ages 5 – 11 years old) with HAE in a registry study conducted in the U.S. and Europe. The safety profile observed in the pediatric population was similar to that observed in adults.
    - The pharmacokinetics of Berinert were evaluated in 5 pediatric patients (ages 6 – 13 years old). When adjusted for baseline, compared to adults, the half-life of Berinert was shorter and clearance (on per kilogram [kg] basis) was faster in this limited cohort of children.
- In addition, Berinert was evaluated in 27 geriatric patients (age 65 – 83 years old) with HAE. While the safety and efficacy of Berinert in this population have not been evaluated in controlled clinical studies, the safety profile was similar to that observed in the younger populations that were studied.
- The recommended dose of Berinert is 20 international units (IU) per kg of body weight given by intravenous injection.
    - Doses lower than 20 IU/kg body weight should not be administered.
    - Berinert must be reconstituted and administered using a silicon-free syringe and given at room temperature within 8 hours of reconstitution.