Andembry® (garadacimab-gxii) – New orphan drug approval

- On June 16, 2025, CSL announced the FDA approval of Andembry (garadacimab-gxii), for **prophylaxis to prevent attacks of hereditary angioedema (HAE) in adult and pediatric patients aged 12 years and older**.

- HAE is a genetic condition that occurs in about 1 in 10,000 to 1 in 50,000 people. HAE is caused by deficient or dysfunctional C1INH, a protein in the blood that helps to control inflammation. Inadequate amounts of properly functioning C1INH can lead to the accumulation of fluid in body tissues.

- Andembry is a first-in-class monoclonal antibody inhibiting factor XIIa (FXIIa). FXII is the first protein activated in the HAE pathway, initiating the cascade of events leading to an HAE attack.

- The efficacy of Andembry was established in VANGUARD, a randomized, double-blind, placebo-controlled study in 64 adult and pediatric patients 12 years of age and older with HAE. Patients were randomized to Andembry or placebo. The primary endpoint was the monthly HAE attack rate at 6 months (number of investigator-confirmed HAE attacks per month).

    — The least squares mean rate of monthly HAE attacks was 0.22 with Andembry and 2.07 for placebo (percent reduction relative to placebo: 89.2, 95% CI: 75.6, 95.2; $p < 0.001$).

- The most common adverse reactions (≥ 7%) with Andembry use were nasopharyngitis and abdominal pain.

- The recommended dosage of Andembry is an initial loading dose of 400 mg (two injections of 200 mg) administered subcutaneously on the first day of treatment followed by a maintenance dosage of 200 mg administered subcutaneously every month.

    — **Andembry is intended for self-administration or administration by a caregiver**. Prior to treatment initiation, patients/caregivers should be trained on proper preparation and subcutaneous administration technique.

- CSL plans to launch Andembry immediately, with availability before the end of June. Andembry will be available as a 200 mg/1.2 mL solution in a single-dose prefilled autoinjector and a 200 mg/1.2 mL solution in a single-dose prefilled syringe with needle safety device.