Takhzyro™ (lanadelumab-flyo) – New orphan drug approval

- On August 23, 2018, the FDA announced the approval of Shire's Takhzyro (lanadelumab-flyo), for prophylaxis to prevent attacks of hereditary angioedema (HAE) in patients 12 years and older.

- HAE is a rare and serious genetic disease that affects an estimated 1 in 50,000 men and women. HAE causes recurrent, unpredictable episodes of severe swelling in different areas of the body, including the stomach, limbs, face and throat. Type I HAE is the most common, and accounts for 85% of cases.

- Lanadelumab-flyo is a fully human monoclonal antibody that binds plasma kallikrein and inhibits its proteolytic activity. Plasma kallikrein is an enzyme which is chronically uncontrolled in people with HAE.

- The efficacy and safety of Takhzyro were demonstrated in a 26-week placebo-controlled study of 125 patients with type 1 or 2 HAE. The primary endpoint was the reduction in mean HAE attack rates vs. placebo.

    — Patients who received Takhzyro had clinically meaningful and statistically significant reductions in the mean rate of monthly HAE attacks vs. placebo. Mean rate of monthly HAE attacks: Takhzyro 150 mg every 4 weeks: 0.48; 300 mg every 4 weeks: 0.53; 300 mg every 2 weeks: 0.26 vs. 1.97 with placebo ($p < 0.001$ for all Takhzyro groups vs. placebo).

- A warning and precaution of Takhzyro includes hypersensitivity reactions.

- The most common adverse reactions with Takhzyro use were injection site reactions, upper respiratory infections, headache, rash, myalgia, dizziness, and diarrhea.

- The recommended starting dose of Takhzyro is 300 mg administered subcutaneously every 2 weeks.

    — A dosing interval of 300 mg every 4 weeks is also effective and may be considered if the patient is well-controlled (eg, attack free) for more than 6 months.
    — Takhzyro is intended for self-administration or administration by a caregiver. The patient or caregiver should be trained by a healthcare professional.

- Shire has launched Takhzyro. Takhzyro is available as a 300 mg/2 mL solution in a single-dose vial.