Nexviazyme® (avalglucosidase alfa-ngpt) – New orphan drug approval

- On August 6, 2021, the FDA announced the approval of Sanofi's Nexviazyme (avalglucosidase alfa-ngpt), for the treatment of patients 1 year of age and older with late-onset Pompe disease (LOPD) [lysosomal acid alpha-glucosidase (GAA) deficiency].

- Patients with Pompe disease have an enzyme deficiency that leads to the accumulation of glycogen in skeletal and heart muscles which cause muscle weakness and premature death from respiratory or heart failure.

    — Pompe disease affects an estimated 3,500 people in the U.S.

- Nexviazyme provides an exogenous source of GAA.

- The efficacy and safety of Nexviazyme were demonstrated in a randomized, double-blinded study of 100 treatment-naïve patients with LOPD. Patients were treated with Nexviazyme or alglucosidase alfa for 49 weeks. The primary endpoint was the change in forced vital capacity (FVC) (% predicted) in the upright position from baseline to week 49.

    — At Week 49, the least squares (LS) mean change in FVC (% predicted) for patients treated with Nexviazyme and alglucosidase alfa was 2.9% and 0.5%, respectively (treatment difference 2.4%; 95% CI: -0.1, 5.0). Noninferiority margin of 1.1% (p = 0.0074). Statistical superiority of Nexviazyne over alglucosidase alfa was not achieved (p = 0.06).
    — In addition, at Week 49, the LS mean change from baseline in 6-minute walk test for patients treated with Nexviazyme and alglucosidase alfa was 32.2 meters and 2.2 meters, respectively (treatment difference; 95% CI: 1.3, 58.7; p = 0.04).

- Nexviazyme carries a boxed warning for severe hypersensitivity reactions, infusion-associated reactions, and risk of acute cardiorespiratory failure in susceptible patients.

- The most common adverse reactions (> 5%) with Nexviazyme use were headache, fatigue, diarrhea, nausea, arthralgia, dizziness, myalgia, pruritus, vomiting, dyspnea, erythema, paresthesia and urticaria.

- The recommended dose of Nexviazyme is given by intravenous infusion every two weeks and based on weight:

    — ≥ 30 kg, the dosage is 20 mg/kg (of actual body weight)
    — < 30 kg, the dosage is 40 mg/kg (of actual body weight)
    — Prior to Nexviazyme administration, consider pretreating with antihistamines, antipyretics, and/or corticosteroids.

- Nexviagyme will be priced the same as alglucosidase alfa (Lumizyme®). Lumizyme is estimated to cost ~$600,000/year.

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- Sanofi plans to launch Nexviazyme in the coming weeks. Nexviazyme will be available as a 100 mg of lyophilized powder in a single-dose vial for reconstitution.

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