Fabrazyme® (agalsidase beta) – Expanded indication

- On March 12, 2021, Sanofi announced the FDA approval of Fabrazyme (agalsidase beta), for the treatment of adult and pediatric patients 2 years of age and older with confirmed Fabry disease.
    - Fabrazyme was previously approved for use in patients with Fabry disease and the safety and effectiveness had not been established in pediatric patients less than 8 years of age.
- In an analysis of 24 Fabrazyme-treated pediatric patients aged 2 to < 8 years, and with elevated plasma globotriaosylceramide (GL-3) levels (ie, > 7.03 µg/mL) at baseline, plasma GL-3 levels fell within the normal range in 91% (20/22), 95% (18/19), and 92% (12/13) of patients at 6, 12, and 24 months, respectively.
- In addition to the expanded pediatric indication, results from a real-world, observational study and a clinical trial on long-term treatment with Fabrazyme are now included in the label. The observational study assessed the rate of decline in renal function in 122 Fabrazyme treated patients (16 years or older) matched with a historical cohort of 122 untreated patients matched based on age, sex, classic or non-classic Fabry disease subtype, and baseline estimated glomerular filtration rate (eGFR).
    - The estimated mean slope of eGFR was -1.5 mL/min/1.73 m^2/year in the Fabrazyme-treated group and -3.2 mL/min/1.73m^2/year in the untreated group with a treatment difference of 1.7 mL/min/1.73m^2/year (95% CI: 0.5, 3.0).
- The recommended dosage of Fabrazyme is 1 mg/kg body weight infused every two weeks as an intravenous infusion.
    - Because of the potential for severe infusion-associated reactions, appropriate medical support measures should be readily available when Fabrazyme is administered.
    - Antipyretics should be administered prior to infusion of Fabrazyme.