Enzyme replacement therapy approved for late-onset Pompe disease

August 11 2021

(HealthDay)—Nexviazyme (avalglucosidase alfa-ngpt) was approved for

the treatment of the rare inherited disorder late-onset Pompe disease in patients 1 year of age and older, the U.S. Food and Drug Administration announced Friday.

The enzyme replacement therapy is delivered intravenously and specifically targets the M6P receptor, the key pathway for enzyme replacement therapy, to help reduce glycogen accumulation.

The approval was based on phase 3 efficacy data from 100 patients randomly assigned to Nexviazyme or another FDA-approved enzyme replacement therapy. Patients taking Nexviazyme had improvements in respiratory function and walking distance measures. Compared with baseline, these patients had a 2.9-point improvement in forced vital capacity (FVC) percent-predicted at week 49 and a 2.4-point greater improvement in FVC percent-predicted compared with patients who received alglucosidase alfa at week 49. This finding met the measurement of noninferiority. For the secondary end point of functional endurance based on the 6-minute walk test, researchers found that patients treated with Nexviazyme walked 32.2 m farther at week 49 and 30 m farther than patients who received alglucosidase alfa.

The most commonly reported side effects with Nexviazyme included headache, fatigue, diarrhea, nausea, arthralgia, dizziness, myalgia, pruritis, vomiting, dyspnea, erythema, paresthesia, and urticaria. Serious reactions were also reported, including hypersensitivity and infusion-associated reactions. The FDA notes that patients susceptible to fluid volume overload or with compromised cardiac or respiratory function may have a risk for serious acute cardiorespiratory failure with Nexviazyme.

Approval was granted to Genzyme Corporation.

More information: More Information