Efficacy and safety results of the avalglucosidase alfa phase 3 COMET trial in participants with late-onset Pompe disease (LOPD)


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Background: Phase 3 COMET trial (NCT02782741) compares avalglucosidase alfa (n=51) with alglucosidase alfa (n=49) in treatment-naïve LOPD. Methods: Primary objective: determine avalglucosidase alfa effect on respiratory muscle function. Secondary/other objectives include: avalglucosidase alfa effect on functional endurance, inspiratory/expiratory muscle strength, lower/upper extremity muscle strength, motor function, health-related quality of life, safety. Results: At Week 49, change (LSmean±SE) from baseline in upright forced vital capacity %predicted was greater with avalglucosidase alfa (2.89%±0.88%) versus alglucosidase alfa (0.46%±0.93%) (absolute difference+2.43%). The primary objective, achieving statistical non-inferiority (p=0.0074), was met. Superiority testing was borderline significant (p=0.0626). Week 49 change from baseline in 6-minute walk test was 30.01-meters greater for avalglucosidase alfa (32.21±0.93m) versus alglucosidase alfa (2.19±10.40m). Positive results for avalglucosidase alfa were seen for all secondary/other efficacy endpoints. Treatment-emergent adverse events (AEs) occurred in 86.3% of avalglucosidase alfa-treated and 91.8% of alglucosidase alfa-treated participants. Five participants withdrew, 4 for AEs, all on alglucosidase alfa. Serious AEs occurred in 8 avalglucosidase alfa-treated and 12 alglucosidase alfa-treated participants. IgG antidrug antibody responses were similar in both. High titers and neutralizing antibodies were more common for alglucosidase alfa. Conclusions: Results demonstrate improvements in clinically meaningful outcome measures and a more favorable safety profile with avalglucosidase alfa versus alglucosidase alfa. Funding: Sanofi Genzyme

Efficacy and Safety of Eptinezumab Initiated During a Migraine Attack: Results from the RELIEF Study


Background: Eptinezumab is approved for migraine prevention, with demonstrated rapid onset of preventive benefit. RELIEF evaluated the efficacy and safety of eptinezumab initiated