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

doi: 10.1017/cjn.2021.307

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±9.93m) 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

Erenumab associated with high persistence among Canadian patients for preventive treatment of chronic and episodic migraine in real-world practice

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doi: 10.1017/cjn.2021.308

Background: Real world use of oral prophylactic migraine therapies is often limited by poor patient persistence. The objective of this study was to describe the demographics and clinical characteristics of patients prescribed erenumab following its launch in Canada (September 2018) and to evaluate the real-world treatment persistence and dose management. Methods: This was a retrospective, descriptive analysis of de-identified secondary patient data that includes baseline demographics, clinical characteristics, plus erenumab treatment management, collected through Novartis’ Go Program® (Patient Support Program). Only data collected from patients with a documented informed consent were included in the analysis. Results: 14,282 patients met eligibility criteria. The mean age of patients was 46.3 years, 83.0% were female, and 66.1% reported having ≥15 monthly migraine days. 52.5% were initiated on the 140 mg dose of erenumab and 59.3% of those who initiated the 70 mg dose escalated to 140 mg within 360 days. After 360 and 450 days, the KM-derived persistence was 71.0% and 63.4%, respectively. Conclusions: The high persistence reported here suggests that erenumab has a meaningful degree of tolerability in the real-world setting and increases confidence that the real-world use and benefits of erenumab will not be undermined by the poor persistence observed with traditional migraine prophylactic agents.

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

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doi: 10.1017/cjn.2021.309

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