P.025

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 ±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

HEADACHE

P.026

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

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Background: Real world use of oral prophylactic migraine therapies is often limited by poor patient tolerance. 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.

P.027

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

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

during a migraine attack. **Methods:** RELIEF (NCT04152083; parallel-group, double-blind, placebo-controlled) randomized adults with migraine (4-15d/mo in 3mo prior to screening) to eptinezumab 100mg or placebo, administered IV within 1-6h of qualifying migraine onset. Co-primary efficacy endpoints were time to headache pain freedom and time to absence of most bothersome symptom (MBS). **Results:** Eptinezumab (n=238) compared with placebo (n=242) achieved significantly faster headache pain freedom (median 4h vs 9h; hazard ratio=1.54, P=0.0006) and absence of MBS (2h vs 3h; 1.75, P<0.0001). At 2h, 23.5% and 12.0% (P=0.0009) of eptinezumab-treated and placebo patients, respectively, reported headache pain freedom, and 55.5% and 35.8% (P<0.0001) reported absence of MBS. Significantly fewer eptinezumab-treated patients used rescue medication within 24h (31.5% vs 59.9%; P<0.0001). Treatment-emergent adverse events occurred in 10.9% eptinezumab-treated and 10.3% placebo patients; no serious adverse events occurred. **Conclusions:** Infusion of the preventive migraine treatment, eptinezumab, during a migraine resulted in rapid and sustained freedom from headache pain and MBS vs placebo, starting 2h post-infusion, decreasing need for acute medication within 24h post-infusion. No notable safety findings were identified.

P.028

Surgical Treatment for Idiopathic Intracranial Hypertension – Strategy for the Better Management

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Background: Idiopathic intracranial hypertension (IIH) is a condition of increased intracranial pressure in the absence of a space-occupying lesion. The goal of this study is to investigate which factors may influence outcomes in order to improve surgical strategy. We hypothesized diabetes, hypertension, smoking, and obesity influence patients prognosis. **Methods:** This retrospective chart review included patients diagnosed with IIH who underwent surgical intervention. All patients receiving surgery between 2008 and 2018 were included, and divided into 2 cohorts. Cohort 1 representing favorable course and cohort 2 representing unfavorable course. Favorable course was defined as requiring single surgery for management. Unfavorable course required multiple surgical revisions. **Results:** Overall, 35/48 (73%) comprised the favorable group. Thirteen patients (27%) comprised the unfavorable group. Of the unfavorable group, 54% had LP shunts, with the remaining receiving VP shunts. There was no association between type of shunt and outcome. Common issues the unfavorable group encountered were persisting symptoms, infections, obstruction of shunt and replacement of shunt. Smoking and frequent follow-up were associated with unfavorable course. Gender, BMI, age, comorbidities and shunt type were not associated with outcome. **Conclusions:** We found smoking and patient follow-up had a significant association with unfavorable outcome. Other factors had no association with patient outcome.

P.029

Oral Daily Atogepant for the Preventive Treatment of Migraine Increases Responder Rates for Reduction in Mean Monthly Migraine Days

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Background: The goal of the study was to assess responder rates at various times after initiating atogepant treatment. **Methods:** A 12-week phase 3 trial evaluated the safety, efficacy, and tolerability of atogepant for preventive treatment of migraine (ADVANCE; NCT03777059) in adult participants with a ≥1-year history of migraine, experiencing 4-14 migraine days/month. Participants were randomized to atogepant 10, 30, or 60mg, or placebo once daily. These analyses evaluated ≥25%, ≥50%, ≥75%, and 100% reductions in mean monthly migraine days (MMDs) across 12 weeks and each 4-week interval. Adverse events (AEs) in ≥5% of participants are reported. **Results:** The efficacy analysis population included 873 participants: placebo: n=214; atogepant: 10mg: n=214; 30mg: n=223; 60mg: n=222. Atogepant-treated participants were more likely to experience a ≥50% reduction in the 3-month mean MMDs (56-61% vs 29% with placebo; P<0.0001). The proportions of participants experiencing ≥25%, ≥50%, ≥75%, and 100% reductions in mean MMDs significantly increased during each 4-week interval (≥50% reduction: 48-71% vs 27-47% with placebo). The most common AEs for atogepant were constipation (6.9-7.7%) and nausea (4.4-6.1%). **Conclusions:** Once-daily atogepant 10, 30, and 60mg significantly increased responder rates at all thresholds with approximately 60% achieving a ≥50% reduction in mean MMDs at 12 weeks.

P.030

Long-term Safety and Tolerability of Atogepant 60 mg Following Once-Daily Dosing Over 1 Year for the Preventive Treatment of Migraine

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Background: The goal of the study was to assess the safety and tolerability of atogepant, an oral, calcitonin gene-related peptide receptor antagonist in development for migraine preventive treatment, once daily over 1 year. **Methods:** Multicenter, open-label trial (NCT03700320). Adults with migraine were randomized 5:2 to atogepant or oral standard-of-care (SOC) migraine prevention. **Results:** 744 randomized participants (n=546 atogepant), 739 safety population participants (n=543 atogepant). Adverse events (AEs) were reported by 67.0% of atogepant participants; 18.0% had AEs