2 (2.90%) patients belong in each aforementioned category. No patients from either group were hospitalized for MG after SFMEG. Conclusions: Preliminary results demonstrate no difference in frequency of poor outcomes between patients who had 20 or more pairs observed and those who had 12 pairs observed, supporting the safety of shortening the test in appropriate situations.

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Post-hoc evaluation of the clinical effects of nipocalimab, a neonatal fragment crystallizable blocker, over time in the Vivacity MG3 study

*R Nowak (New Haven) M Ait-Tihyaty (Titusville) I Turkoz (Titusville) K Gandhi (Titusville) R Rodriguez (Titusville) S Pease (Titusville) S Gingerich (Toronto) H Katzberg (Toronto)**

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Background: In generalized myasthenia gravis (gMG), there remains an unmet need for treatments providing meaningful symptom control. Methods: Mean changes in MG-ADL were compared between nipocalimab + standard-of-care (SoC) and placebo+SoC. The proportion of patients achieving: Minimal Symptom Expression (MSE), MG-ADL score 0/1, time with MSE, sustained within person meaningful change (WPMC) starting from Week 4, and time spent with WPMC were compared. Results: Nipocalimab+SoC demonstrated significant improvement in MG-ADL compared to placebo+SOC, LS-mean-change[SE] -4.7[0.329] vs -3.25[0.335]; Difference in means [SE]=-1.45 [0.470], p=0.002. The mean difference favoured nipocalimab+SoC, and was significant as early as week 1: LS-mean-change[SE]: -2.72[2.979] vs -1.77[2.426]; Difference in means[SE] -0.82[0.410], p=0.046. Nipocalimab+SoC patients were three times more likely to achieve MSE at any point during the study vs placebo; Odds Ratio[95% CI]: 3.0[1.3, 6.8]; 31.2% vs. 13.2%. For the 25 patients reaching MSE, the time sustaining MSE [percent time with MSE] was 101.5 days, (60.4%, nipocalimab+SOC) vs 55 days, (32.7%, placebo+SOC). Similarly, the proportion of patients with sustained WPMC favored nipocalimab+SOC, 55.8% vs 26.3%, placebo+SOC, p<0.001. The median percent time spent with WPMC was 84.5%, nipocalimab+SOC vs 39.9%, placebo+SOC, p=0.007. Conclusions: Based on MG-ADL data from Phase 3, nipocalimab an FcRn blocker, demonstrated rapid, substantial, and sustained symptom control.

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A randomized, open-label study on the effect of Nipocalimab on vaccine responses in healthy participants

M Cossu (Leiden) C Bobadilla Mendez (Spring House) A Jackson (La Jolla) E Myshkin (Cambridge) G Liu (Raritan) E Lam (Spring House) U Beier (Spring House) K Weisel (Spring House) B Scott (Spring House) M Ait-Tihyaty (Spring House) B Sattin (Toronto) J Leu (Spring House) S Gao (Spring House) D Dimitrova (Spring House)*

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Background: Nipocalimab is a human IgG1 monoclonal antibody targeting FcRn that selectively reduces IgG levels without impacting antigen presentation, T- and B-cell functions. This study describes the effect of nipocalimab on vaccine response. Methods: Open-label, parallel, interventional study randomized participants 1:1 to receive intravenous 30mg/kg nipocalimab at Week0 and 15mg/kg at Week2 and Week4 (active) or no drug (control). On Day 3, participants received Tdap and PPSV®23 vaccinations and were followed through Wk16. Results: Twenty-nine participants completed the study and are included (active, n=15; control, n=14). Participants with a positive anti-tetanus IgG response was comparable between groups at Wk2 and Wk16, but lower at Wk4 (nipocalimab 3/15 [20%] vs control 7/14 [50%]; *P*=0.089). All maintained anti-tetanus IgG above the protective threshold (0.16IU/mL) through Wk16. While anti-pneumococcal-capsular-polysaccharide (PCP) IgG levels were lower during nipocalimab treatment, the percent increase from baseline at Wk2 and Wk16 was comparable between groups. Post-vaccination, anti-PCP IgG remained above 50mg/L and showed a 2-fold increase from baseline throughout the study in both groups. Nipocalimab co-administration with vaccines was safe and well-tolerated. Conclusions: These findings suggest that nipocalimab does not impact the development of an adequate IgG response to T-cell–dependent/independent vaccines and that nipocalimab-treated patients can follow recommended vaccination schedules.

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Miglustat: a first-in-class enzyme stabiliser for late-onset Pompe Disease

B Fox (Princeton) T Mozaffar (Irvine) M Roberts (Salford) BJ Byrne (Gainesville) MM Dimachkie (Kansas City) RJ Hopkin (Cincinnati) PS Kishnani (Durham) B Schoser (Munich) AT van der Ploeg (Rotterdam) J Brudvig (Princeton) F Holdbrook (Princeton) V Jain (Princeton) F Johnson (Princeton) J Zhang (Princeton) G Parenti (Naples)*

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Background: Late-onset Pompe disease (LOPD) is caused by a deficiency of acid ⍰-glucosidase (GAA), leading to progressive muscle and respiratory decline. Cipaglucosidase alfa (cipa), a recombinant human GAA naturally enriched with bis-mannose-6-phosphate, exhibits improved muscle uptake but is limited by inactivation at near-neutral blood pH. Miglustat (mig), an enzyme stabiliser, binds competitively and reversibly to cipa, enhancing its stability and activity. Methods: In dose-finding studies, *Gaa*-/- mice were treated with cipa (20 mg/kg) +/- mig (10 mg/kg; equivalent human dose ~260 mg). Clinical study methodologies have been published (Schoser *et al. Lancet Neurol* 2021:20;1027–37; Schoser *et al. J Neurol* 2024:271;2810–23). Results: In *Gaa*-/- mice, cipa+mig improved muscle glycogen reduction more than cipa alone and grip strength to levels approaching wild-type mice. LOPD patients (n=11) treated with cipa alone showed dose-dependent decreases in hexose tetrasaccharide (Hex4) levels by ~15% from baseline, decreasing another ~10% with added mig (260 mg). In a head-to-head study, cipa+mig had a similar safety profile to alglucosidase alfa. Among 151 patients (three trials), mig-related adverse events occurred in 21 (13.9%), none serious. Conclusions: Mig stabilised cipa in circulation, improving cipa exposure, further