Safety and Efficacy Results of Nipocalimab in Adolescents with Generalized Myasthenia Gravis During Active-Treatment and Long-Term Extension Phases: vibrance-mg Phase 2/3 Study

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Introduction

Nipocalimab is a fully human Immunoglobulin G1 (IgG1) monoclonal antibody that binds to neonatal Fc receptor (FcRn) with high specificity and affinity blocking its interaction with IgG (**Figure 1**).[1,2]

In the pivotal phase 3 Vivacity-MG3 study involving adults with generalized myasthenia gravis (gMG), nipocalimab treatment lowered levels of circulating IgG and pathogenic IgG autoantibodies.[3]

Patients receiving nipocalimab also demonstrated symptom improvement sustained over 24 weeks[3] and up to 60 weeks in the open label extension.[4]

These results recently supported the United States Food and Drug Administration approval of nipocalimab for the treatment of both adult and adolescent patients (≥12 years) with gMG.[5]

Objective

- **To evaluate the effect of nipocalimab on pharmacodynamics (IgG), safety and efficacy in adolescents aged 12 to <18 years with gMG who exhibit an insufficient clinical response to standard-of-care (SOC) therapy.**
- **Here, we have summarized the study results in through a clinical cut-off of August 23, 2024.**

Methods

- Vibrance-mg is a global, multicenter, open-label phase 2/3 study evaluating nipocalimab + SOC in adolescents (Cohort 1; **Figure 2**) and children with gMG.
- Cohort 1 participants received an initial loading dose of nipocalimab 30 mg/kg intravenously (IV), followed by 15 mg/kg IV every 2 weeks (Q2W). During the Long-Term Extension (LTE), dosing could be adjusted at the investigator's discretion to either 15 mg/kg Q2W or 30 mg/kg every 4 weeks (Q4W).
- Primary endpoints included the effect of nipocalimab on total serum IgG levels, along with assessments of safety and tolerability. Secondary endpoints evaluated treatment response through changes in Quantitative Myasthenia Gravis (QMG) and Myasthenia Gravis Activities of Daily Living (MG-ADL) scores.
- Results for Cohort 1 (adolescents) are reported from the AT phase (Day 1 to Week 24) expanding on previously reported data from 7 patients to now include 8 patients. Efficacy data through the LTE (up to Week 72) and safety data until data cut-off are presented.

Study Endpoints

Primary Endpoint

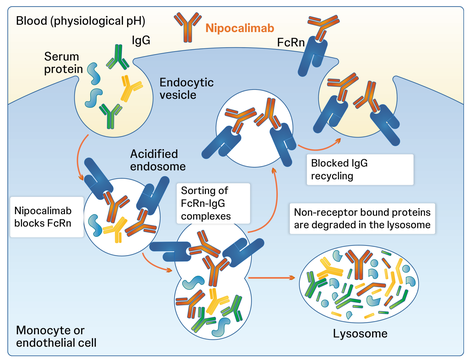
- The effect of nipocalimab on total serum IgG
- Safety and tolerability

Secondary Endpoint

The effect of nipocalimab on:
- MG-ADL Score
- QMG Score

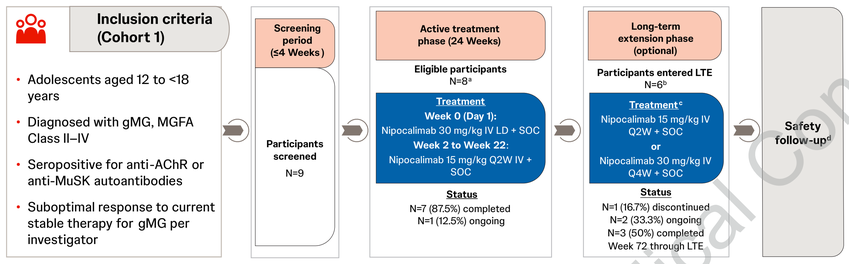
- Results are presented from an analysis of adolescent participants in the ongoing study

Figure 1: Nipocalimab's mechanism of action



FcRn=neonatal Fc receptor, **gMG**=generalized myasthenia gravis, **IgG**=immunoglobulin. This figure was previously presented at the MGFA Scientific Session of the AANEM Annual Meeting and AANEM Annual Meeting; Savanah, Georgia, USA; October 15–18, 2024.

Figure 2: The vibrance-mg study design



[a]One participant failed during the Screening phase. [b]Out of 7 participants who completed the AT phase, 6 entered the LTE phase. [c]Four participants switched from Q2W to Q4W at various times and were ongoing in LTE at data cutoff. [d]Participants who withdraw or discontinue after receiving any amount of study intervention will be required to complete a safety follow-up visit 8 weeks after their last dose. **AChR**=acetylcholine receptor, **AT**=active treatment, **gMG**=generalized myasthenia gravis, **IV**=Intravenous, **LD**=loading dose, **LTE**=long-term extension, **MGFA**=Myasthenia Gravis Foundation of America, **MuSK**=muscle-specific kinase, **Q2W**=every 2 weeks, **Q4W**=every 4 weeks, **SOC**=standard-of-care. This figure has been adapted from the figure previously presented at the MGFA Scientific Session of the AANEM Annual Meeting and AANEM Annual Meeting; Savanah, Georgia, USA; October 15–18, 2024.

Key Takeaways

- **In adolescents with gMG (12 to <18 years of age), nipocalimab demonstrated a rapid, substantial and sustained reduction in total serum IgG levels over 24 weeks, with effects maintained through Week 72 in LTE.**
- **Clinically meaningful improvements in MG-ADL and QMG scores were achieved over 24 weeks of the AT and sustained in most participants through Week 72 in LTE.**
- **Treatment was well-tolerated in adolescents with gMG, with a favorable safety profile observed throughout the study.**
- **Vibrance-mg is the first study to evaluate an FcRn blocker in adolescents with gMG, and nipocalimab is now the first FcRn blocker approved for treating both adult and adolescent patients with gMG, addressing the unmet needs across this broad population.**

Results

Demographics and Baseline Characteristics

Table 1: Demographics and AT phase baseline characteristics of adolescent participants in cohort 1

Characteristics	Cohort 1 (N=8)
Age, years	13.5 (12–16)
Sex, Female, n (%)	7 (87.5)
Race, n (%)	
Asian	5 (62.5)
Black or African American	1 (12.5)
Unknown	2 (25.0)
Ethnicity, n (%)	
Hispanic or Latino	1 (12.5)
Not Hispanic or Latino	6 (75.0)
Unknown	1 (12.5)
Weight, kg	43.1 (30.9–95.5)
BMI, kg/m²	18.5 (15.9–37.2)
Duration of MG in years	3.6 (0.8–11.5)

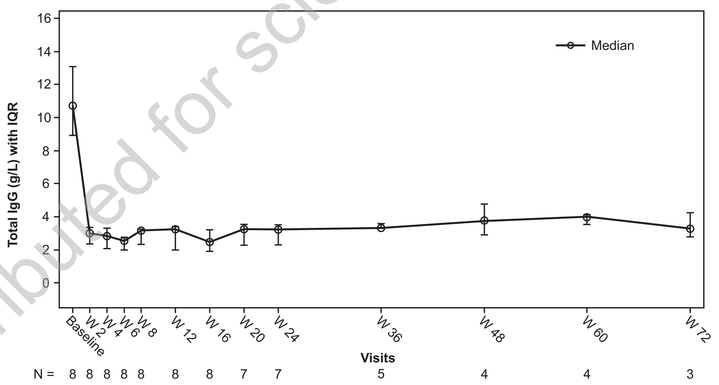
Characteristics	Cohort 1 (N=8)
Baseline MG-ADL total score, median (IQR)	3.5 (3.0–5.0)
Autoantibody type, Anti-AChR+, n (%)	8 (100.0)
Baseline QMG total score, median (IQR)	14.3 (10.5–15.8)
Age at onset of MG in years	10.5 (0.5–13.4)
Baseline MGFA Clinical Classification, n (%)	
IIa	4 (50.0)
IIIa	3 (37.5)
IIIb	1 (12.5)
Participants with ≥1 concomitant MG medications, n (%)	8 (100.0)
Immunosuppressants	6 (85.7)
Corticosteroids for systemic use	5 (71.4)
Other nervous system drugs[a]	3 (42.9)

Data shown are median (range) unless otherwise indicated. The IQR represents the first quartile and third quartile of the data at each timepoint. [a]Includes AChEIs of pyridostigmine and pyridostigmine bromide. **AChEIs**=acetylcholinesterase inhibitors, **AChR**=acetylcholine receptor, **AT**=active treatment, **BMI**=body mass index, **IQR**=interquartile range, **MG**=myasthenia gravis, **MG-ADL**=Myasthenia Gravis Activities of Daily Living, **QMG**=Quantitative Myasthenia Gravis, **MGFA**=Myasthenia Gravis Foundation of America, **SD**=standard deviation, **SE**=standard error.

Primary Efficacy Endpoint: Total Serum IgG

- Nipocalimab treatment resulted in a rapid and sustained IgG reduction in adolescent participants with gMG (**Figure 3**):
- Median (Interquartile range [IQR]) serum IgG in g/L was 10.7 (8.9; 13.1) at baseline and **3.2 (2.3; 3.5) at Week 24.**
  - Effect was sustained in the LTE to 3.3 (2.8; 4.3) at Week 72.
- Median (IQR) **percent change from baseline** to Week 2 was −72.6% (−79.1; −70.1) and to **Week 24 was −73.3% (−78.7; −62.8)**.
  - Effect was generally sustained in the LTE to −60.6% (−72.0; −48.8) at Week 72.

Figure 3: Median (IQR) total serum IgG levels over time

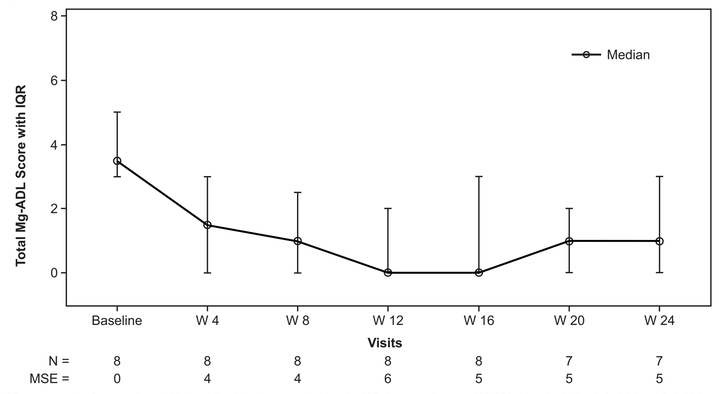


The IQR represents the first quartile and third quartile of the data at each timepoint. Total IgG (g/L) values are presented up to the last visit at which IgG values were available for at least 3 patients. **IgG**=immunoglobulin, **IQR**=interquartile range, **W**=week.

Secondary Efficacy Endpoint: MG-ADL Score

- A clinically meaningful reduction in MG-ADL score was observed by Week 4 and was maintained through both AT (**Figure 4**) and LTE phases.
  - Baseline: Median (IQR) score of 3.5 (3.0; 5.0).
  - Week 4: Reduced to 1.5 (0.0; 3.0).
  - Week 24: Maintained at 1.0 (0.0; 3.0).
- 2 out of 3 patients who completed the LTE up to Week 72 maintained symptom improvement; one patient had worsening MG-ADL scores without any documented adverse event (AE) of MG worsening at Week 72.
- Over **half of participants had Minimal Symptom Expression (MG-ADL score of 0 or 1) at each time point** during AT and/or LTE.

Figure 4: Median (IQR) total MG-ADL score over time

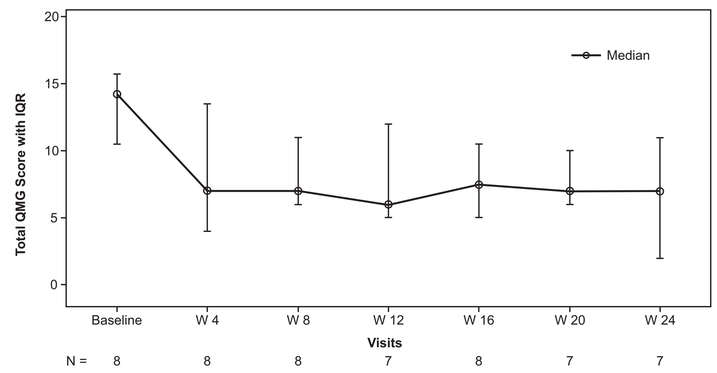


The IQR represents the first quartile and third quartile of the data at each timepoint. **IQR**=interquartile range, **MG-ADL**=Myasthenia Gravis Activities of Daily Living, **MSE**=Minimal Symptom Expression **W**=week.

Secondary Efficacy Endpoint: QMG Score

- A clinically meaningful reduction in QMG score was observed by Week 4 and maintained through both AT (**Figure 5**) and LTE phases.
  - Baseline: Median (IQR) score of 14.3 (10.5; 15.8).
  - Week 4: Reduced to 7.0 (4.0; 13.5).
  - Week 24: Maintained at 7.0 (2.0; 11.0).
- 2 out of 3 patients completing the LTE up to Week 72 maintained symptom improvement; one patient had worsening QMG scores without any documented AE of MG worsening at Week 72.

Figure 5: Median (IQR) total QMG score over time



The IQR represents the first quartile and third quartile of the data at each timepoint. **IQR**=interquartile range, **QMG**=Quantitative Myasthenia Gravis, **W**=week.

Primary Safety Endpoint: Overall Safety

- Nipocalimab was generally well-tolerated across both the AT and LTE phases.
- No serious adverse events (SAE) or AEs leading to discontinuation were reported in the AT phase (**Table 2**).
- One patient experienced an SAE (gMG worsening) after Week 72 in LTE (at Week 84).
- Another participant had an AE (influenza) (~Week 30) that led to temporary treatment discontinuation.
- No AEs of special interest were reported during the study.

Table 2: Summary of TEAEs in AT and LTE phases.

	AT phase (n=8)	LTE phase (n=6)
Average duration of follow-up, in weeks (SD)	24.2 (3.5)	44.3 (29.3)
Patients with ≥1 TEAEs	8 (100.0)	4 (66.6)
Related TEAEs	3 (37.5)	1 (16.7)
Patients with TEAEs leading to death	0	0
Patients with SAEs	0	1 (16.7)[a]
TEAEs leading to temporary discontinuation of study treatment[b]	0	1 (16.7)[c]
AEs leading to termination of study participation	0	0
COVID-19 associated TEAEs[d]	2 (25.0)	0
AESI[e]	0	0

Data are presented as n (%) unless otherwise indicated. TEAE overview was presented beyond Week 72. The AT phase was conducted over 24 weeks, followed by a 48-week LTE phase. [a]Worsened myasthenia gravis. [b]No TEAEs led to permanent discontinuation of study treatment during the AT and LTE phase. [c]Influenza. [d]No SAEs related to COVID-19 were reported during the AT and LTE phase [e]Defined as severe infections requiring systemic treatment or intervention, hypoalbuminemia (albumin <20 g/L), and opportunistic infections. **AESI**=adverse event of special interest, **AT**=active treatment, **COVID-19**=coronavirus disease 2019, **LTE**=long-term extension, **SAEs**=serious adverse events, **SD**=standard deviation, **TEAEs**=treatment-emergent adverse events.

Primary Safety Endpoint: Adverse Events

- Nasopharyngitis (37.5%) and COVID-19 (25.0%) were most common TEAEs (**Table 3**) in AT phase (median follow-up: 24.0 [18–31]).
- Influenza (33.3%) and nasopharyngitis (33.3%) were most common in LTE phase (median follow-up: 44.5 [range: 12–79]).

Table 3: Number of patients with TEAEs in AT and LTE phases

	AT phase (n=8)	LTE phase (n=6)
Participants with ≥1 TEAEs	8 (100.0)	4 (66.7)
Nasopharyngitis	3 (37.5)	2 (33.3)
COVID-19	2 (25.0)	0
Upper respiratory tract infection	1 (12.5)	1 (16.7)
Headache	1 (12.5)	0
Migraine	1 (12.5)	1 (16.7)
Somnolence	1 (12.5)	0
Abdominal pain upper	1 (12.5)	0
Diarrhea	1 (12.5)	1 (16.7)
Glossitis	1 (12.5)	0
Anemia	1 (12.5)	0
Face edema	1 (12.5)	0
Blood cholesterol increased	1 (12.5)	0
Hypercholesterolemia	1 (12.5)	0
Muscle spasms	1 (12.5)	1 (16.7)
Bacterial vaginosis	1 (12.5)	0
Influenza	1 (12.5)	2 (33.3)

	AT phase (n=8)	LTE phase (n=6)
Participants with ≥1 TEAEs	8 (100.0)	4 (66.7)
Nausea	1 (12.5)	0
Stomatitis	1 (12.5)[a]	0
Vomiting	1 (12.5)	0
Fatigue	1 (12.5)	1 (16.7)
Blood pressure increased	1 (12.5)	0
White blood cell count increased	1 (12.5)	0
Seasonal allergy	1 (12.5)	0
Hordeolum	0	1 (16.7)
Tinea versicolour	0	1 (16.7)
Injection site swelling	0	1 (16.7)
Nasal congestion	0	1 (16.7)
Productive cough	0	1 (16.7)[b]
Rash	0	1 (16.7)
Rash pruritic	0	1 (16.7)
Low density lipoprotein increased	0	1 (16.7)
Worsened myasthenia gravis	0	1 (16.7)[c]

Data are presented as n (%). The AT phase was conducted over 24 weeks, followed by a 48-week LTE phase. [a]Stomatitis, observed in one participant during the AT phase, was of moderate severity. [b]Productive cough, observed in one participant during the LTE phase, was of moderate severity. [c]Worsened myasthenia gravis, observed in one participant during the LTE phase, was of severe intensity. **AE**=adverse event, **AT**=active treatment, **COVID-19**=coronavirus disease 2019, **LTE**=long-term extension, **TEAEs**=treatment-emergent adverse events.

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