A Phase 3 Study to Evaluate the Efficacy and Safety of Telitacicept in Patients With Generalized Myasthenia Gravis: Trial Design Update

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Abstract

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    - Introduction: 953
    - Methods: 507
    - Results: 101
    - Conclusions: 189
- All abbreviations must be written out at first use
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Suggested Abstract Topic

- 19. Muscle and neuromuscular junction disorder

Introduction

Generalized myasthenia gravis (gMG) is an autoimmune neuromuscular disease driven by autoreactive B cells, that include plasmablasts and plasma cells responsible for autoantibody production. B-cell activating factor (BAFF) and a proliferation-inducing ligand (APRIL) are required for B cell development, differentiation, and survival. BAFF is a key player in the pathway to overcome negative selection of autoreactive B cells at immune checkpoints, allowing for the development of pathogenic B cells and autoimmunity. Telitacicept is a fully human, novel TACI-Fc fusion protein that binds soluble BAFF and APRIL, preventing binding to their receptors (TACI, BCMA, BAFF-R) on B cells. Phase 2 (NCT04302103) and 3 (NCT05737160) studies demonstrated efficacy/safety of telitacicept in Chinese adults with acetylcholine receptor autoantibody–positive gMG. Here, we report an update on enrollment in an ongoing global phase 3 study (NCT06456580).

Methods

The global study will enroll approximately ~180 adults with confirmed gMG. Eligibility criteria are detailed in **Figure 1**. The study will comprise a ≤4-week screening period, a 24-week double-blind treatment period in which patients are randomized 1:1 to weekly subcutaneous telitacicept (240 mg) or placebo, and a 48-week open-label extension. The primary endpoint is change from baseline in Myasthenia Gravis-Activities of Daily Living score at week 24. Secondary endpoints are detailed in **Figure 1**.

Results

Study enrollment is ongoing across 11 countries with top-line results anticipated in mid-2027.

Conclusions

Results from this global population of adults with gMG will augment the established efficacy and safety of BAFF/APRIL inhibition with telitacicept in a broader global population.

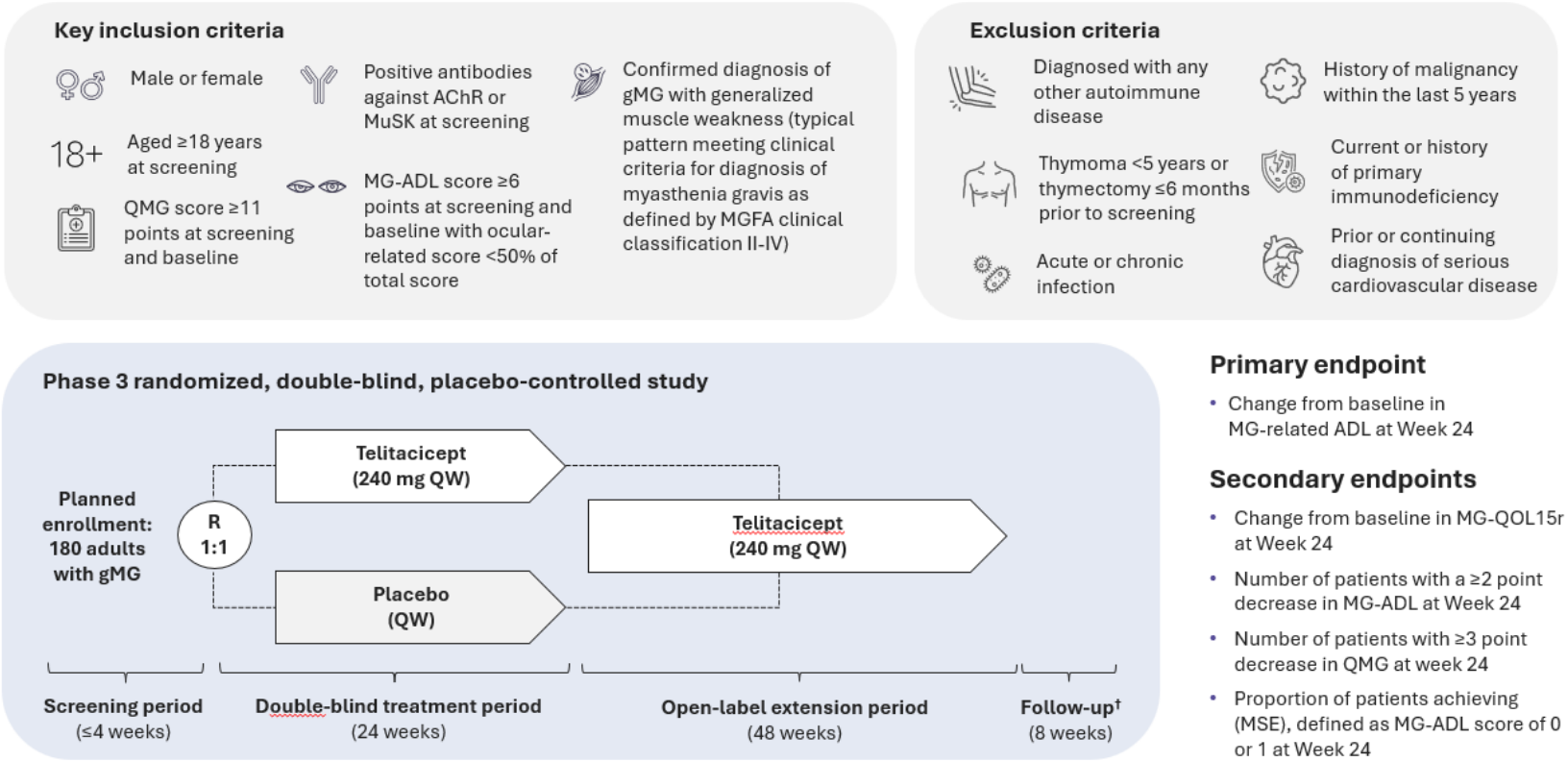
Disclosures:

1. Kristl G. Claeys has received speaker/advisory board honoraria from Alexion, Alnylam, Amicus, ArgenX, Biogen, Ipsen, Janssen Pharmaceutics, Lupin, Pfizer, Roche, Sanofi-Genzyme, UCB, CSL Behring, Alnylam, Pfizer, Sanofi-Genzyme, Biogen, Roche, Vertex.
2. Francesco Saccà has received public speaking honoraria from Alexion, argenx, Biogen, Genpharm, Johnson&Johnson, Medpharma, Medison Pharma, Neopharm Israel, UCB, Zai Lab; he also received compensation for Advisory boards or consultation fees from Alexion, Amgen, argenx, Astrazeneca, Avexis, Biogen, Dianthus, Johnson&Johnson, Lexeo , Novartis GmBh, Reata, UCB, Zai Lab; he is PI in clinical trials for Alexion, argenx, Dianthus, Immunovant, Lediant, Lexeo, Novartis, Prilenia, Remgen, Sanofi.
3. Ali A. Habib has received research support/honoraria from Alexion/AstraZeneca, Amgen, Arcellx, argenx, Cabaletta Bio, Cartesian, COUR Pharmaceuticals, GC Biopharma, Grifols,

Immunovant, Jansen/J&J, Kyverna, Merck, MGNet (grant number: U54NS115054), Nkarta, NMD Pharma, Novartis, Regeneron, and UCB; has served on a Data and Safety Monitoring Board for Genentech/Roche, Immunis Biomedical, and the National Institutes of Health/National Institute of Neurological Disorders and Stroke; and has served on a Trial Steering Committee for Dianthus, Jansen/J&J, and Kyverna.

4. Richard J. Nowak has received research support from the National Institutes of Health, Genentech, Inc., Alexion Pharmaceuticals, Inc., argenx, Annexon Biosciences, Inc., Ra Pharmaceuticals, Inc. (now UCB S.A.), the Myasthenia Gravis Foundation of America, Inc., Momenta Pharmaceuticals, Inc. (now Janssen), Immunovant, Inc., Grifols, S.A., and Viela Bio, Inc. (Horizon Therapeutics, now Amgen Inc.), and has served as a consultant and advisor for Alexion Pharmaceuticals, Inc., argenx, Cabaletta Bio, Inc., Cour Pharmaceuticals, Ra Pharmaceuticals, Inc. (now UCB S.A.), Immunovant, Inc., Momenta Pharmaceuticals, Inc. (now Janssen), Vor Bio, and Viela Bio, Inc. (Horizon Therapeutics, now Amgen Inc.).
5. Mamatha Pasnoor is a University of Kansas site principal investigator for RemeGen MG trial; served as medical advisor or consultant for Alexion, Amgen, Annexon, argenx, BVBA, Catalyst, CSL Behring, Grifols, Immunovant Pharmaceuticals, Jansen, Johnson and Johnson, Momenta, Takeda, and Terumo BCT; serves on the board of directors for the Myasthenia Gravis Association (MGA).
6. Gil I. Wolfe serves as an advisor for Alexion, argenx, BPL, Canopy, Cartesian, Dianthus, Grifols, Johnson & Johnson, Takeda, and UCB and receives research support from Immunovant, Novartis, Roche, and Vor.
7. Tuan Vu is a University of South Florida site principal investigator for MG clinical trials sponsored by Alexion/AstraZeneca Rare Disease, Amgen, argenx, Cartesian Therapeutics, COUR, Dianthus Therapeutics, EMD Serono, ImmunAbs, Immunovant, Johnson & Johnson, NMD Pharma, Novartis, Regeneron, UCB, and Vor. He has served as a speaker for Alexion/AstraZeneca Rare Disease, Amgen, argenx, and CSL Behring and participated on advisory boards for Alexion/AstraZeneca Rare Disease, Amgen, argenx, Dianthus Therapeutics, ImmunAbs, Johnson & Johnson, NMD Pharma, Regeneron, and Vor.
8. Jeremy Sokolove is an employee of Vor Bio and owns company stock.
9. James F. Howard, Jr has received research funding (paid to his institution) from Ad Scientiam, Alexion AstraZeneca Rare Disease, argenx, Cartesian Therapeutics, Centers for Disease Control and Prevention, Merck EMD Serono, MGFA, Muscular Dystrophy Association, NIH, NMD Pharma, and UCB Bioscience, He has served as medical advisor or consultant for Alexion AstraZeneca Rare Disease, Amgen, argenx, Biohaven Ltd, Cartesian Therapeutics, CoreEvitas, Curie.bio, H. Lundbeck A/S, Japan Tobacco Company, Kyverna Therapeutics, Merck EMB Serono, NMD Pharma, Novartis Pharma, Regeneron Pharmaceuticals, Seismic Therapeutics, TG Therapeutics, Toleranzia AB, and UCB Bioscience. He has received speaker fees from AcademicCME, CheckRare CME, PeerView CME, Physicians' Education Resource (PER) CME, PlatformQ CME.

Figure 1. Ongoing Phase 3 Global Study Design and Eligibility



† Estimated. For patients who discontinue treatment before the OLE period, EOT and EOS time points are at 24 and 32 weeks, respectively. For those continuing with the OLE, EOT and EOS time points are at 72 and 80 weeks, respectively.
AChR, acetylcholine receptor; ADL, Activities of Daily Living; EOS, end of study; EOT, end of treatment; gMG, generalized myasthenia gravis; MG-ADL, Myasthenia Gravis-Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MG-QOL15r, Quality Of Life 15-item revised scale; MSE, minimal symptomatic expression; MuSK, muscle-specific kinase; OLE, open-label extension; QMG, Quantitative Myasthenia Gravis; QW, per week; R, randomized.
ClinicalTrials.gov. NCT06456580. Accessed December 9, 2025. https://clinicaltrials.gov/study/ NCT06456580.