Real-World Clinical Outcomes with Efgartigimod and Efgartigimod Hyaluronidase in Patients with Generalized Myasthenia Gravis at an Adult Neuromuscular Clinic: A Single-Center Retrospective Review

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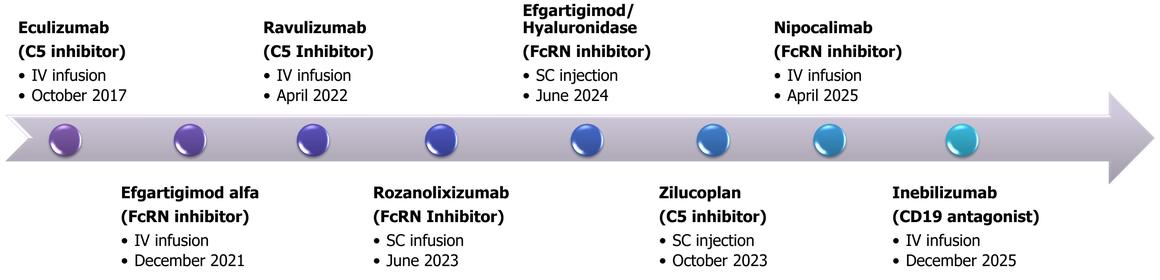
BACKGROUND

- Generalized myasthenia gravis (gMG) is a rare and heterogenous disease affecting an estimated 30 to 40 per 100,000 people in the United States.
- gMG is an archetypal example of a neurological autoimmune disorder in which distinct pathogenic origins results in a common clinical phenotype: fatigable muscle weakness.
- The most recent International Consensus Guideline for the Management of Myasthenia Gravis, published in 2021, reviewed corticosteroids, pyridostigmine, IVIG, plasma exchange, rituximab, and thymectomy as treatment options.
- The therapeutic landscape for gMG changed dramatically since the release of these guidelines. Three novel therapeutic drug classes and seven new distinct drug formulations have emerged as treatment options for gMG in the last ten years, offering rapid symptomatic control, potential for corticosteroid dose reduction or elimination, improvement in patient reported outcomes, and achievement of minimal symptom manifestations.
- The UVA Neurology Clinic Neuromuscular Division is composed of five neurologists, one advanced practice provider, one fellow, two nurse care coordinators, and one pharmacist. This practice follows about 300 gMG patients per year and has prescribed efgartigimod in more than 50 instances.

OBJECTIVE

To evaluate the real-world utilization patterns, tolerability, time-to-therapy initiation, and treatment durability among patients ≥ 16 years receiving efgartigimod for gMG within an academic neuromuscular specialty clinic.

BACKGROUND



PROPOSED METHODS

Study Design
- Prospective, single center, observational cohort study

Timeline
- January 2024 to December 2026

Inclusion Criteria
- Patient ≥ 16 years old
- Diagnosis of generalized myasthenia gravis confirmed by physical exam, electrodiagnostics, and/or serologies
- Prescribed by a UVA Neurology provider
- Meets MGFA criteria for class II, III, or IV
- Received at least one cycle or 4 doses
- At least 1 clinical assessment performed within 6 months of therapy initiation

Exclusion Criteria
- Diagnosis of ocular MG
- A thymectomy within 3 months of initiating therapy
- Concomitant use of rituximab, any FcRN inhibitor, or a complement inhibitor
- Concomitant use of IVIG within 14 days of efgartigimod initiation

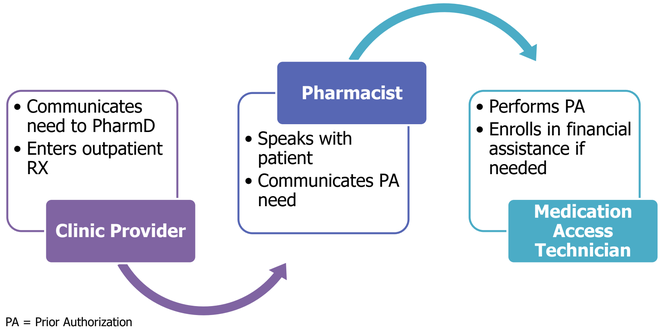
PROPOSED OUTCOMES

Primary Outcome
- Average time on therapy from initiation to discontinuation

Secondary Outcomes
- Average MG ADL total score change from baseline through week 12 or at first assessment
- Number of therapy discontinuation events
- Percentage of patients who achieve minimal symptom manifestation

Disclosures: : **A Holian** has received consulting fees from Genentech and TG Therapeutics **E Chen** has nothing to disclose concerning possible financial or personal relationships with commercial entities that may have a direct or indirect interest in the subject matter of this presentation.

CURRENT WORKFLOW FOR HOME INJECTION



PA = Prior Authorization

FUTURE DIRECTIONS

- Stratify patients by antibody status and reanalyze outcomes data to determine if there is a difference in meaningful response.
- Stratify patients by early-onset versus late-onset disease and reanalyze outcomes data to determine if there is a difference in meaningful response.
- Standardize protocol for pharmacy involvement in therapy initiation, assessment, and monitoring.
- Determine if pharmacist-led coordination shortens time to first dose or expeditious therapy transition.

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