Baseline Characteristics and Demographics of Patients Enrolled in an Expanded Access Program for Efgartigimod in Adult Patients with Generalized Myasthenia Gravis

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Objective:

To provide compassionate access to adults with generalized myasthenia gravis (gMG), who are not enrolled in an ongoing clinical trial, prior to regulatory approval.

Background:
gMG is a rare, chronic, and debilitating IgG autoantibody mediated disease that negatively impacts patients' quality of life. Efgartigimod is a human IgG1 antibody Fc-fragment that blocks the neonatal Fc receptor; thereby decreasing IgG recycling and reducing IgG autoantibody levels. In the phase 3 ADAPT study efgartigimod was shown to be well tolerated and efficacious in patients with gMG. The multinational expanded access program (EAP, NCT04777734) for efgartigimod addresses the unmet need for patients not enrolled in clinical trials who are unable to effectively manage their disease with currently approved therapies

Design/Methods:

Adult patients are eligible for the EAP if they had a confirmed diagnosis of gMG, regardless of antibody status, total MG-ADL score ≥5 at screening (with >50% of the total score due to non-ocular symptoms) and had a documented IgG level of > 6 g/L one month prior to screening. Enrolled patients receive IV efgartigimod (10 mg/kg) on an individualized treatment cycle dosing pattern.

Results:
As of September 24, 2021, 10 patients have enrolled in the EAP. The majority were female (n=8) and the median age was 57 (range 32-73). Six of the patients were AChR-Ab+, while three were seronegative, and one was MuSK-AB+. Patients had previously been on several current gMG treatment options, including six who had received eculizumab, rituximab, or both (n = 3, 1, and 2 respectively). Additionally, four patients had been hospitalized due to their gMG in the 12 months prior to screening

Conclusions:

Efgartigimod was well tolerated and efficacious in the phase 3 ADAPT trial. The EAP offers an important treatment option for those patients lacking an effective management strategy for their gMG, not enrolled in an ongoing clinical trial.