Post-intervention Status in Patients With Refractory Myasthenia Gravis Treated With Eculizumab During REGAIN and Its Open-Label Extension

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Study Question
Can eculizumab help patients with anti-acetylcholine receptor-positive (AChR+) refractory generalized myasthenia gravis (gMG) to achieve the Myasthenia Gravis Foundation of America (MGFA) post-intervention status of minimal manifestations (MM)?

What Is Known and What This Paper Adds
The REGAIN trial yielded evidence for the efficacy and safety of eculizumab as a treatment for AChR+ refractory gMG. The present analyses of data from the open-label extension provide Class II evidence that eculizumab leads to sustained MM status in patients with AChR+ refractory gMG.

Methods
For these longitudinal analyses, the investigators analyzed data from 117 adults with AChR+ refractory gMG who participated in the international, 26-week, phase 3, double-blind, randomized REGAIN trial and the subsequent open-label extension period. Of these participants, 61 received placebo during the double-blind phase (REGAIN) before switching to eculizumab treatment for the open-label extension. The other 56 participants received eculizumab throughout REGAIN and the open-label study. For this tertiary endpoint analysis, the participants were assessed for the MGFA post-intervention status of improved, unchanged, worse, MM and pharmacologic remission at weeks 4, 12 and 26 of REGAIN and at weeks 26, 40, 52, 78, 104 and 130 of the open-label extension.

Results and Study Limitations
Eculizumab-treated patients were more likely to have achieved MM status by week 26 of REGAIN than placebo-treated patients (common odds ratio, 2.3; 95% confidence interval [CI], 1.1–4.5). This is Class II evidence that eculizumab helps patients with AChR+ refractory gMG to achieve MM status. After 130 weeks of eculizumab treatment, 57.3% (95% CI, 45.4–68.7%) of the patients had achieved MM status, and 88.0% (95% CI, 78.4%–94.4%) had achieved at least improved status. Eculizumab’s long-term safety profile was consistent with previous reports. The main limitation of the present analysis is its reliance on open-label data.

Registration, Study Funding and Competing Interests
This study was funded by Alexion Pharmaceuticals and was registered with ClinicalTrials.gov (REGAIN, NCT01997229; open-label extension, NCT02301624). Some authors report receiving personal fees, consulting fees, conference and travel expenses, and research support from healthcare companies, including Alexion Pharmaceuticals; participating in scientific advisory boards for healthcare companies, including Alexion Pharmaceuticals; receiving consulting fees and funding from foundations and the US and German governments; owning stock in Alexion Pharmaceuticals; and being employees of healthcare companies, including Alexion Pharmaceuticals. Full disclosures can be found at Neurology.org/N.

Table
Percentages of Patients Achieving Improved or MM Status After Various Durations of Eculizumab Treatment

<table>
<thead>
<tr>
<th>Duration of eculizumab treatment</th>
<th>Proportion of patients, % (95% CI)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Improved status</td>
</tr>
<tr>
<td>26 wk</td>
<td>66.1 (56.5–74.7)</td>
</tr>
<tr>
<td>52 wk</td>
<td>78.4 (69.2–86.0)</td>
</tr>
<tr>
<td>78 wk</td>
<td>86.5 (78.0–92.6)</td>
</tr>
<tr>
<td>104 wk</td>
<td>84.5 (75.0–91.5)</td>
</tr>
<tr>
<td>130 wk</td>
<td>88.0 (78.4–94.4)</td>
</tr>
</tbody>
</table>

A draft of the short-form article was written by M. Dalefield, a writer with Editage, a division of Cactus Communications. The corresponding author(s) of the full-length article and the journal editors edited and approved the final version.

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