Clinical trials in myasthenia gravis

**Thymectomy in non-thymomatous seropositive, generalized myasthenia gravis patients receiving prednisone**

Overview: This multicenter, international trial is comparing prednisone monotherapy to thymectomy plus prednisone in patients with non-thymomatous seropositive, generalized myasthenia gravis. The three-year trial is being conducted at over 60 centers around the world. As of late March 2009 approximately 70 patients have been enrolled, with a goal of enrollment of 200 patients.

Primary outcome measures:
- Area under the Curve (AUC) of QMG Score over 3 years
- Area under the prednisone Dose Time Curve (AUDTC), conditional on the results of AUC of the QMG score, over 3 years

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**Safety and efficacy of Eculizumab in patients with refractory, generalized myasthenia gravis**

Overview: Eculizumab is a monoclonal antibody that blocks the activation of complement by binding to C5 and preventing the cleavage of C5 to C5a and C5b. The rationale for studying eculizumab in MG is based on our knowledge that MG is a complement-dependent, autoimmune disorder, and thus the hope is that eculizumab will inhibit complement activation in MG and lessen disease severity. This study is a randomized, double-blind, placebo-controlled, cross-over, multi-center study lasting ~ 10 months. The trial is sponsored by Alexion Pharmaceuticals.

Primary Outcome Measures:
- Adverse events
- QMG scores

Secondary Outcome Measures:
- MGFA Post-Intervention Status
- MG-Activity of Daily Living Profile (MG-ADL)
- SF-36
- Forced vital capacity and negative inspiratory force

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**Methotrexate in myasthenia gravis**
Overview: This multicenter study will compare prednisone monotherapy to prednisone plus methotrexate in patients with generalized myasthenia gravis. Enrollment is anticipated to start in late 2009 or early 2010.

Primary outcome measures:
• Total prednisone dose AUC during months 4 – 12 of study.

Secondary Outcome Measures:
• QMG scores monthly

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