INTRODUCTION and PURPOSE

- In the most recent American Society for Apheresis Guidelines on the Use of Therapeutic Apheresis in Clinical Practice, employing therapeutic plasma exchange (TPE) for long-term treatment of myasthenia gravis (MG) patients
  - New indication
  - Category II, grade 2B recommendation
- Data for this indication is evolving
- Subjective assessments of these patients are often uninformative.
- We sought to better characterize the impacts of long-term TPE in these MG patients using validated instruments.

METHODS

In this prospective observational study, we used a combination of validated instruments and open-ended questions clarifying concerns that are routinely applied to MG patients. The two validated instruments were the MG Activities of Daily Living (MG-ADL) and MG Quality of Life 15 (MG-QoL15r) profiles. Based on previous literature, a 2-point change in the MG-ADL and a 10-point change in the MG-QoL15r indicates significant improvement or worsening. Over a 3-month period, MG patients receiving long-term TPE were assessed using a single-form questionnaire that integrated all of the MG-ADL and MG-QoL15r elements and was completed at every visit. Patients unable to complete the survey due to their medical condition were exempted.

QUESTIONNAIRE

- Active pharmacotherapy included prednisone, azathioprine, mycophenolate, rituximab, and pyridostigmine.
- All patients reported that lengthening the interval between successive TPE treatments, even by a few days, resulted in noticeable MG changes.
- During the study period, 4 patients (44%) had significant changes identified by the MG-ADL, a mean of 5.5 times per patient (range 2-8) and 2 (22%) had significant changes identified by the MG-QoL15r, a mean of 2 times per patient (range 1-3).
- MG-ADL appeared to be more sensitive in correlating with patient-reported clinical changes, with clinical improvements identified a mean of 3.2 times per patient and clinical deteriorations identified a mean of 2.3 times per patient (compared to 1.5 and 1 times per patient, respectively, for the MG-QoL15r; p=0.03 for interaction effect).
- Subjective clinical deteriorations were correlated with objectively worsening MG-ADL scores, and was used as evidence to medically justify intensification of TPE therapy.

RESULTS

- Time Interval Between Treatments
  - Just right: 70%
  - Too long (need less time in between treatments): 9%
  - Too short (need fewer treatments): 3%
  - Don’t know/not sure: 18%

- Most Bothersome Symptom
  - Breathing/chest pain: 46%
  - Fatigue: 6%
  - Choking/swallowing: 6%
  - Chewing: 3%
  - Talking: 5%
  - Large muscle weakness: 31%
  - Double vision: 3%

- Frequency of treatments
  - Most Bothersome Symptom
  - Too short (need fewer treatments)
  - Too long (need less time in between treatments)
  - Clinical deterioration

CONCLUSIONS

- Objective longitudinal assessments in MG patients receiving long-term TPE may be helpful for accurate disease monitoring.
- A subset of MG patients receiving long-term TPE still has dynamic changes in disease status as assessed by clinical history and two different validated instruments.
- In all patients with stable MG, both the MD-ADL and MG-QoL15r accurately indicated no significant changes.
- In patients with fluctuating disease status, MG-ADL was more sensitive to both clinical improvement and worsening.
- These findings need to be validated in larger studies.