EFGARTIGIMOD IN MYASTHENIA GRAVIS: A REAL LIFE SINGLE-CENTER EXPERIENCE

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INTRODUCTION

Efgartigimod (EFGA) is a competitive ligand to IgG for binding to the neonatal receptor for the crystallizable fragment (FcRn), promoter of immunoglobulin recycling with reduction of anti-AchR ab levels. The phase 3 study ADAPT led to approval of EFGA for treatment of generalized miastenia gravis (gMG). We aim to report our real-world experience with EFGA in 19 patients (pts) with gMG.

METHODS

EFGA is administered by cycles of 4 endovenous weekly infusions and readministered in relation to clinical worsening (at least 2 points ADL). Pts were treated with EFGA according to AIFA inclusion criteria. Clinical outcome was assessed through scales (MGADL, MGFA, MGFA-PIS) at the beginning and at the end of each cycle and 10 days after the end of the cycle. Steroid dosage was also recorded at the same timepoints. Distance between each cycle was recorded. Pts were retreated if responders, after an increase of at least 2 ADL points.

In a subset of patients, repetitive nerve stimulation (RNS) before the first and fourth infusion of the EFGA cycle was performed in order to evaluate decremental response and study the relation between eventual decremental response variation and clinical outcome. 3 Hz RNS was performed in 4 selected muscles of the facial/bulbar, axial or limb district with 10 supramaximal stimuations. Decremental response (a percentage value) was recorded for each muscle and defined as the % difference between basal CMAP and 4th CMAP amplitude in basal conditions and after 1, 2 and 3 minutes after one minute voluntary maximal effort.

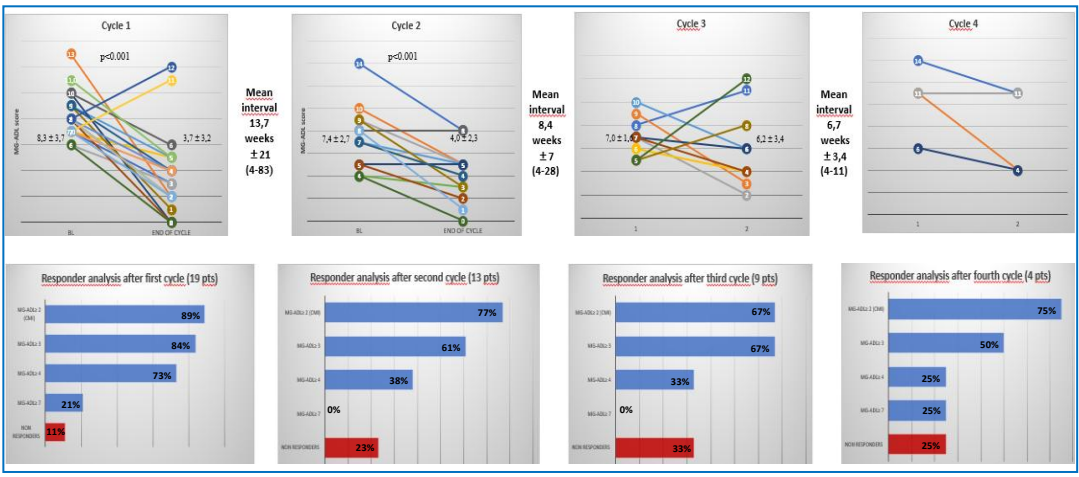

Figure 1. MGADL value at between first and last infusion of the first, second, third and fourth EFGA cycle along with responder analysis (responders= pts with at least 2 points ADL decrease).

RESULTS

19 pts EFGA treated (13M, 6F, age 53 ±15, disease duration 10 years ± 2). Maximun follow up: 12 months. Maximum number of cycles: 7. ADL variations and responder analysis is reported in figure 1 (responders: a minimum decrease of 2 points in ADL scale). The MGADL decreased significantly (p<0.001) from 8.3 (SD: 1.7) to 3.7 (SD: 3.2) during the first cycle and from 7.4 (SD: 2.7) to 4.0 (SD: 2.3) during the second cycle (p=0.001). QMG decreased significantly (p<0.001) from 15.7 (SD: 2.2) to 10.3 (SD: 2.1) in the first and from 15 (SD: 3.3) to 10.8 (SD:2.9) in the second cycle (p=0.013). The steroid dosage decreased (p=0.13) at the end of the first cycle from 14,6 (SD: 9.2) to 13,3 (SD: 8.9), at the end of second cycle from 13 (SD: 8.8) to 12,6 (SD: 8,3) [p=0.74] and at the end of the third cycle from 13,6 (SD:10) to 13.3 (SD:9.7) (p=0.58). Steroid dosage between onset of EFGA and the last visit was increased from 14,6 to 17 mg (p=0.69). No rescue therapies were needed after EFGA start. About the safety, 1 pt presented a severe VZV reactivation and 1 pt presented pneumonia with MG worsening after the third infusion of the cycle. Only preliminary data on RNS are available and no conclusions can be drawn: figure 2 reports an example of RNS decrease variation in a responder and non responder patient.

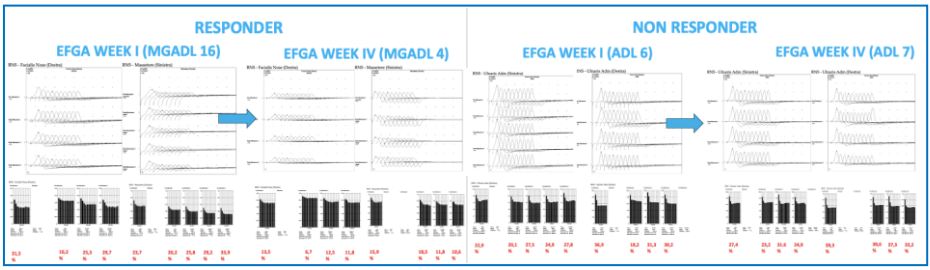

Figure 2. RNS at week I and IV of EFGA cycle in a responder (left) and non responder patient (right).

CONCLUSION

In our population, EFGA proved effective with a good safety profile. We did not observe a significant steroid sparing effect, maybe because the population mainly included refractory patients with high disease activity. We consider RNS as a promising biomarker for clinical response and encourage further studies.