Efgartigimod in refractory multifocal motor neuropathy: a case report.

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OBJECTIVE

Describe the first patient with multifocal motor neuropathy (MMN) treated with Efgartigimod.

CASE REPORT

In 2020, a 40-year-old man developed within a year asymmetric weakness in the hands and upper limbs. Nerve conduction studies revealed conduction blocks with sparing of sensory fibers. Cerebrospinal fluid showed albumin-cytological dissociation, and serum anti-GM1 IgM and anti-GD1b IgM antibodies were positive at high titer. He was diagnosed with multifocal motor neuropathy (MMN) and started IV immunoglobulin (IVIg) treatment (2 g/kg every 5 weeks). Over the following 3 years, he became progressively less responsive to IVIg and developed bilateral foot drop, with severe walking difficulties. Rituximab (1 g × 2 cycles) was administered without reported improvement. We met the patient in July 2024 for a second opinion. Nerve ultrasound supported the MMN diagnosis (enlargement of the brachial plexus, cervical roots, and median nerve), and a genetic panel for neuromuscular diseases (especially Charcot-Marie-Tooth and motor neuron diseases) resulted to be negative. We thus shortened the IVIg 1g/kg interval to 3 weeks, leading to slight improvement of the strength in the lower (but not in the upper) limbs. In February 2025, during influenza infection, he was hospitalized because of an acute disease flare with respiratory failure. For this, he received IV methylprednisolone (500 mg for 3 days). The patient became then rapidly tetraplegic, requiring continuous mechanical ventilation and artificial nutrition. Since IVIg (1 g/kg) were not effective, we initiated plasma exchange (PLEX), observing rapid improvement in motor functions. However, PLEX had to be prematurely interrupted due to a catheter infection.

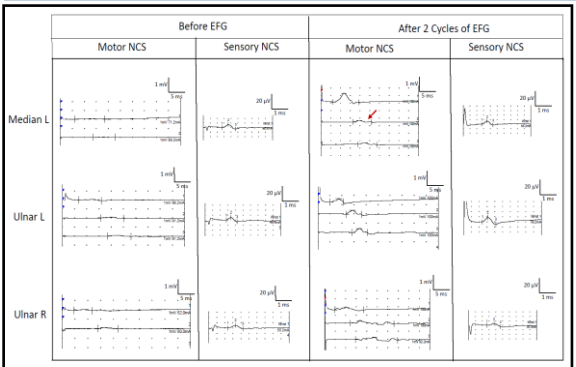

Fig.1. Nerve conductions studies in the upper limbs before and after 2 cyces of EFG. Motor fibers were inexcitable during the disease flare, and partially recovered after EFG. A conduction block is shown (red arrow).

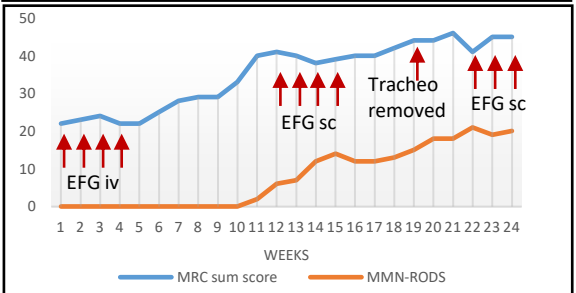

Fig.2. Muscle strength (MRC score) and disability (MMN-RODS) over 6 months, after the onset of EFG.

METHODS

Efgartigimod (EFG), a monoclonal antibody that binds to the neonatal Fc receptor (FcRn) and promotes accelerated degradation of circulating IgG antibodies, was initiated as an off-label therapy as alternative to PLEX. The patient received EFG 10 mg/kg (800 mg) IV once weekly for 4 consecutive weeks, resulting in a steady and progressive improvement in muscle strength in the 4 limbs over the next 8 weeks. Subsequently, maintenance therapy was continued with a free interval of 8 weeks with EFG 1000 mg SC weekly for 4 weeks. The patient has just started the third cycle, without side effects.

RESULTS

The prospective collection of clinical (MRC scale, MMN-RODS) and neurophysiological (ENG) data [Figs. 1 and 2] of this patient demonstrate a progressive and constant improvement. Three weeks after the second cycle of EFG, the patient was weaned off mechanical ventilation and resumed oral feeding. The progress is still ongoing along the treatment cycles.

CONCLUSIONS

Efgartigimod treatment was safe and associated with improvement in muscle strength and disability.