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CASE REPORT

Efgartigimod as Fast-Acting Rescue Therapy in Very Late-Onset Myasthenia Gravis

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ABSTRACT

Myasthenic crisis (MC) is a potentially fatal complication of myasthenia gravis (MG), often requiring intensive care and rescue therapies such as intravenous immunoglobulin (IVIG) and corticosteroids. Efgartigimod, a neonatal Fc receptor (FcRn) antagonist, offers a novel approach by selectively reducing circulating pathogenic IgG. We describe a 77-year-old man with anti-AChR-positive generalized MG who developed a severe MC, with little response to IVIG and high-dose corticosteroids. Intravenous efgartigimod was administered as rescue therapy with rapid and sustained clinical improvement. This case supports the utility of efgartigimod in MC unresponsive to conventional therapies, including in older patients with significant comorbidities.

Keywords: Efgartigimod; FcRn inhibition; Rescue therapy; Very late-onset myasthenia gravis; AChR antibodies; Myasthenic crisis

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Myasthenia gravis (MG) is a chronic autoimmune disorder affecting the neuromuscular junction, characterized by fluctuating weakness and fatigability of skeletal muscles [1]. In 85–90% of generalized MG cases, autoantibodies target the acetylcholine receptor (AChR), activating complement pathways and inducing structural damage to the postsynaptic membrane. Myasthenic crisis (MC), the most life-threatening presentation of MG, is defined by respiratory failure requiring mechanical ventilation or profound bulbar weakness and occurs in approximately 15–20% of patients with MG during their disease course [2, 3].

Standard rescue treatments for MC include intravenous immunoglobulin (IVIG), plasma exchange (PLEX) and high-dose corticosteroids [4]. However, some patients remain refractory to these interventions [2]. Efgartigimod, a selective FcRn antagonist, offers a novel immunomodulatory mechanism that reduces circulating IgG, including pathogenic anti-AChR antibodies [5]. Unlike PLEX or IVIG, efgartigimod does not rely on plasma donor availability or invasive vascular access, and its selective effect spares other immunoglobulin isotypes and immune functions.

Here, we present a case of a patient with severe, treatment-refractory MC who experienced a rapid and sustained clinical improvement following administration of efgartigimod, highlighting its emerging role in acute MG management.

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A 77-year-old man was diagnosed with anti-AChR-positive generalized MG. His initial symptoms included ptosis, diplopia, cervical weakness and exertional dyspnoea. He presented history of hypertension, diabetes mellitus, asthma, vascular encephalopathy, renal/hepatic cysts and diverticulosis. He was started on pyridostigmine, azathioprine and low-dose corticosteroids.

Eight months after the clinical onset, the patient presented to the emergency department with worsened asthenia, postural instability and a fall. Neurological examination revealed bilateral ptosis (right-predominant), cervical flexor weakness, marked fatigability of the ocular and facial muscles and global tetraparesis (worse in the lower limbs). The MG-ADL score was 10, which progressed to 17 within a few days.

The patient was treated with IVIG (0.4 g/kg/day for 5 days) without significant improvement. Non-invasive ventilation was initiated for respiratory distress. High-dose corticosteroids (1 mg/kg/day) also failed to produce clinical benefit. Laboratory tests showed IgG levels of 1134 mg/dl (normal: 700—1600 mg/dl), anti-AChR antibody > 20 nmol/l (normal: < 0.40 nmol/l) and negative anti-MuSK, IL-6 (24 pg/ml; normal: < 7 pg/ml). Furthermore, elevated anti-GAD65 (46.9 IU/ml; normal: < 5.0 IU/ml) and abnormal glycaemic profile supported the diagnosis of autoimmune diabetes. Two weeks later, considering the lack of control on MC and inadequate response to IVIg plus steroids with high risk of further deterioration, the patient was a candidate for intravenous infusion of efgartigimod at a dose 10 mg/kg (700 mg), administered in four weekly infusions per cycle. Within the first infusion, the MG-ADL score was reduced from 10 to 2, with improved glycaemic control despite high and stable steroids doses (Fig. 1). The patient was discharged home in stable condition and completed the first cycle in an outpatient setting. By the end of the cycle, the patient achieved minimal symptom expression (MSE) with MG-ADL of 0, while QMG improving by 5 points, and no adverse events were reported. A second cycle was administered in the following months with continued remission. Anti-GAD65 antibodies became undetectable (< 1 U/ml). After 6 months of follow-up,

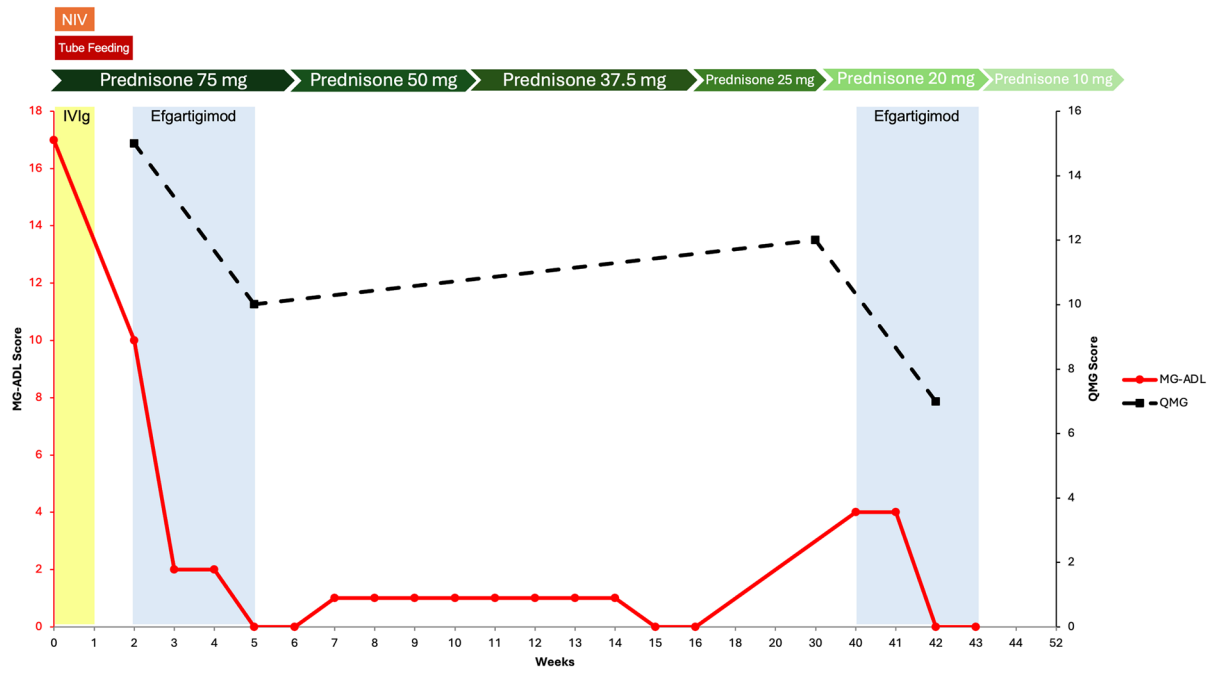

Fig. 1 Treatment timeline and patient's clinical course after myasthenic crisis as assessed by myasthenia gravis-activities of daily living (MG-ADL) and quantitative myasthenia gravis (QMG)

the patient remained clinically stable, and MG-ADL 1 (ocular only) and corticosteroids were reduced (Fig. 1).

This case highlights the clinical efficacy and rapid onset of action of efgartigimod in a patient with myasthenic crisis unresponsive to conventional rescue therapies. The patient's improvement aligns with the known pharmacodynamics of efgartigimod and the findings of the ADAPT phase III trial [5]. Efgartigimod is an engineered IgG1 Fc fragment that binds with high affinity to the neonatal Fc receptor (FcRn), preventing IgG recycling and accelerating lysosomal degradation of circulating IgG antibodies. This includes pathogenic autoantibodies such as anti-AChR, central to the pathogenesis of generalized MG. Unlike PLEX, which requires repeated sessions and central venous access, or IVIG, which may have delayed and unpredictable responses, efgartigimod acts rapidly, with effects often observed within 1 week.

While patients in myasthenic crisis were excluded from the ADAPT trial, recent case reports and real-world data have shown that efgartigimod may offer effective and safe treatment in acute settings [6, 7]. In our patient, MG-ADL improvement was evident after the first infusion and complete remission (MSE) was achieved by the end of the first cycle. The ability to taper corticosteroids and improve glucose control is also notable, particularly in older patients with comorbid diabetes, offering an interesting opportunity to treat IgG-mediated comorbidity through FcRn inhibition, as already reported in stiff person syndrome [8].

The response in this case further supports the use of efgartigimod as a potential rescue therapy in refractory MC, expanding its use. Of interest, this report confirms data on efficacy and safety of efgartigimod in VLOMG patients as reported in recent studies from Asian countries [6, 9, 10].

Efgartigimod induced rapid and complete remission in an elderly patient with treatment-refractory myasthenic crisis, with excellent tolerability and a steroid-sparing effect. This case supports the potential role of FcRn antagonism in the acute management of MG and warrants further investigation in prospective studies including patients with MC.

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Data Availability. Data are available upon reasonable request to the corresponding author.

Declarations

Conflict of Interest. On behalf of all authors, the corresponding author states that Nicasio Rini, Flora D'Amico, Valentina Portera, Filippo Brighina and Vincenzo Di Stefano have no conflicts of interest to declare.

Ethical Approval. All human and animal studies have been approved by the appropriate ethics committee and have therefore been performed in accordance with the ethical standards laid down in the 1964 Declaration of Helsinki and its later amendments. Patient informed consent was obtained and signed for the study and publication. All the authors of the present study were the patient's clinicians.

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