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CASE REPORT

Case Report: A Patient with Lambert-Eaton Myasthenic Syndrome Successfully Treated with Efgartigimod

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Background: Lambert-Eaton myasthenic syndrome (LEMS) is a neuromuscular junction disorder which is frequently associated with tumors. Those patients generate pathogenic antibodies such as voltage-gated calcium channel (VGCC) antibodies. Existing studies have established the efficacy of efgartigimod in MG. This article reports the effect of efgartigimod following its administration to a patient with LEMS.

Case Presentation: A 73-year-old male was diagnosed with small cell lung cancer two years ago. Gradually he developed difficulties in speaking, eating, and lifting his head, along with limb weakness. The neuroelectrophysiological examination findings were consistent with LEMS. Laboratory tests detected VGCC antibodies and SOX-1 antibodies in the serum. The patient's symptoms continued to worsen after one week of corticosteroids use. Consequently, efgartigimod at a dose of 10 mg/kg was added once a week. After three weeks of treatment, the patient's clinical manifestations had improved significantly. Laboratory tests revealed a decrease in serum VGCC antibody and IgG levels.

Conclusion: This is the first report on the use of efgartigimod in LEMS. The patient demonstrated substantial clinical improvement following treatment. Nevertheless, more clinical studies are required to verify whether efgartigimod offers a novel treatment approach for patients with LEMS.

Keywords: LEMS, Efgartigimod, VGCC, SOX-1

Background

Lambert-Eaton myasthenic syndrome (LEMS) is an autoimmune disorder affecting the presynaptic neuromuscular junction, caused by P/Q-type voltage-gated calcium channel (VGCC) antibodies. These antibodies impair acetylcholine release from the presynaptic membrane, leading to a series of clinical manifestations. Efgartigimod (efgartigimod alfa-fcab, Vyvgart™), the world's first approved neonatal Fc receptor (FcRn) antagonist, reduces pathogenic IgG antibodies and blocks IgG circulation to exert efficacy. In China, it is mainly used for acetylcholine receptor (AchR)-positive MG patients, with few reports on its efficacy in other diseases. This article presents an efficacy observation of LEMS treated with efgartigimod. The study was approved and performed underthe guidelines of the Institutional Ethics Committee of Huashan Hospital.

Case Presentation

A 73-year-old male diagnosed with small cell lung cancer (SCLC) in May 2023 received 8 courses of chemotherapy without surgery. In June 2024, he developed neurological symptoms: ptosis, dysarthria, masticatory difficulty, head elevation impairment, and predominant lower limb weakness, leading to bedridden status. His muscle weakness was mild, fluctuating, and exacerbated by activity. Methylprednisolone (40 mg/day) for 3 days yielded no significant improvement.

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After admission, the patient had a positive eyelid fatigue test. MG Activities of Daily Living (MG-ADL) score was 11/24, Quantitative Myasthenia Gravis (QMGs) 17/39, and Myasthenia Gravis Composite (MGC) 21/50 (Figure 1). Routine blood tests were unremarkable. Cerebrospinal fluid pressure was 85 mmH_2O; analysis showed no malignant cells, normal white blood cell count, elevated protein (713 mg/L, normal 150–450 mg/L), and normal glucose, chloride, lactic acid, and lactate dehydrogenase levels. Serum IgG was 13.58 g/L (normal 7–16 g/L; Figure 2), IgA 2.91 g/L (normal 0.7–4 g/L), IgM 1.51 g/L (normal 0.4–2.3 g/L), and IgE 198.36 IU/mL (normal 0–100 IU/mL; Figure 3). Serum VGCC antibody was 162.12 pmol/L (normal ≤30 pmol/L; Figure 2), and anti - SOX - 1 antibody titer was 1:100. Serum AChR antibody was negative. Lower extremity color Doppler ultrasound showed left lower extremity intermuscular vein

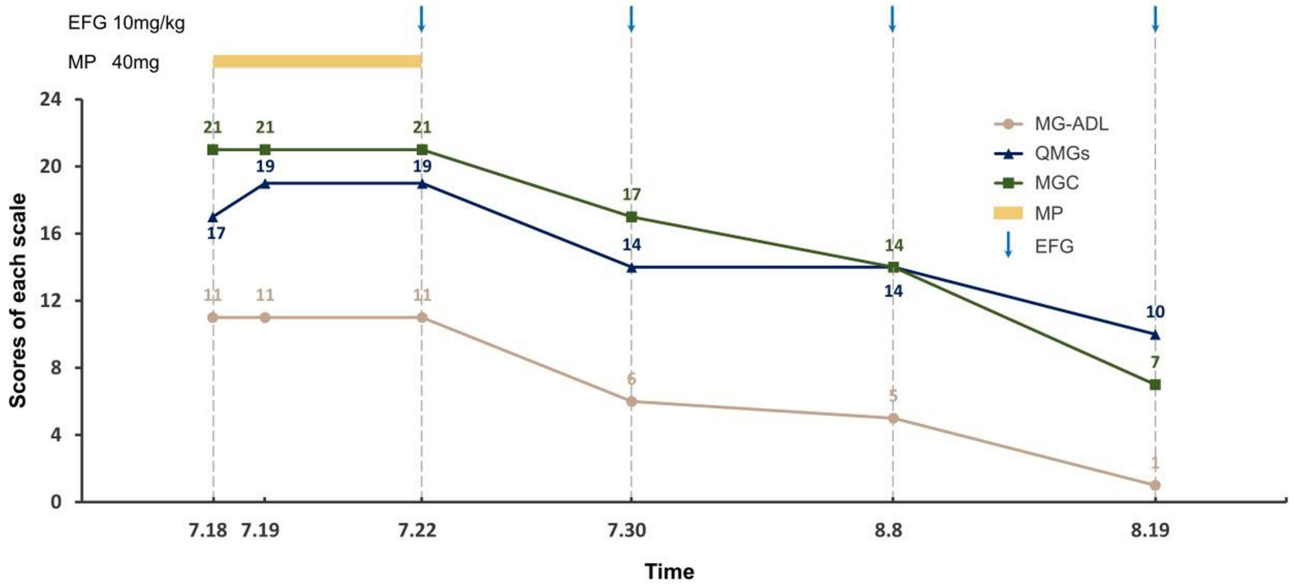

Figure 1 The scores of each scale.

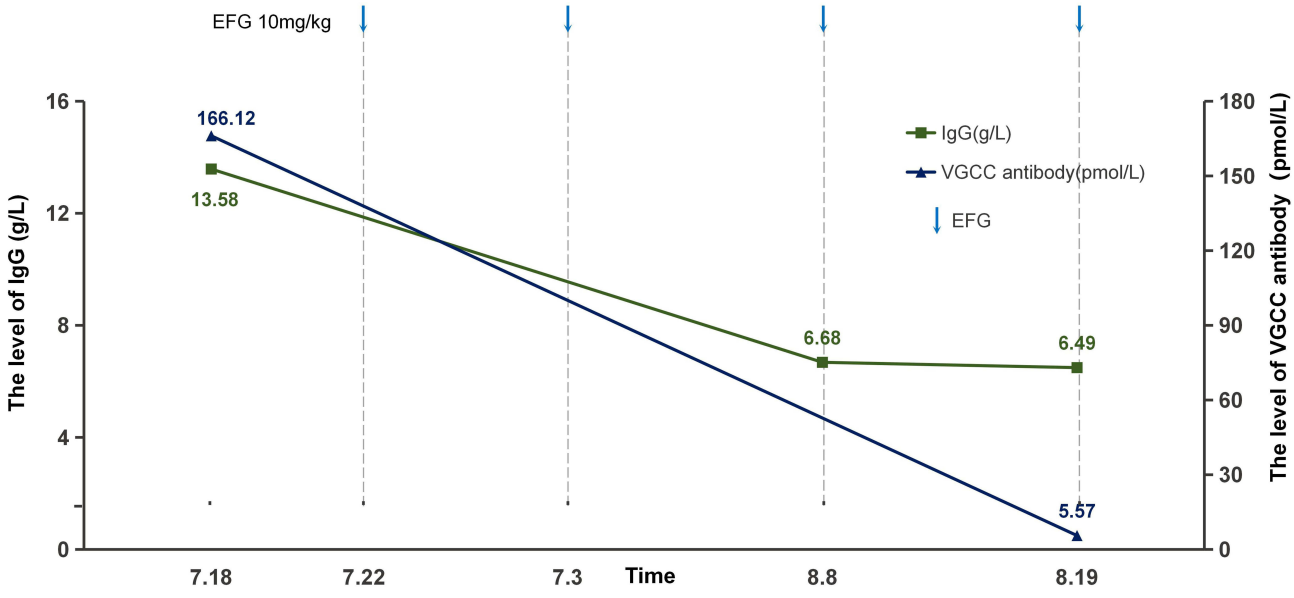

Figure 2 The level of IgG and VGCC antibody.

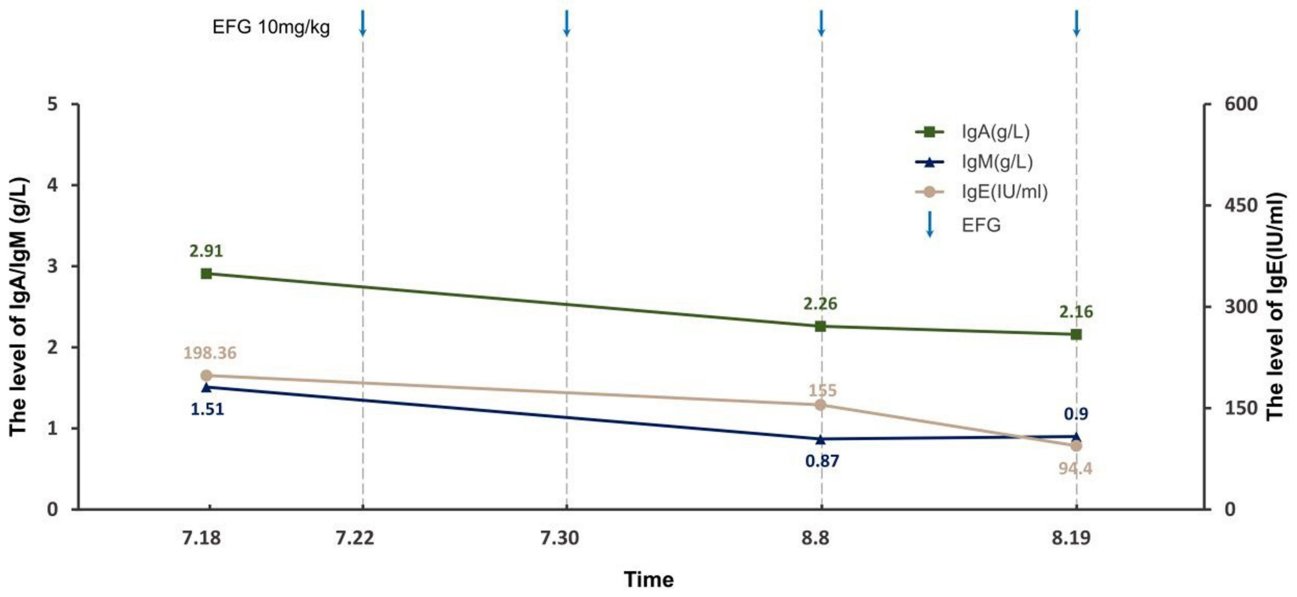

Figure 3 The level of IgA/IgM/IgE.

thrombosis. Neuroelectrophysiological findings: Compound Muscle Action Potential (CMAP) amplitude decreased or at the lower normal limit, with or without reduced conduction velocity. Sensory Nerve Action Potential (SNAP) amplitude decreased or normal, with or without slightly reduced conduction velocity. Repetitive Nerve Stimulation (RNS) showed low-frequency stimulation-induced CMAP amplitude attenuation beyond normal; high-frequency stimulation or strong contraction led to >100% significant amplitude increment, suggesting LEMS.

At the beginning 80mg/d methylprednisolone and pyridostigmine were administered. Over the following days, the patient showed marked worsening of bilateral lower extremity weakness and significantly increased resting dyspnea, with clinical scores (MG-ADL 11, QMGs 19, MGC 21; Figure 1). Although we had increased the amount of hormones, the symptoms had worsened. We were in need of new treatments to improve patients' symptoms quickly. On the fifth day of corticosteroid administration (July 22nd), a single dose of 400mg efgartigimod was administered. One week later, the patient could sit independently, feed and brush teeth unassisted, and actively elevate lower limbs from the bed, with improved scores (MG-ADL 6, QMGs 14, MGC 17; Figure 1). Subsequently, the patient received 400mg efgartigimod on July 30th, August 8th, and August 19th respectively. During hospitalization for the fourth efgartigimod dose (August 19), serum IgG dropped to 6.49 g/L and VGCC antibody to 5.57 pmol/L (Figure 2); IgA to 2.16 g/L, IgM to 0.9 g/L, and IgE to 94.4 IU/mL (Figure 3). MG-ADL score further decreased to 1, QMGs to 10, and MGC to 7 (Figure 1). These results indicate a significant positive response to efgartigimod in this LEMS patient.

One month after the fourth efgartigimod dose, telephone follow-up showed the patient's daily life was largely unaffected. However, over a month after the last efgartigimod dose, the patient died suddenly at home. The family reported the patient had chest and back pain that morning, took painkillers, and died later that day. Given the patient had lower extremity venous thrombosis, we hypothesize that the cause of death was pulmonary embolism or a cardiac event.

Discussion

LEMS is a rare autoimmune disorder characterized by proximal muscle weakness, absent tendon reflexes, and autonomic dysfunction. Approximately 90% of LEMS patients exhibit autoantibodies against presynaptic P/Q-type VGCC.[1] Approximately half of LEMS patients are associated with tumors, especially SCLC. Several antibodies have been identified in patients with SCLC and LEMS. Specifically, VGCC antibodies, primarily of the IgG class, cause a reduction in the amount of ACh released from the presynaptic nerve endings,[1,2] which in turn leads to insufficient activation of postsynaptic ligand-gated sodium and potassium channels,[2] eventually leading to muscle weakness and

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autonomic symptoms. Additionally, some patients exhibit positive SOX-1 antibodies. While VGCC is a cell-surface autoantigen, SOX-1 represents an intracellular autoantigen.

The preferred treatment method for LEMS is anti-tumor treatment. For patients who cannot undergo surgery or have undetected tumors, effective symptomatic and immunomodulatory treatments are needed. There's no unified drug regimen for LEMS currently; symptomatic treatment commonly uses amifampridine and pyridostigmine to enhance neurotransmission. Amifampridine, the current first-line treatment, is not yet available in China. Since it cannot be used, immunotherapy - including corticosteroids, immunosuppressants and immunomodulators- becomes particularly important. However, these drugs may take effect slowly and some have unbearable side effects. In this case, the patient's symptoms worsened during one week of corticosteroid use. Higher glucocorticoid doses also raise risks of gastrointestinal bleeding and femoral head necrosis. Moreover, the patient presented with manifestations of the pre-crisis stage. Therefore, a treatment method that can rapidly improve the symptoms is urgently needed. In the past, for LEMS patients with emergency conditions, plasma exchange or intravenous immunoglobulin (IVIG), which have relatively rapid onset, were recommended.

Targeting FcRn is a new strategy for treating antibody-mediated diseases. FcRn prolongs IgG lifespan by protecting it from lysosomal degradation.[3] Studies confirm that inhibiting the neonatal Fc receptor in MG patients accelerates IgG catabolism, reducing total IgG and pathological autoantibody levels.[3] Efgartigimod, an FcRn antagonist, is approved in the US for anti-AChR antibody-positive MG patients,[4] with confirmed safety and efficacy.[5] Japan recommends it for MG patients unresponsive to corticosteroids.[6] It does not affect other immunoglobulin (eg, IgA, IgM) or albumin levels, while the total IgG level returns to baseline about 8 weeks after the last intravenous injection.[7]

Compared to traditional immunomodulatory therapies, FcRn inhibitors offer significant advantages, particularly their ability to rapidly reduce IgG levels. This reduction is comparable to that achieved through large-volume plasma exchange but with the added benefit of inducing significant clinical responses within just 1 to 2 weeks after treatment begins.[8] Unlike plasma exchange, FcRn inhibitors selectively target IgG while retaining other plasma proteins like albumin, thus providing a more precise treatment approach. In studies on MG patients, efgartigimod has demonstrated to have a faster onset of action compared to IVIG, with significant advantages in improving MG-ADL scores during the first week and fourth week of treatment.[9] Plasma exchange and IVIG can cause severe adverse reactions like cardiovascular issues, infections, acute renal failure, and thromboembolism, but these are rare with efgartigimod.[5]

In this urgent case, corticosteroids had limited effect, probably due to delayed action. Plasma exchange and IVIG (which increase thrombosis risk) were unsuitable because of the patient's lower extremity deep venous thrombosis. Efgartigimod was thus justified but this constitutes off-label use. Following three cycles of efgartigimod, the patient's muscle strength significantly improved, and all relevant clinical scores showed a marked reduction (Figure 1). Additionally, we observed a substantial decrease in serum IgG and VGCC antibody levels (Figure 2), while the levels of IgA, IgM, and IgE showed no obvious decline (Figure 3). It indicates that efgartigimod effectively clears pathogenic antibodies in LEMS patients.

The most common adverse events of efgartigimod were headache and nasopharyngitis.[5,10] However, this patient did not experience these side effects. Notably, he died a month after his last efgartigimod dose. No literature evidence links efgartigimod to sudden death to date.[5,8] Furthermore, given that the half-life of efgartigimod is approximately 92 hours,[11] we inferred that efgartigimod would be fully eliminated from the body in about 15 days. Therefore, we assumed it might be an independent event. The primary symptom preceding death was chest pain, indicating possible pulmonary embolism or cardiovascular complications.

The first-line treatment for LEMS is removing primary tumor. If unresectable, efgartigimod is a choice. Limitations were as follows: it is a case report and more evidence is needed; whether long-term use will cause additional side effects or promote tumor growth is unknown; whether the efficacy will fade after long-term use needs observation. Despite the unfortunate sudden death of our patient, we observed a significant improvement in the patient's clinical symptoms and laboratory indicators. Accordingly, efgartigimod is an alternative of treatment for LEMS.

Data Sharing Statement

The raw data supporting the conclusions of this article will be made available by the authors, without undue reservation.

Ethics Approval and Informed Consent

We confirm that institutional approval for the publication of this case details has been obtained from the Institutional Ethics Committee of Huashan Hospital.

Consent for Publication

The patient's family gave written informed consent for the participation and publication of his anonymised case details.

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Disclosure

The authors have no competing interests to declare that are relevant to the content of this article.

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