WEP Warwick Evans Publishing

International Journal of Public Health and Medical Research

ISSN: 3006-7707 (Print), ISSN: 3006-3752 (Online)
Volume 6, Number 7, Year 2026
DOI: https://doi.org/10.62051/ijphmr.v6n7.03



Research Advances and Future Directions of Biotechnology in the Treatment of Myasthenia Gravis

Jiaqi Lin

International Department of Zhejiang Fuyang High School, Hangzhou 311422, China
LJQ210805@163.com

ABSTRACT

Myasthenia gravis (MG) is an autoimmune disease with a core pathological mechanism involving specific attacks by the immune system on proteins at the neuromuscular junction. This study reveals that our understanding of its pathogenesis has expanded beyond mere neuromuscular transmission dysfunction to encompass multiple aspects, including the dysregulation of immune regulatory networks, abnormal activation of the complement system, and damage mediated by specific autoantibodies. While conventional pharmacological therapies can alleviate clinical symptoms and control disease progression, they generally suffer from significant long-term side effects, substantial individual variability in patient response, and an inability to achieve a cure in most patients. In contrast, targeted biologics enable precise intervention in key disease pathways, whereas stem cell therapy and gene therapy offer novel strategies for restoring immune tolerance and achieving long-term functional control of the disease, collectively advancing the therapeutic paradigm from broad nonspecific immunosuppression toward precision medicine. This study reviews the pathogenesis of MG, systematically outlines the current applications of conventional drugs, targeted biologics, thymectomy, and gene therapy in its treatment, analyzes existing therapeutic challenges, and explores future directions, such as precision medicine and novel targeted therapies.

KEYWORDS

Myasthenia gravis; Autoimmune diseases; Targeted biologics; Precision medicine; Gene therapy

1. INTRODUCTION

Myasthenia gravis (MG) is an autoimmune neuromuscular disorder characterized primarily by progressive muscle weakness and fatigue that worsens with physical activity. Patients often present with symptoms such as ptosis, diplopia, dysphagia, and limb weakness; in severe cases, it may even impair respiratory function [3].

Unlike many neurological disorders, myasthenia gravis is not caused by damage to neurons but rather by the immune system mistakenly identifying its own tissues. Under normal circumstances, the human immune system can distinguish between self-cells and foreign pathogens, thereby maintaining proper bodily functions. However, in patients with myasthenia gravis, the immune system produces autoantibodies that target neuromuscular junction proteins, impairing the transmission of signals between nerves and muscles and ultimately leading to reduced muscle contractility [2].

Over the past few decades, scientists have proposed various therapeutic approaches. Early treatments primarily focused on improving neuromuscular transmission efficiency; however, with advancements in immunological research, researchers have increasingly recognized that abnormal immune system responses constitute the fundamental cause of disease pathogenesis. Consequently, modern treatment

Content from this work may be used under the terms of CC BY-NC 4.0 licence (https://creativecommons.org/licenses/by-nc/4.0/).
Published by Warwick Evans Publishing.

strategies have shifted from merely alleviating symptoms to modulating aberrant immune responses [8].

In recent years, the rapid advancement of biotechnology has further propelled innovations in the treatment of myasthenia gravis. The development of monoclonal antibody (mAb) technology has enabled researchers to deliver precise interventions targeting specific immune molecules; progress in stem cell technology has provided novel approaches for restoring immune homeostasis; and the emergence of genome editing technology has made molecular-level disease therapy a reality [1]. These emerging technologies are progressively transforming the therapeutic landscape of myasthenia gravis and offer new hope to patients.

Therefore, this article begins by examining the pathogenic mechanisms of myasthenia gravis, systematically reviewing the current applications of bioengineering, biochemistry, and biomedical technologies in its treatment, analyzing their advantages and limitations, and exploring how modern biotechnologies can facilitate the transition from broad immunosuppression to precision medicine in myasthenia gravis management. Additionally, based on the current landscape, we propose promising future research directions and feasible recommendations to inform related research and therapeutic strategies for myasthenia gravis.

2. PATHOGENESIS OF MYASTHENIA GRAVIS

This disease primarily affects the neuromuscular junction (NMJ), a specialized structure responsible for information transmission between motor neurons and skeletal muscle cells.

Under normal conditions, when a nerve impulse reaches the motor nerve terminal, it triggers the release of acetylcholine (ACh) into the synaptic cleft. Acetylcholine then binds to acetylcholine receptors (AChR) on the surface of the muscle cell membrane, inducing membrane depolarization and thereby initiating the muscle contraction process [3].

However, in most patients with myasthenia gravis, the immune system produces autoantibodies that target acetylcholine receptors. These antibodies impair neuromuscular transmission through multiple mechanisms: first, they directly bind to acetylcholine receptors, preventing proper neurotransmitter binding; second, they promote receptor degradation and clearance, leading to reduced receptor levels; additionally, they activate the complement system, causing structural damage at the neuromuscular junction [2].

Studies have shown that anti-acetylcholine receptor antibodies (AChR-Ab) can be detected in approximately 80% of patients with systemic myasthenia gravis. However, not all patients exhibit the same type of autoantibody. Some patients develop antibodies targeting muscle-specific kinase (MuSK), while others may produce antibodies against low-density lipoprotein receptor-related protein 4 (LRP4). These distinct antibody types influence disease manifestations and therapeutic responses [9].

From an immunological perspective, myasthenia gravis is essentially the result of dysregulation of immune tolerance. Under normal circumstances, the human immune system prevents attacks on its own tissues. However, when the immune tolerance mechanism fails, abnormally activated T cells promote the proliferation and differentiation of B cells, which subsequently produce large amounts of autoantibodies, thereby triggering the disease [7]. Recent studies have further revealed that B cells not only serve as the source of antibody production but also play a role in the progression of the disease, making them increasingly important therapeutic targets [12].

The thymus also plays a significant role in the pathogenesis of myasthenia gravis. The thymus is a vital organ involved in the development and maturation of T cells. Many patients with myasthenia gravis exhibit thymic hyperplasia, and some are additionally diagnosed with thymoma. Studies

suggest that thymic abnormalities may lead to the production of auto-reactive T cells, thereby promoting the progression of autoimmune responses [3].

With a deeper understanding of these mechanisms, treatment strategies for myasthenia gravis have progressively evolved. Modern research no longer focuses solely on enhancing neuromuscular transmission efficiency but also emphasizes the need to intervene in critical pathways, such as autoantibody production, complement activation, and immune dysregulation. These findings establish a fundamental theoretical basis for the subsequent development of bioengineering, biochemical, and biomedical therapeutic technologies.

3. TRADITIONAL DRUG THERAPY AND RESEARCH ON BIOCHEMICAL MECHANISMS

3.1. Cholinesterase Inhibitors

Cholinesterase inhibitors (acetylcholinesterase inhibitors) are one of the most commonly used symptomatic treatments for myasthenia gravis. Among them, pyridostigmine is widely employed in clinical practice and is considered the first-line medication for most patients undergoing treatment [6].

Under normal circumstances, acetylcholine released by nerve terminals is rapidly degraded by acetylcholinesterase (AChE) after completing the signal transmission, thereby terminating neural stimulation. However, in patients with myasthenia gravis, the reduced number of acetylcholine receptors leads to diminished efficiency of neural signal transmission, resulting in weakened muscle contractility [3].

Cholinesterase inhibitors inhibit acetylcholinesterase activity, thereby slowing the degradation rate of acetylcholine and prolonging its retention in the synaptic cleft. Consequently, even with a reduced number of receptors, a greater amount of acetylcholine can bind to the remaining receptors, enhancing the efficiency of information transmission at the neuromuscular junction and alleviating muscle weakness symptoms [6].

3.2. Glucocorticoids and Immunosuppressants

As researchers have increasingly recognized that myasthenia gravis is an autoimmune disease (AD), treatment strategies have shifted from solely improving neuromuscular transmission to regulating the immune system function.

Corticosteroids are among the most widely used immunomodulatory agents. Their primary mechanism of action involves suppression of inflammatory responses and immune cell activation, thereby reducing the production of autoantibodies. Studies have demonstrated that corticosteroids can effectively alleviate patient symptoms and lower the risk of disease recurrence [8].

In addition to glucocorticoids, immunosuppressants such as azathioprine, mycophenolate mofetil, and cyclosporine are widely used for long-term treatment. These drugs reduce persistent damage to the neuromuscular junction by interfering with the proliferation and functional activity of immune cells, thereby mitigating abnormal immune responses [6].

4. APPLICATION OF TARGETED BIOLOGICS (MONOCLONAL ANTIBODIES)

4.1. Complement Pathway Inhibitors

The complement system is a vital component of human innate immune defense, with its primary function being to eliminate pathogens. However, in patients with myasthenia gravis who test positive for acetylcholine receptor antibodies, these autoantibodies can activate the complement system, leading to disruption of the neuromuscular junction structure [3].

Eculizumab and ravulizumab are two humanized monoclonal antibodies that target complement component C5 (C5). These drugs inhibit the complement cascade by blocking the cleavage of C5, thereby reducing damage at the neuromuscular junction [5].

In the REGAIN Phase III clinical trial, the results demonstrated significant improvements in muscle function scores, daily living abilities, and overall quality of life among patients treated with enecadotuzumab. The study also indicated that the drug exhibits a favorable safety profile and tolerability [5].

This study marks the official entry of myasthenia gravis into the era of targeted biological therapy.

4.2. FcRn Inhibitors

In addition to the complement system, autoantibodies are significant pathogenic factors in myasthenia gravis. Consequently, researchers have begun exploring novel strategies to directly reduce the levels of pathogenic antibodies in the body.

The neonatal Fc receptor (FcRn) is a critical protein that protects IgG antibodies from degradation. Under normal circumstances, FcRn prolongs the survival of IgG antibodies. However, in patients with myasthenia gravis, this mechanism also extends the duration of pathogenic autoantibodies.

Efgartigimod and rozanolixizumab are two engineered antibody fragments that target FcRn, which blocks the binding of FcRn to IgG, thereby promoting the degradation of IgG antibodies in vivo and reducing the levels of pathogenic autoantibodies [4].

In the ADAPT Phase III clinical trial, researchers observed that patients treated with aigamol exhibited significant symptomatic improvement within several weeks. Concurrently, IgG antibody levels in patients declined markedly, with relatively few treatment-related adverse reactions [4].

This study is regarded as one of the most significant breakthroughs in the treatment of MG in recent years.

5. CLINICAL TECHNIQUES, GENE THERAPY, AND DISEASE MONITORING

5.1. Thymectomy

Studies have revealed that many patients with myasthenia gravis exhibit abnormal thymic changes, such as thymic hyperplasia or thymoma [3]. Abnormal thymus tissue may serve as a significant source of autoreactive T cells, thereby promoting the production of autoantibodies against neuromuscular junction proteins, including acetylcholine receptors. Consequently, thymectomy for abnormal thymic lesions is considered effective in mitigating aberrant immune responses at the pathogenic level [11].

To evaluate the therapeutic efficacy of thymectomy, Wolfe et al. (2016) conducted a randomized controlled clinical trial (the MGTX Study). The results demonstrated that patients who underwent thymectomy combined with pharmacotherapy exhibited superior outcomes in terms of symptom

improvement, glucocorticoid dosage requirements, and disease control compared to those who received only pharmacotherapy [11].

This study confirms the value of thymectomy in the treatment of myasthenia gravis and promotes its adoption as a key standard therapy for certain patients. Currently, thymectomy is widely recommended as a long-term treatment strategy [6].

5.2. Gene Therapy

With advancements in molecular biology and genetic technologies, gene therapy has emerged as a promising research direction in modern medicine. Its core principle involves modulating gene expression or correcting aberrant genes to achieve fundamental disease interventions.

Although myasthenia gravis is primarily classified as an autoimmune disorder rather than a typical monogenic genetic disease, its pathogenesis is closely associated with genetic susceptibility to multiple immune regulatory genes. Consequently, researchers have begun to explore the use of genetic technologies to modulate immune system function and mitigate abnormal autoimmune responses.

In recent years, advances in CRISPR-Cas9 gene editing technology have significantly propelled research in this field. This technology enables the precise modification of specific DNA sequences, paving the way for genetic-level interventions in disease management.

From a theoretical perspective, future research may leverage gene-editing technologies to achieve the following objectives:

First, it regulates key genes involved in the autoimmune response, thereby reducing the production of abnormal autoantibodies.

Second, it enhances the immune system's tolerance to its own antigens.

Thirdly, it promotes the repair and regeneration of damaged tissues at the neuromuscular junction.

Fourth, it enhances the ability of muscle cells to resist immune-mediated injury.

However, most relevant studies remain at the preclinical stage. Before gene therapy can be advanced toward clinical application, numerous challenges must be overcome, including editing precision, long-term safety, and related ethical considerations [1].

5.3. Biomarkers and Disease Surveillance

In addition to drug development, biochemical technologies are widely applied in disease diagnosis and therapeutic monitoring. With advancements in laboratory testing techniques, researchers can utilize various biomarkers to assess disease activity levels and treatment efficacy.

The most commonly used biomarkers currently include anti-acetylcholine receptor antibodies (AChR-Ab), anti-MuSK antibodies, and anti-LRP4 antibodies. These antibodies not only aid in disease diagnosis but also assist physicians in determining the patient's disease type and potential prognosis [9].

Furthermore, serum immunoglobulin levels, complement activation markers, and inflammation-related factors are increasingly being utilized to assess patients' response to treatment. With continuous advancements in detection technologies, more precise molecular biomarkers may emerge in the future, thereby enabling physicians to implement personalized therapy [3].

6. MAJOR CHALLENGES IN TREATMENT

Despite the continuous enrichment of treatment modalities in recent years, the management of myasthenia gravis still faces several challenges.

First, the vast majority of current treatment modalities can only control the disease rather than eliminate its root cause completely. Patients typically require long-term or even lifelong treatment [10].

Second, although biologics demonstrate significant therapeutic efficacy, their high cost limits widespread application. Additionally, the safety of long-term use of these novel drugs requires further investigation.

Third, significant individual variations exist among different patients. The same treatment approach may demonstrate remarkable efficacy in some patients but limited effectiveness in others [6].

7. FUTURE RESEARCH DIRECTIONS AND FEASIBILITY RECOMMENDATIONS

With the continuous advancements in bioengineering, biochemistry, and biomedical technologies, the treatment of myasthenia gravis has progressively shifted from traditional symptom control toward precision immunomodulation. However, current therapeutic approaches still fail to completely address the root cause of the disease, and most patients require long-term treatment. Therefore, exploring safer, more effective, and personalized treatment strategies will become a key focus of future research.

First, precision medicine is poised to become a pivotal trend in the future treatment of myasthenia gravis. Currently, clinical treatment plans are often formulated based on the severity of patients' symptoms; however, growing evidence indicates significant individual variations among patients. Future research can integrate genetic testing, immunological assays, and artificial intelligence analytics to achieve more precise patient classification, thereby enabling the development of personalized treatment strategies. This approach not only enhances therapeutic efficacy but also helps minimize unnecessary drug-related adverse effects.

Secondly, the development of novel targeted biopharmaceuticals still holds significant potential for advancement. Future research can further identify new immune regulatory targets, such as key molecules that modulate abnormal B-cell activation, inhibit autoantibody production, or promote the formation of immune tolerance. With advancements in antibody engineering and protein engineering technologies, scientists are expected to develop more precise and less side-effect-laden novel drugs, thereby further enhancing therapeutic efficacy.

Third, cell therapy may offer novel solutions for long-term management of myasthenia gravis. In recent years, mesenchymal stem cells (MSCs) have demonstrated significant immunomodulatory potential in research on various autoimmune diseases. Unlike conventional drugs that primarily suppress immune responses, stem cell therapy focuses more on restoring immune system homeostasis. Theoretically, if stem cells can be utilized to promote the generation of regulatory T cells (Tregs) and inhibit aberrant autoimmune responses, more durable disease remission may be achieved. Future studies are required to further evaluate the long-term safety, optimal administration methods, and stability of therapeutic efficacy of stem cell therapy to facilitate its clinical application.

Gene therapy is regarded as a highly promising research direction for the future, with gene editing technologies providing potential tools for regulating immune-related genes or enhancing the repair capacity of neuromuscular junctions. Artificial intelligence and big data technologies can employ machine learning algorithms to conduct in-depth analysis of patients' clinical data, enabling prediction of recurrence risks, evaluation of therapeutic efficacy, and optimization of treatment

strategies. Furthermore, there is an urgent need to strengthen research on the repair mechanisms of neuromuscular junctions, with efforts focused on developing biomaterials or tissue engineering approaches that promote their regeneration, thereby improving patients' long-term motor function.

8. CONCLUSION

This article provides a systematic review of the pathogenesis of myasthenia gravis (MG) and the advancements in modern biotechnology applications for its treatment. Myasthenia gravis is essentially an autoimmune disorder, with its core pathological mechanism involving the immune system mistakenly attacking critical proteins at the neuromuscular junction, leading to impaired neural signal transmission and ultimately resulting in symptoms such as muscle weakness and fatigue. With ongoing medical research, scientists' understanding of the disease mechanisms has expanded beyond mere neuromuscular dysfunction to encompass multiple aspects, including immune regulatory dysregulation, complement activation, and autoantibody-mediated damage.

In terms of treatment, traditional medications such as cholinesterase inhibitors, glucocorticoids, and immunosuppressants have played a significant role in alleviating symptoms and controlling disease progression; however, they still exhibit limitations, including pronounced long-term side effects, considerable variability in therapeutic efficacy among individuals, and an inability to achieve a cure. In recent years, advancements in bioengineering, biochemistry, and biomedicine have provided novel approaches for the treatment of myasthenia gravis. Targeted biologics, such as complement inhibitors and FcRn inhibitors, enable more precise intervention at key disease mechanisms, while stem cell therapy and gene therapy offer new possibilities for restoring immune homeostasis and achieving long-term disease control. These emerging technologies signify a progressive shift in myasthenia gravis management from conventional broad-spectrum immunosuppression toward precision medicine.

Nevertheless, most novel therapeutic approaches currently face challenges such as high treatment costs, insufficient long-term safety data, and limited clinical applicability. Moreover, the pathogenic mechanisms of these diseases have not been fully elucidated, and individual variations among patients further complicate the design of treatment regimens. Therefore, future research must continue to investigate the molecular mechanisms underlying these diseases and optimize existing therapeutic strategies.

Precision medicine, novel targeted drugs, cell therapy, gene therapy, and AI-assisted diagnosis and treatment all demonstrate promising development prospects. With continuous advancements in biotechnology and medical research, researchers are expected to develop safer, more effective, and personalized treatment strategies, thereby further improving patients' quality of life and advancing the management of myasthenia gravis from symptom control toward fundamental disease intervention. Although achieving a complete cure remains a long-term goal, the progress in modern biotechnology is providing an increasingly robust scientific foundation for this objective.

REFERENCES

[1] Doudna, J. A. (2020). The promise and challenge of therapeutic genome editing. Nature, 578(7794), 229–236. https://doi.org/10.1038/s41586-020-1978-5

[2] Gilhus, N. E. (2016). Myasthenia gravis. New England Journal of Medicine, 375(26), 2570–2581. https://doi.org/10.1056/NEJMra1602678

[3] Gilhus, N. E., Tzartos, S., Evoli, A., Palace, J., Burns, T. M., & Verschuuren, J. J. G. M. (2019). Myasthenia gravis. Nature Reviews Disease Primers, 5, 30. https://doi.org/10.1038/s41572-019-0079-y

[4] Howard, J. F., Jr., Bril, V., Vu, T., Karam, C., Peric, S., Margania, T., Murai, H., Bilinska, M., Shakarishvili, R., Smilowski, M., et al. (2021). Safety, efficacy, and tolerability of efgartigimod in patients with generalized myasthenia gravis (ADAPT): A multicenter, randomised, placebo-controlled, phase 3 trial. The Lancet Neurology, 20(7), 526–536. https://doi.org/10.1016/S1474-4422(21)00159-9

[5] Howard, J. F., Jr., Utsugisawa, K., Benatar, M., Murai, H., Barohn, R. J., Illa, I., Jacob, S., Vissing, J., Sommer, N., et al. (2017). Safety and efficacy of eculizumab in anti-acetylcholine receptor antibody-positive refractory generalized myasthenia gravis (REGAIN): A phase 3, randomised, double-blind, placebo-controlled, multicenter study. The Lancet Neurology, 16(12), 976–986. https://doi.org/10.1016/S1474-4422(17)30369-1

[6] Narayanaswami, P., Sanders, D. B., Wolfe, G., Benatar, M., Cea, G., Evoli, A., Gilhus, N. E., Illa, I., Kuntz, N., et al. (2021). International consensus guidance for management of myasthenia gravis: 2020 update. Neurology, 96(3), 114–122. https://doi.org/10.1212/WNL.0000000000011124

[7] Rosenblum, M. D., Remedios, K. A., & Abbas, A. K. (2015). Mechanisms of human autoimmunity. Journal of Clinical Investigation, 125(6), 2228–2233. https://doi.org/10.1172/JCI78088

[8] Sanders, D. B., Wolfe, G. I., Benatar, M., Evoli, A., Gilhus, N. E., Illa, I., Kuntz, N., Massey, J. M., Melms, A., et al. (2016). International consensus guidance for management of myasthenia gravis. Neurology, 87(4), 419–425. https://doi.org/1212/WNL.0000000000002790

[9] Tzartos, S. J., & Lazaridis, K. (2020). Myasthenia gravis: Autoantibody specificities and their role in MG management. Frontiers in Neurology, 11, 596981. https://doi.org/10.3389/fneur.2020.596981

[10] Verschuuren, J. J. G. M., Palace, J., Murai, H., Tannemaat, M. R., Kaminski, H. J., Bril, V., Cea, G., et al. (2022). Advances and ongoing research in the treatment of autoimmune myasthenia gravis. The Lancet Neurology, 21(2), 189–202. https://doi.org/10.1016/S1474-4422(21)00379-3

[11] Wolfe, G. I., Kaminski, H. J., Aban, I. B., Minisman, G., Kuo, H. C., Marx, A., Ströbel, P., Mazia, C., et al. (2016). Randomized trial of thymectomy in myasthenia gravis. New England Journal of Medicine, 375(6), 511–522. https://doi.org/10.1056/NEJMoa1602489

[12] Yi, J. S., Guptill, J. T., Stathopoulos, P., Nowak, R. J., O'Connor, K. C., & Hehir, M. K. (2022). B cells in the pathophysiology of myasthenia gravis. Muscle & Nerve, 66(1), 7–17. https://doi.org/10.1002/mus.27564