16 November 2026

Klygefa (gefurulimab) approved in the US for the treatment of generalised myasthenia gravis (gMG) in adult patients

Approval supported by PREVAIL Phase III results showing statistically significant and clinically meaningful improvements in activities of daily living starting as early as week one and sustained through one year

First and only approved dual-binding nanobody C5 inhibitor in the US, offering once-weekly subcutaneous self-administration via autoinjector with rapid and sustained disease control

Alexion, AstraZeneca Rare Disease's *Klygefa* (gefurulimab) has been approved in the US for the treatment of generalised myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody positive. *Klygefa* is the first and only dual-binding nanobody C5 inhibitor, offering once-weekly subcutaneous self-administration via autoinjector.

The approval by the US Food and Drug Administration (FDA) was based on positive results from the pivotal PREVAIL Phase III trial. Results from the trial showed that *Klygefa* met its primary endpoint, demonstrating improvement from baseline in Myasthenia Gravis Activities of Daily Living (MG-ADL) total score at week 26 compared to placebo (treatment difference: -1.6 [95% CI: -2.4, -0.8], p<0.0001). In addition to the week 26 prespecified results, clinical improvement was observed as early as week one.[1]

gMG is a rare autoimmune disorder characterised by reduced muscle function and severe muscle weakness.[2] In the US, an estimated 98,500 people are diagnosed with gMG with 81,000 being AChR-positive.[3]

Kelly Gwathmey, MD, associate professor in the Department of Neurology's Division of Neuromuscular Medicine at Columbia University Vagelos College of Physicians and Surgeons; member of the MGFA Medical & Scientific Advisory Council; and principal investigator in the trial at Virginia Commonwealth University, said: "gMG is a disease marked by unpredictability and variability, for which early intervention and reliable disease control are critical treatment goals. In the PREVAIL Phase III trial, gefurulimab achieved both, delivering rapid and sustained functional improvements with a once-weekly subcutaneous self-administered injection. With this approval, patients in the US gain a targeted treatment option that empowers greater autonomy with the established efficacy of C5 complement inhibition."

Samantha Masterson, President & Chief Executive Officer, Myasthenia Gravis Foundation of America (MGFA), said: "For the gMG community, every new approved treatment represents more than a clinical advance; it also represents choice. Because no two patients experience gMG in the same way, expanding the range of options available is fundamental to how we improve lives. The MGFA welcomes this approval and the new possibilities it opens for those who have been seeking more independence and greater control of their treatment administration."

Marc Dunoyer, Chief Executive Officer, Alexion, said: "The US approval of *Klygefa* delivers a first-of-its-kind treatment for gMG that offers the efficacy of C5 inhibition with the flexibility of once-weekly subcutaneous self-administration via autoinjector. We are proud to have discovered and developed this novel dual-binding nanobody, and look forward to soon

delivering this pioneering innovation to adults with gMG in the US, offering the established efficacy and safety of C5 inhibition with added convenience."

Klygefa was generally well-tolerated, and the safety profile was consistent with previous trials of C5 inhibitors eculizumab and ravulizumab in gMG. The percentage of participants with treatment emergent adverse events (TEAEs) was similar between those who received *Klygefa* and placebo, and most TEAEs observed were mild to moderate in severity (grade 1 or 2). The most common TEAEs seen in those receiving *Klygefa* were injection site reactions (12%), back pain (8%) and vomiting (5%).[1]

The data from the PREVAIL trial were presented at the Myasthenia Gravis Foundation of America (MGFA) Scientific Session during the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) 2025 Annual Meeting and published in *JAMA Neurology*.[1]

Klygefa is approved in the European Union (EU), Japan and other countries for certain adults with gMG. Regulatory submissions based on the PREVAIL results are under review in China and additional countries.

Notes

Generalised Myasthenia Gravis (gMG)

Generalised myasthenia gravis (gMG) is a rare autoimmune disorder characterised by reduced muscle function and severe muscle weakness.[2]

Eighty-five percent of people with gMG are AChR antibody-positive meaning they produce specific antibodies (anti-AChR) that bind to signal receptors at the neuromuscular junction (NMJ), the connection point between nerve cells and the muscles they control.[4] This binding activates the complement system, causing the immune system to attack the NMJ, leading to inflammation and a breakdown in communication between the brain and the muscles.[5]

gMG can occur at any age, but it most commonly begins for women before the age of 40 and for men after the age of 60.[6] Initial symptoms may include slurred speech, double vision, droopy eyelids and lack of balance; these can often lead to more severe symptoms as the disease progresses, such as impaired swallowing, choking, extreme fatigue and respiratory failure.[7,8]

PREVAIL (ALXN1720-MG-301)

PREVAIL (ALXN1720-MG-301) is a global, Phase III, randomised, double-blind, placebo-controlled, parallel, multicentre study evaluating the safety and efficacy of *Klygefa* (gefurulimab) in adults with generalised myasthenia gravis (gMG). The trial enrolled 260 patients from 20 countries across North America, Europe, Asia and the Pacific region. Participants were required to have a confirmed myasthenia gravis diagnosis at least three months prior to the screening visit with a positive serological test for autoantibodies against AChR and Myasthenia Gravis Foundation of America Clinical Classification Class II to IV at screening.[9]

Patients were randomised 1:1 to receive *Klygefa* or placebo for a total of 26 weeks in the randomised controlled treatment period. Patients received a single weight-based loading dose on Day 1, followed by regular weight-based maintenance dosing beginning on Day 8 and once every week thereafter. The primary endpoint of the change from baseline in the Myasthenia Gravis Activities of Daily Living (MG-ADL) total score, a patient-reported scale that assesses patients' abilities to perform daily activities, was assessed at week 26 along with multiple secondary endpoints evaluating improvement in disease-related measures.[9]

Patients who completed the randomised controlled treatment period were eligible to continue into an open-label extension period evaluating the safety and efficacy of *Klygefa*, which is ongoing.[9]

***Klygefa* (gefurulimab)**
Klygefa (gefurulimab), a complement C5 inhibitor, is a novel dual-binding nanobody optimised for subcutaneous self-administration for the treatment of AChR-Ab+ gMG. The medication works by binding to the C5 protein in the terminal complement cascade, a part of the body's immune system. When activated in an uncontrolled manner, the complement cascade over-responds, leading the body to attack its own healthy cells. *Klygefa's* concurrent binding to serum albumin provides an extended half-life, enabling once-weekly dosing.

Klygefa is approved in the European Union (EU), Japan and other countries for certain adults with gMG. Regulatory submissions based on the PREVAIL results are under review in China and additional countries.

Alexion
Alexion, AstraZeneca Rare Disease, is focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and delivery of life-changing medicines. A pioneering leader in rare disease for more than three decades, Alexion was the first to translate the complex biology of the complement system into transformative medicines, and today it continues to build a diversified pipeline across disease areas with significant unmet need, using an array of innovative modalities. As part of AstraZeneca, Alexion is continually expanding its global geographic footprint to serve more rare disease patients around the world. It is headquartered in Boston, US.

AstraZeneca
AstraZeneca (LSE/STO/NYSE: AZN) is a global, science-led biopharmaceutical company that focuses on the discovery, development, and commercialisation of prescription medicines in Oncology, Rare Disease, and BioPharmaceuticals, including Cardiovascular, Renal & Metabolism, and Respiratory & Immunology. Based in Cambridge, UK, AstraZeneca's innovative medicines are sold in more than 125 countries and used by millions of patients worldwide. Please visit astrazeneca.com and follow the Company on Social Media @AstraZeneca.

Contacts
For details on how to contact the Investor Relations Team, please click here. For Media contacts, click here.

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