21 September 2026

Klygefa (gefurulimab) recommended for approval in the EU by CHMP for the treatment of adults with generalised myasthenia gravis (gMG)

Recommendation based on PREVAIL Phase III trial demonstrating statistically significant and clinically meaningful improvement in Myasthenia Gravis Activities of Daily Living score

If approved, Klygefa will be the first and only dual-binding nanobody C5 inhibitor for gMG in the EU, offering once weekly subcutaneous self-administration with rapid and sustained symptom control

Alexion, AstraZeneca Rare Disease's *Klygefa* (gefurulimab) has been recommended for approval in the European Union (EU) as an add-on to standard therapy for the treatment of generalised myasthenia gravis (gMG) in adults who are anti-acetylcholine receptor (AChR) antibody-positive. If approved, *Klygefa* will be the first and only dual-binding nanobody C5 inhibitor for this patient population.

The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) based its positive opinion on results from the pivotal PREVAIL Phase III trial, which 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]

gMG is a rare autoimmune disorder characterised by reduced muscle function and severe muscle weakness.[2] An estimated 82,500 people are diagnosed with gMG in Europe 5 countries (Germany, France, UK, Italy and Spain) with 66,000 being AChR-positive.[3]

Tobias Ruck, MD, Director Department of Neurology, BG University Hospital Bergmannsheil Bochum, Ruhr-University Bochum and investigator in the trial, said: "For people living with gMG, unpredictable symptoms can quickly become incapacitating or life-threatening. In the PREVAIL Phase III trial, gefurulimab demonstrated the ability to improve measures of disease severity and daily function with the convenience of once weekly subcutaneous self-administration, as early as one week and through the 26-week study period. With this positive CHMP opinion patients may soon have the option of a novel treatment option that could help them spend less time thinking about their care and more time living their lives."

Marc Dunoyer, Chief Executive Officer, Alexion, said: "This positive CHMP opinion is an important step towards bringing *Klygefa*, an innovative dual-binding nanobody C5 inhibitor, to people living with gMG in the EU. Building on our pioneering work demonstrating the efficacy of C5 inhibition with *Soliris* and *Ultomiris*, *Klygefa* is designed to offer rapid and sustained symptom control with convenient once-weekly subcutaneous self-administration via autoinjector."

Results from the PREVAIL 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). A clinically meaningful improvement was observed as early as week one, and was sustained through week 26.[1]

Klygefa was generally well-tolerated, and the safety profile was consistent with previous trials of C5 inhibitors eculizumab and ravulizumab in gMG.[1]

Klygefa is approved in Japan and other countries for certain adults with gMG. Regulatory submissions based on the PREVAIL results are under review in the US, 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* 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 in development as a treatment for 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 Japan and other countries for certain adults with gMG. Regulatory submissions based on the PREVAIL results are under review in the US, 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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Matthew Bowden

Company Secretary

AstraZeneca PLC

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