NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Efgartigimod for treating antibody-positive generalised myasthenia gravis (review of TA1069)

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of efgartigimod within its marketing authorisation for treating antibody-positive generalised myasthenia gravis (review of TA1069).

Background

Myasthenia gravis is a long-term condition which causes certain muscles to become weak and tire easily. It is caused by a problem with the immune system, which mistakenly produces antibodies against the nicotinic acetylcholine receptor (AChR) or muscle-specific tyrosine kinase (MuSK). The antibodies block the chemical signals between nerves and muscles, meaning that muscles are unable to tighten (contract). The thymus gland is the main source of the abnormal antibodies. The muscles around the eyes are commonly affected first, which causes drooping of the eyelid and double vision. Muscles controlling facial expression, chewing, swallowing, speaking and, less commonly, breathing and neck and limb movements can also be affected. When muscle groups other than the eye muscles are affected, the condition is known as generalised myasthenia gravis. In very severe cases, muscle weakness causes life-threatening difficulties with breathing and swallowing. This is known as myasthenic crisis.

Myasthenia gravis affects about 15 in every 100,000 people in the UK.[1,2] It can develop at any age, but most commonly affects women under 40 and men over 60.[3] Around 80% of people with myasthenia gravis will progress to generalised myasthenia gravis within 2 years.[2] About 80% to 90% of people with myasthenia gravis have detectable antibodies against AchR, while 3% to 7% have antibodies against MuSK.[4-6] In around 10% of people antibodies are not detected.[7]

Myasthenia gravis severity can be classified according to the Myasthenia Gravis Foundation of America (MGFA) class.[8] Mild myasthenia gravis is usually treated with anticholinesterases (such as pyridostigmine or, less commonly, neostigmine) which delay the breakdown of acetylcholine, the chemical which stimulates muscle contraction.[9] If treatment with anticholinesterases is not effective, or they are not suitable for long term use, then corticosteroid tablets such as prednisolone are used. Immunosuppressive therapies such as azathioprine are offered in addition to corticosteroids, with the aim of reducing the corticosteroid dose over time. If the condition does not respond to the first immunosuppressive treatment, alternative immunosuppressants may be offered (including mycophenolate mofetil, methotrexate, ciclosporin and rituximab). There will be some people who have ongoing disabling symptoms despite optimal immunosuppression. Surgery to remove the thymus gland may be an option for some people. Some people with symptoms despite immunosuppressive therapies may receive maintenance treatment with intravenous injections of antibodies (immunoglobulins) from healthy donor blood or have plasma exchange. Plasma exchange involves removing the plasma from the

blood to reduce the number of abnormal antibodies. Myasthenic crisis is treated in hospital with intravenous immunoglobulins or with plasma exchange.
NICE technology appraisal guidance 1155 recommends rozanolixizumab, as an add-on to standard treatment, as an option for treating generalised myasthenia gravis in adults who test positive for anti- AChR or anti- MuSK antibodies only if:

- the condition is classified as MGFA class 2 to 4a
- the condition is uncontrolled after 2 or more treatments, excluding acetylcholinesterase inhibitors, and
- intravenous immunoglobulin or plasma exchange would otherwise be offered or has been tried and stopped because of side effects or because it did not work well enough.

In 2025, NICE evaluated efgartigimod as an add-on to standard treatment for generalised myasthenia gravis in adults who test positive for anti- AChR, but it was not recommended (NICE technology appraisal guidance 1069). Following the recommendation of rozanolixizumab (NICE technology appraisal guidance 1155), the manufacturer requested a review of the guidance. This scope is a review of TA1069 and will appraise the clinical and cost effectiveness of efgartigimod within its marketing authorisation for treating anti-acetylcholine receptor antibody positive generalised myasthenia gravis.

The technology

Efgartigimod (Vyvgart, argenx) is indicated as an add-on to standard therapy for the treatment of adult patients with generalised myasthenia gravis who are anti-acetylcholine receptor antibody positive. Efgartigimod can be administered by subcutaneous injection, prefilled syringe, or intravenously.

Intervention(s)	Efgartigimod
Population(s)	Adults with anti-acetylcholine receptor antibody positive generalised myasthenia gravis
Subgroups	People with myasthenia gravis that is refractory to 2 or more treatments excluding anticholinesterase inhibitors and in whom intravenous immunoglobulin or plasma exchange would be considered
Comparators	• established clinical management without efgartigimod including corticosteroids and immunosuppressive therapies, with or without intravenous immunoglobulin or plasma exchange. This should be considered as a standard of care basket, reflecting the proportion of people who have corticosteroids and non-steroidal immunosuppressive therapy, intravenous immunoglobulin, and plasma exchange in clinical practice. • rozanolixizumab • zilucoplan (subject to NICE evaluation)

Outcomes	The outcome measures to be considered include: • improvement in myasthenia gravis • time to clinically meaningful improvement • time on treatment • need for retreatment • mortality • number and duration of hospitalisations • adverse effects of treatment • health-related quality of life.
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. If the technology is likely to provide similar or greater health benefits at similar or lower cost than technologies recommended in published NICE technology appraisal guidance for the same indication, a cost comparison may be carried out. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	**Related technology appraisals:** Rozanolixizumab for treating antibody-positive generalised myasthenia gravis (2026) NICE technology appraisal guidance TA1155. Efgartigimod for treating antibody-positive generalised myasthenia gravis (2025) NICE technology appraisal guidance TA1069. **Related technology appraisals in development:**

	Zilucoplan for treating antibody positive generalised myasthenia gravis. NICE technology appraisal guidance [ID4008] Publication to be confirmed. **Related NICE guidelines:** Suspected neurological conditions: recognition and referral (2023). NICE guideline 127. **Related quality standards:** Suspected neurological conditions: recognition and referral (2021). NICE quality standard 198.

Questions for consultation

The company has requested that this topic goes through a cost comparison process (versus a single technology appraisal). For this to be considered, do stakeholders consider that efgartigimod would be appropriately positioned as an alternative to rozanolixizumab in a refractory population:

Please provide comments on the appropriateness of appraising this topic through this process.
(Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

Technologies can be evaluated through the cost-comparison process if they are expected to provide similar or greater health benefits, at a similar or lower cost, compared with technologies that have been previously recommended (as an option) in published NICE guidance for the same indication. Companies can propose cost-comparison topics to NICE at any stage during topic selection and scoping. NICE will route technologies for evaluation through the cost-comparison process if it is agreed during scoping that the process is an appropriate route to establish the clinical and cost effectiveness of the technology.

NICE's health technology evaluations: the manual states the methods to be used where a cost comparison case is made.

- Is the technology likely to be similar in its clinical effectiveness and resource use to any of rozanolixizumab? Or in what way is it different to rozanolixizumab?
- Will the intervention be used in the same place in the treatment pathway as rozanolixizumab? Have there been any major changes to the treatment pathway recently? If so, please describe.
- Will the intervention be used to treat the same population as rozanolixizumab specified in the NICE guidance for rozanolixizumab?

- Overall is the technology likely to offer similar or improved health benefits compared with rozanolixizumab

- Would it be appropriate to use the cost-comparison methodology for this topic? Is there anything else that may not be captured by this approach?

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which efgartigimod is licensed

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

Please select from the following, will efgartigimod be:
A. Prescribed in primary care with routine follow-up in primary care
B. Prescribed in secondary care with routine follow-up in primary care
C. Prescribed in secondary care with routine follow-up in secondary care
D. Other (please give details):
For comparators and subsequent treatments, please detail if the setting for prescribing and routine follow-up differs from the intervention.

References

1. Spillane J, Higham E, Kullmann DM (2012) Myasthenia gravis. *BMJ*; 345:e8497.

2. Patient (2022) Myasthenia Gravis. Accessed June 2026.

3. Meriggioli MN and Sanders DB (2009) Autoimmune myasthenia gravis: emerging clinical and biological heterogeneity. *Lancet Neurology*; 8(5):475-90.

4. Guptill JT and Sanders DB (2010) Update on muscle-specific tyrosine kinase antibody positive myasthenia gravis. *Current Opinion Neurology*; 23(5):530-5.

5. Ruff RL and Lisak RP (2018) Nature and action of antibodies in myasthenia gravis. *Neurologic Clinics*; 36(2):275-91.

6. Maddison P, Ambrose PA, Sadalage G et al. (2019) A Prospective Study of the Incidence of Myasthenia Gravis in the East Midlands of England. *Neuroepidemiology*; 53(1-2):93-99.

7. Leite M, Jacob S, Viegas S et al. (2008) IgG1 antibodies to acetylcholine receptors in 'seronegative' myasthenia gravis. *Brain* 131:1940-52

8. Myasthenia gravis hope foundation: Classification of myasthenia gravis. Accessed June 2026

9. BMJ Best Practice (2025) Myasthenia gravis. Accessed June 2026.