

Health Technology Briefing

January 2026

Nipocalimab for treating generalised myasthenia gravis in patients aged 2 to less than 18 years old

Company/Developer

Janssen-Cilag Limited (a Johnson & Johnson company)

☒ New Active Substance

☐ Significant Licence Extension (SLE)

NIHRO ID: 34875

NICE ID: Not Available

UKPS ID: 674898

Licensing and Market Availability Plans

Currently in phase III clinical trials.

Summary

Nipocalimab is being developed as a treatment for generalised myasthenia gravis (gMG) in children. This condition, also known as juvenile myasthenia gravis in children, is a rare autoimmune disorder that causes muscle weakness and fatigue. It happens when the immune system mistakenly attacks the connection between nerves and muscles. This can affect the eyes, face, limbs, and throat, causing drooping eyelids, double vision, difficulty speaking or swallowing, and problems with movement or breathing. Symptoms can vary over time, with periods of worsening called flare-ups. Current treatments for young children are limited, and some can cause side effects. There is a need for treatments that target the underlying cause of the disease safely and effectively.

Nipocalimab is a laboratory-made antibody given by intravenous infusion (injected into the vein). It works by blocking a protein called FcRn, which normally helps the body keep antibodies in the blood. In gMG, harmful antibodies attack the nerve-muscle connection. Blocking FcRn helps the body remove these antibodies more quickly, which may reduce muscle weakness and improve physical function. Nipocalimab is innovative because it directly targets the process that produces harmful antibodies. If licensed, it could provide an additional treatment option for children whose gMG is not well controlled with current therapies.

Proposed Indication

Treatment of gMG in children and adolescents who have insufficient clinical response to ongoing, stable, standard-of-care therapy.¹

Technology

Description

Nipocalimab (JNJ-80202135, M281, Imaavy) is a fully human, aglycosylated IgG1 (a subclass of immunoglobulin G) monoclonal antibody designed to selectively bind to and block the IgG binding site on the neonatal Fc receptor (FcRn).^{2,3} FcRn plays a critical role in prolonging the half-life of IgG antibodies by protecting them from lysosomal degradation. By inhibiting the interaction between FcRn and IgG, nipocalimab accelerates the degradation of circulating IgG antibodies, including pathogenic autoantibodies.⁴ In gMG, IgG autoantibodies target components of the neuromuscular junction, thus the mechanism of nipocalimab is intended to reduce circulating levels of these autoantibodies, and therefore alleviate gMG symptoms.²

Nipocalimab is in clinical development for the treatment of gMG in children and adolescent patients. In the phase 2/3 clinical trial (NCT05265273), participants receive nipocalimab through intravenous infusion.¹

Key Innovation

Current therapies, such as glucocorticoids and immunosuppressive drugs, may be effective in reducing pathogenic IgG antibody production, but they are broadly immunosuppressive and frequently limited by toxicities.⁵ Nipocalimab represents a novel treatment approach for gMG by targeting FcRn. FcRn normally recycles IgG antibodies, including the harmful autoantibodies that drive gMG symptoms. Nipocalimab binds to FcRn with high affinity and specificity, blocking this recycling process and allowing the body to break down and eliminate IgG autoantibodies more rapidly.² Blockade of FcRn has the potential to reduce overall autoantibody levels while preserving immune function without causing broad immunosuppression.⁶

Regulatory & Development Status

Nipocalimab does not currently have marketing authorisation in the EU/UK for any indication, including for paediatric populations.

Nipocalimab is currently in phase 3 clinical development for the following indications for adult patients:⁷

- Sjogren's syndrome
- In pregnancies at risk of foetal and neonatal alloimmune thrombocytopenia
- In pregnancies at risk of severe haemolytic disease of the foetus and newborn
- Chronic inflammatory demyelinating polyneuropathy
- Warm autoimmune haemolytic anaemia

Patient Group

Disease Area and Clinical Need

gMG is an antibody-mediated disorder of the neuromuscular junction that affects both children and adults. It typically is a lifelong condition, requiring ongoing treatment, though it may go in and out of remission. Broadly, symptoms include weakness in the muscles of the eyes, eyelids, and face, causing droopy eyelids in one or both eyes, double vision, and difficulty making facial expressions. This weakness can also affect the muscles of the mouth, throat, and chest, causing difficulty chewing and swallowing, slurred speech, a husky, quiet, or nasal-sounding voice, accidental choking, and shortness of breath.⁸ In children, indicators of gMG include tiring easily while playing, and appearing uncoordinated.⁹ Symptoms range from mild to severe, worsen with activity and can sometimes improve with rest.¹⁰ Although gMG is considered treatable, many patients require long-term immunosuppression to achieve disease control or remission.

In England, the prevalence of juvenile myasthenia gravis was 2.5 per 100,000 in 2018.¹¹ In England in 2024-2025, there were 7596 finished consultant episodes (FCE) and 5785 admissions for gMG and other myoneural disorders (ICD-10 code G70.0) which resulted in 22,121 FCE bed days and 4102 day cases.¹²

Recommended Treatment Options

There is currently no treatment option recommended by NICE specifically for gMG in children and adolescents. However, current treatments for paediatric gMG include:¹³

- Acetylcholinesterase inhibitors
- Intravenous immunoglobulin
- Immunosuppressants, including corticosteroids, mycophenolate mofetil, and rituximab

Clinical Trial Information

Trial	NCT05265273 ; 2021-002479-20 ; An Open-Label Uncontrolled Multicenter Study to Evaluate the Pharmacokinetics, Pharmacodynamics, Safety and Activity of Nipocalimab in Children Aged 2 to Less Than 18 Years With Generalized Myasthenia Gravis Phase II/III – active, recruiting Location(s) : 2 EU countries, USA and Japan Primary completion date : June 2026
Trial Design	Allocation, single group assignment, open label
Population	N=12 (estimated); Participants with generalised myasthenia gravis; aged 2-17 years
Intervention(s)	Nipocalimab administered as intravenous infusion
Comparator(s)	No comparator
Outcome(s)	Primary outcome measure: change from baseline in total serum IgG levels [time frame: up to 3 years] See trial record for list of other outcomes
Results (efficacy)	-
Results (safety)	-
Trial	NCT04951622 ; 2020-005732-29 ; Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy, Safety,

	Pharmacokinetics, and Pharmacodynamics of Nipocalimab Administered to Adults With Generalized Myasthenia Gravis Phase III – active, not recruiting Location(s) : 8 EU countries, USA and other countries Primary completion date : November 2023
Trial Design	Allocation, parallel assignment, triple blind
Population	N=219 (actual); participants with generalised myasthenia gravis; adults aged 18 years and older
Intervention(s)	Nipocalimab administered as intravenous infusion
Comparator(s)	Matched placebo
Outcome(s)	Primary outcome measure: Double-blind (DB) phase: average change from baseline in Myasthenia Gravis - Activities of Daily Living (MG-ADL) total score over weeks 22, 23, and 24 [time frame: baseline, weeks 22, 23, and 24] See trial record for list of other outcomes.
Results (efficacy)	Between July 15, 2021, and Nov 17, 2023, 199 patients were enrolled, and 196 patients received study drug (98 in the nipocalimab group and 98 in the placebo group); of these, 153 (77 in the nipocalimab group and 76 in the placebo group) were antibody-positive. The least-squares mean change in MG-ADL score from baseline to weeks 22, 23, and 24 was -4.70 (SE 0.329) in the nipocalimab group versus -3.25 (0.335) in the placebo group (difference -1.45 [95% CI -2.38 to -0.52]; p=0.0024). ²
Results (safety)	The incidence of adverse events was similar between groups (82 [84%] of 98 in both the nipocalimab and placebo groups), including infections (42 [43%] of 98 in the nipocalimab group and placebo group) and headache (14 [14%] of 98 in the nipocalimab group and 17 [17%] of 98 in the placebo group). Serious adverse events were reported for nine (9%) of 98 patients in the nipocalimab group and 14 (14%) of 98 patients in the placebo group, three of which had a fatal outcome (nipocalimab: myasthenic crisis; placebo: cardiac arrest and myocardial infarction). ²

Estimated Cost

The cost of nipocalimab is not yet known.

Relevant Guidance

NICE Guidance

There is no treatment option recommended by NICE for treatment of gMG in children and adolescents.

NHS England (Policy/Commissioning) Guidance

Other Guidance

- Narayanaswami P, Sanders DB, Wolfe G, Benatar M, Cea G, Evoli A, et al. International Consensus Guidance for Management of Myasthenia Gravis. 2021.¹⁴
- Munot P, Robb SA, Niks EH, Palace J, Palace J, et al. 242nd ENMC International Workshop: Diagnosis and management of juvenile myasthenia gravis. 2020.¹⁵

References

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