Johnson&Johnson

NEWS RELEASE

Nipocalimab granted U.S. FDA Priority Review for the treatment of generalized myasthenia gravis

2025-01-09

Biologics License Application acceptance supported by results from the Phase 3 Vivacity-MG3 study

Results demonstrate sustained disease control over 24 weeks in a broad population of antibody positive adult patients: anti-AChR, anti-MuSK, anti-LRP4

SPRING HOUSE, Pa., Jan. 9, 2025 /PRNewswire/ -- Johnson & Johnson (NYSE: JNJ) today announced the nipocalimab Biologics License Application (BLA) received Priority Review designation from the U.S Food and Drug Administration (FDA) for the treatment of antibody positive (anti-AChR, anti-MuSK, anti-LRP4) patients with generalized myasthenia gravis (gMG), as supported by findings from the Phase 3 Vivacity-MG3 study. The FDA grants Priority Review to applications for medicines that, if approved, would offer significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications.[1]

"We welcome the FDA's decision to grant Priority Review for the treatment of generalized myasthenia gravis, which underscores the need for additional treatment options in a broad population of people living with gMG," said Katie Abouzahr, M.D., Vice President, Autoantibody Portfolio and Maternal Fetal Immunology Disease Area Leader at Johnson & Johnson Innovative Medicine. "We are committed to working closely with the FDA to help bring nipocalimab as a potential treatment to certain patients living with gMG, and we especially thank the participants in the Phase 2 and 3 studies. If approved, nipocalimab has the potential to treat gMG in antibody positive individuals, including anti-AChR, anti-MuSK, and/or anti-LRP4."

gMG is a chronic, life-long, rare, autoantibody-driven disease, for which no cure is currently available.[2,3] gMG impacts an estimated 700,000 people worldwide.[2,3] In the Phase 3 study, nipocalimab plus standard of care (SOC)

demonstrated a significantly greater reduction in MG-ADL response (≥2-point improvement from baseline) compared with placebo plus SOC (p=0.0213).[4] For someone living with gMG, a 1- to 2-point change on MG-ADL may be the difference between normal eating and frequent choking on food, or shortness of breath at rest and being on a ventilator.[5]

Johnson & Johnson also submitted a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) seeking approval of nipocalimab in gMG on September 11, 2024.[6] In addition, nipocalimab recently received U.S. FDA Breakthrough Therapy Designation for the treatment of adults with moderate-to-severe Sjögren's disease as supported by results from the Phase 2 DAHLIAS study.[7]

Editor's notes:

---

a. MG-ADL (Myasthenia Gravis – Activities of Daily Living) provides a rapid clinical assessment of the patient's recall of symptoms impacting activities of daily living, with a total score range of 0 to 24; a higher score indicates greater symptom severity.[5]

ABOUT GENERALIZED MYASTHENIA GRAVIS (gMG)

Myasthenia gravis (MG) is an autoantibody disease in which the immune system mistakenly makes antibodies (e.g., anti-acetylcholine receptor [AChR], anti-muscle-specific tyrosine kinase [MuSK] or anti-low density lipoprotein-related protein 4 [LRP4]), which target proteins at the neuromuscular junction and can block or disrupt normal signaling from nerves to muscles, thus impairing or preventing muscle contraction.[2,8,9] The disease impacts an estimated 700,000 people worldwide.[2] Approximately 10 to 15% of new cases of MG are diagnosed in adolescents (12 – 17 years of age).[10,11,12] Among juvenile MG patients, girls are affected more often than boys with over 65% of pediatric MG cases in the US diagnosed in girls.[13,14,15]

Initial disease manifestations are usually ocular but in 85% or more cases, the disease generalizes (gMG), which is characterized by fluctuating weakness of the skeletal muscles leading to symptoms like limb weakness, drooping eyelids, double vision and difficulties with chewing, swallowing, speech, and breathing.[16,17,18,19] Approximately 100,000 individuals in the U.S. are living with gMG.[20] Vulnerable gMG populations, such as pediatric patients, have more limited therapeutic options.[21] Currently, SOC treatments for adolescents with gMG are extrapolated from adult trials.[12] Other than symptomatic treatments, there are no approved FcRn blockers for adolescents with gMG in the United States.[12]

ABOUT THE PHASE 3 VIVACITY-MG3 STUDY

The Phase 3 Vivacity-MG3 study (**NCT04951622**) was specifically designed to measure sustained efficacy and safety with consistent dosing in this unpredictable chronic condition where unmet need remains high. Antibody positive or negative adult gMG patients with insufficient response (MG-ADL ≥6) to ongoing standard of care (SOC) therapy were identified and 199 patients, 153 of whom were antibody positive, enrolled in the 24-week double-blind placebo-controlled trial.[4,22] Randomisation was 1:1, nipocalimab plus current SOC (30 mg/kg IV loading dose followed by 15 mg/kg every two weeks) or placebo plus current SOC.[4] Baseline demographics were balanced across arms (77 nipocalimab, 76 placebo).[4] The primary endpoint of the study was mean change in MG-ADL[a] score from baseline over Weeks 22, 23 and 24 in antibody positive patients. A key secondary endpoint included change in QMG score. Long-term safety and efficacy were further assessed in an ongoing open-label extension (OLE) phase.[22]

ABOUT NIPOCALIMAB

Nipocalimab is an investigational monoclonal antibody, designed to bind with high affinity to block FcRn and reduce levels of circulating immunoglobulin G (IgG) antibodies potentially without impact on other immune functions. This includes autoantibodies and alloantibodies that underlie multiple conditions across three key segments in the autoantibody space including Rare Autoantibody diseases, Maternal Fetal diseases mediated by maternal alloantibodies and Rheumatology.[22, 23, 24,25,26,27,28,29,30] Blockade of IgG binding to FcRn in the placenta is also believed to limit transplacental transfer of maternal alloantibodies to the fetus.[31,32]

The FDA and European Medicines Agency (EMA) have granted several key designations to nipocalimab including:

- U.S. FDA Fast Track designation in hemolytic disease of the fetus and newborn (HDFN) and warm autoimmune hemolytic anemia (wAIHA) in July 2019, gMG in December 2021 and fetal neonatal alloimmune thrombocytopenia (FNAIT) in March 2024
- U.S. FDA Orphan drug status for wAIHA in December 2019, HDFN in June 2020, gMG in February 2021, chronic inflammatory demyelinating polyneuropathy (CIDP) in October 2021 and FNAIT in December 2023
- U.S. FDA Breakthrough Therapy designation for HDFN in February 2024 and for Sjögren's disease in November 2024
- U.S. FDA granted Priority Review in gMG in Q4 2024
- EU EMA Orphan medicinal product designation for HDFN in October 2019

ABOUT JOHNSON & JOHNSON

At Johnson & Johnson, we believe health is everything. Our strength in healthcare innovation empowers us to build a world where complex diseases are prevented, treated, and cured, where treatments are smarter and less invasive, and solutions are personal. Through our expertise in Innovative Medicine and MedTech, we are uniquely positioned to innovate across the full spectrum of healthcare solutions today to deliver the breakthroughs of

tomorrow, and profoundly impact health for humanity.

Learn more at **https://www.jnj.com/** or at **www.innovativemedicine.jnj.com**

Follow us at **@JNJInnovMed**.

Janssen Research & Development, LLC and Janssen Biotech, Inc. are Johnson & Johnson companies.

CAUTIONS CONCERNING FORWARD-LOOKING STATEMENTS

This press release contains "forward-looking statements" as defined in the Private Securities Litigation Reform Act of 1995 regarding product development and the potential benefits and treatment impact of nipocalimab. The reader is cautioned not to rely on these forward-looking statements. These statements are based on current expectations of future events. If underlying assumptions prove inaccurate or known or unknown risks or uncertainties materialize, actual results could vary materially from the expectations and projections of Janssen Research & Development, LLC, Janssen Biotech, Inc. and/or Johnson & Johnson. Risks and uncertainties include, but are not limited to: challenges and uncertainties inherent in product research and development, including the uncertainty of clinical success and of obtaining regulatory approvals; uncertainty of commercial success; manufacturing difficulties and delays; competition, including technological advances, new products and patents attained by competitors; challenges to patents; product efficacy or safety concerns resulting in product recalls or regulatory action; changes in behavior and spending patterns of purchasers of health care products and services; changes to applicable laws and regulations, including global health care reforms; and trends toward health care cost containment. A further list and descriptions of these risks, uncertainties and other factors can be found in Johnson & Johnson's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, including in the sections captioned "Cautionary Note Regarding Forward-Looking Statements" and "Item 1A. Risk Factors," and in Johnson & Johnson's subsequent Quarterly Reports on Form 10-Q and other filings with the Securities and Exchange Commission. Copies of these filings are available online at **www.sec.gov**, www.jnj.com or on request from Johnson & Johnson. None of Janssen Research & Development, LLC, Janssen Biotech, Inc. nor Johnson & Johnson undertakes to update any forward-looking statement as a result of new information or future events or developments.

References

---

[1] U.S. Food & Drug Administration. Priority Review. Available at: **https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review**. Last accessed: November 2024.
[2] Chen J, Tian D-C, Zhang C, et al. Incidence, mortality, and economic burden of myasthenia gravis in China: A nationwide population-based study.

The Lancet Regional Health - Western Pacific. 2020;5(100063). **https://doi.org/10.1016/j.lanwpc.2020.100063**.
[3] National Institute of Neurological Disorders and Stoke. Myasthenia Gravis. Available at: **https://www.ninds.nih.gov/health-information/disorders/myasthenia-gravis**. Last accessed: November 2024.
[4] Antozzi, C et al., Efficacy and Safety of Nipocalimab in patients with Generalized Myasthenia Gravis- Top Line Results from the Double-Blind, Placebo-Controlled, Randomized Phase 3 Vivacity-MG3 study. 2024 European Academy of Neurology Congress. June 2024.
[5] Wolfe GI Myasthenia gravis activities of daily living profile. Neurology. 1999;22;52(7):1487-9. doi: 10.1212/wnl.52.7.1487.
[6] Johnson & Johnson EMEA. Johnson & Johnson seeks first EU approval of nipocalimab to treat a broad population of patients living with antibody-positive generalised myasthenia gravis. Available at: **https://innovativemedicine.jnj.com/emea/newsroom/immunology/johnson-johnson-seeks-first-eu-approval-of-nipocalimab-to-treat-a-broad-population-of-patients-living-with-antibody-positive-generalised-myasthenia-gravis**. Last accessed: November 2024.
[7] Johnson & Johnson. Nipocalimab is the first and only investigational therapy granted U.S. FDA Breakthrough Therapy Designation for the treatment of adults living with moderate-to-severe Sjögren's disease. Available at: **https://www.jnj.com/media-center/press-releases/nipocalimab-is-the-first-and-only-investigational-therapy-granted-u-s-fda-breakthrough-therapy-designation-for-the-treatment-of-adults-living-with-moderate-to-severe-sjogrens-disease**. Last accessed: November 2024.
[8] Bacci ED et al. Understanding side effects of therapy for myasthenia gravis and their impact on daily life. BMC Neurol. 2019;19(1):335.
[9] Wiendl, H., et al., Guideline for the management of myasthenic syndromes. Therapeutic advances in neurological disorders, 16, 17562864231213240. **https://doi.org/10.1177/17562864231213240**. Last accessed: September 2024.
[10] Evoli A, Batocchi AP, Bartoccioni E, Lino MM, Minisci C, Tonali P. Juvenile myasthenia gravis with prepubertal onset. Neuromuscul Disord. 1998 Dec;8(8):561-7. doi: 10.1016/s0960-8966(98)00077-7.
[11] Evoli A. Acquired myasthenia gravis in childhood. Curr Opin Neurol. 2010 Oct;23(5):536-40. doi: 10.1097/WCO.0b013e32833c32af.
[12] Finnis MF, Jayawant S. Juvenile myasthenia gravis: a paediatric perspective. Autoimmune Dis. 2011;2011:404101. doi: 10.4061/2011/404101.
[13] Haliloglu G, Anlar B, Aysun S, Topcu M, Topaloglu H, Turanli G, Yalnizoglu D. Gender prevalence in childhood multiple sclerosis and myasthenia gravis. J Child Neurol. 2002 May;17(5):390-2. doi: 10.1177/088307380201700516.
[14] Parr JR, Andrew MJ, Finnis M, Beeson D, Vincent A, Jayawant S. How common is childhood myasthenia? The UK incidence and prevalence of autoimmune and congenital myasthenia. Arch Dis Child. 2014 Jun;99(6):539-42. doi: 10.1136/archdischild-2013-304788.
[15] Mansukhani SA, Bothun ED, Diehl NN, Mohney BG. Incidence and Ocular Features of Pediatric Myasthenias. Am J Ophthalmol. 2019 Apr;200:242-249. doi: 10.1016/j.ajo.2019.01.004.
[16] Bever, C.T., Jr, Aquino, A.V., Penn, A.S., Lovelace, R.E. and Rowland, L.P. (1983), Prognosis of ocular myasthenia. Ann Neurol., 14: 516-519. **https://doi.org/10.1002/ana.410140504**
[17] Kupersmith MJ, Latkany R, Homel P. Development of generalized disease at 2 years in patients with ocular myasthenia gravis. Arch Neurol. 2003 Feb;60(2):243-8. doi: 10.1001/archneur.60.2.243. PMID: 12580710.
[18] Myasthenia gravis fact sheet. Retrieved April 2024 from **https://www.ninds.nih.gov/sites/default/files/migrate-documents/myasthenia_gravis_e_march_2020_508c.pdf**.
[19] Myasthenia Gravis: Treatment & Symptoms. (2021, April 7). Retrieved April 2024 from **https://my.clevelandclinic.org/health/diseases/17252-myasthenia-gravis-mg**.
[20] DRG EPI (2021) & Optum Claims Analysis Jan 2012-December 2020.
[21] O'Connell K, Ramdas S, Palace J. Management of Juvenile Myasthenia Gravis. Front Neurol. 2020 Jul 24;11:743. doi: 10.3389/fneur.2020.00743. PMID: 32793107; PMCID: PMC7393473.
[22] ClinicalTrials.gov Identifier: NCT04951622. Available at: **https://clinicaltrials.gov/ct2/show/NCT04951622**. Last accessed: June 2024.
[23] ClinicalTrials.gov Identifier: NCT04951622. Available at: **https://clinicaltrials.gov/ct2/show/NCT04951622**. Last accessed: October 2024.
[24] ClinicalTrials.gov. NCT03842189. Available at: **https://clinicaltrials.gov/ct2/show/NCT03842189**. Last accessed: October 2024
[25] ClinicalTrials.gov Identifier: NCT05327114. Available at: **https://www.clinicaltrials.gov/study/NCT05327114**. Last accessed: October 2024
[26] ClinicalTrials.gov Identifier: NCT04119050. Available at: **https://clinicaltrials.gov/study/NCT04119050**. Last accessed: October 2024.
[27] ClinicalTrials.gov Identifier: NCT05379634. Available at: **https://clinicaltrials.gov/study/NCT05379634**. Last accessed: October 2024.
[28] ClinicalTrials.gov Identifier: NCT05912517. Available at: **https://www.clinicaltrials.gov/study/NCT05912517**. Last accessed: October 2024
[29] ClinicalTrials.gov Identifier: NCT06028438. Available at: **https://clinicaltrials.gov/study/NCT06028438**. Last accessed: October 2024.
[30] ClinicalTrials.gov Identifier: NCT04968912. Available at: **https://clinicaltrials.gov/study/NCT04968912**. Last accessed: October 2024.
[31] ClinicalTrials.gov Identifier: NCT04882878. Available at: **https://clinicaltrials.gov/study/NCT04882878**. Last accessed: October 2024.
[32] Lobato G, Soncini CS. Relationship between obstetric history and Rh(D) alloimmunization severity. Arch Gynecol Obstet. 2008 Mar;277(3):245-8. DOI: 10.1007/s00404-007-0446-x. Last accessed: November 2024.
[33] Roy S, Nanovskaya T, Patrikeeva S, et al. M281, an anti-FcRn antibody, inhibits IgG transfer in a human ex vivo placental perfusion model. Am J Obstet Gynecol. 2019;220(5):498 e491-498 e499.

Media contact:
Bridget Kimmel
Mobile: (215) 688-6033
bkimmel@its.jnj.com

Investor contact:
Lauren Johnson
investor-relations@its.jnj.com

View original content to download multimedia:**https://www.prnewswire.com/news-releases/nipocalimab-granted-us-fda-priority-review-for-the-treatment-of-generalized-myasthenia-gravis-302346976.html**

SOURCE Johnson & Johnson