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NEWS RELEASE

Findings from pivotal nipocalimab Phase 3 study in a broad antibody positive population of people living with generalized myasthenia gravis (gMG) published in The Lancet Neurology

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The first FcRn blocker to demonstrate sustained disease control over 24 weeks in antibody positive adult patients: anti-AChR+, anti-MuSK+, anti-LRP4+

Nipocalimab demonstrated a sustained reduction in autoantibody levels, one of the underlying causes of gMG, by up to 75% over a period of 24 weeks

The investigational therapy was recently granted U.S. FDA Priority Review for the treatment of gMG

SPRING HOUSE, Pa., Jan. 23, 2025 /PRNewswire/ -- Johnson & Johnson (NYSE: JNJ) today announced The Lancet Neurology has published results from the pivotal Phase 3 study of nipocalimab, an investigational FcRn blocker, evaluated in a broad population of antibody positive (anti-AChR+, anti-MuSK+, anti-LRP4+) adults with generalized myasthenia gravis (gMG).[1] The Vivacity-MG3 study met its primary endpoint demonstrating statistically significant and clinically meaningful improvement over 24 weeks in the MG-ADL[a] score.[1] Nipocalimab had a tolerable safety profile, with adverse events leading to discontinuation rates similar to placebo (5.1% with nipocalimab vs. 7.1% with placebo).[1]

"Nipocalimab has been shown in multiple clinical studies to help reduce IgG, including autoantibodies, among this broad population of antibody positive adults with gMG. The positive results from the Vivacity-MG3 study further support the potential of nipocalimab to address the underlying cause of this debilitating autoantibody disease," said Carlo Antozzi, M.D., Neuroimmunology and Muscle Pathology Unit of the Neurological Institute Foundation C.

Besta of Milan, Italy.[b] "It's promising to see this positive data published in The Lancet Neurology as there is a continued need for additional approved targeted therapies with demonstrated safety profiles that offer sustained disease control for a broad range of antibody positive patients living with gMG."

gMG is a chronic, life-long, rare, autoantibody-driven disease, for which there currently is no cure. gMG impacts an estimated 700,000 people worldwide.[2,3] Nipocalimab, a fully human IgG1 antibody, is an immunoselective investigational therapy that has been shown in clinical trials to lower immunoglobulin G (IgG), including pathogenic IgG, one of the root causes of autoantibody diseases.[1,4] Data from the Phase 3 study showed up to a 75% reduction in the median pre-dose total IgG from baseline. Additionally, reduction in levels of common pathogenic IgG subclasses, including AChR antibody and MuSK antibody, was observed over 24 weeks of the study.[1] No changes were observed in total IgE, IgA, and IgM, highlighting the potential ability to maintain a protective immune system even after reduction of pathogenic IgG autoantibodies is observed.[1]

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).[1] 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 requiring the assistance of a ventilator.[5]

"The Phase 3 Vivacity-MG3 data demonstrates our steadfast pursuit of researching and developing potential innovative and transformational approaches for autoantibody-driven diseases, such as gMG," said Sindhu Ramchandren, M.D., Executive Medical Director, Neuroscience, Johnson & Johnson Innovative Medicine. "We are delighted by the publication of this robust Phase 3 data in The Lancet Neurology as well as the Priority Review granted by the FDA. People living with gMG require additional effective immunoselective therapeutic options that can potentially preserve the ability to maintain a protective immune response even after reduction of IgG."

Johnson & Johnson submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) and a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) seeking approval of nipocalimab in gMG on August 29 and September 11, 2024, respectively. Nipocalimab was granted Priority Review by the FDA which was supported by findings from the Phase 3 Vivacity-MG3 study.[6,7] 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.[8]

Editor's notes:

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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]
b. Dr. Carlo Antozzi has provided consulting, advisory and speaking services to Johnson & Johnson. He has not been paid for any media work.

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.[2,9,10] 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).[11,12,13] Among juvenile MG patients, girls are affected more often than boys with over 65% of pediatric MG cases in the U.S. diagnosed in girls.[14,15,16]

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.[2,17,18,19,20] Approximately 100,000 individuals in the U.S. are living with gMG.[21] Vulnerable gMG populations, such as pediatric patients, have more limited therapeutic options.[22] Currently, SOC treatments for adolescents with gMG are extrapolated from adult trials.[13] Other than symptomatic treatments, there are no approved FcRn blockers that may address the root cause of the disease for adolescents with gMG in the United States.[13]

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 SOC therapy were identified and 199 patients, 153 of whom were antibody positive, enrolled in the 24-week double-blind placebo-controlled trial.[23,24] Randomization 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.[23] Baseline demographics were balanced across arms (77 nipocalimab, 76 placebo).[23] 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.[24]

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 Rheumatic diseases.[25,26,27,28,29,30,31,32,33] Blockade of IgG binding to FcRn in the placenta is also believed to limit transplacental transfer of maternal alloantibodies to the fetus.[34,35]

The U.S. FDA and 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

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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.

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[1] Antozzi, C et al.,Efficacy and safety of nipocalimab in adults with generalised myasthenia gravis (Vivacity MG3): a randomised, double-blind, placebo-controlled phase 3 study. The Lancet Neurology. Feb 2025; 24: 105–16. **https://www.thelancet.com/journals/laneur/article/PIIS1474-4422(24)00498-8/fulltext**.
[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 Stroke. Myasthenia Gravis. Available at: **https://www.ninds.nih.gov/health-information/disorders/myasthenia-gravis**. Last accessed: January 2025.
[4] Boes M, et al. Accelerated development of IgG autoantibodies and autoimmune disease in the absence of secreted IgM. Proc Natl Acad Sci U S A. 2000 Feb 1;97(3):1184-9. doi: 10.1073/pnas.97.3.1184.
[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. Nipocalimab granted U.S. FDA Priority Review for the treatment of generalized myasthenia gravis (gMG). Available at: **https://www.jnj.com/media-center/press-releases/nipocalimab-granted-u-s-fda-priority-review-for-the-treatment-of-generalized-myasthenia-gravis-gmg**. Last accessed: January 2025
[7] 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: January 2025.
[8] 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: January 2025.
[9] Bacci ED et al. Understanding side effects of therapy for myasthenia gravis and their impact on daily life. BMC Neurol. 2019;19(1):335.
[10] 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: January 2025
[11] 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.
[12] Evoli A. Acquired myasthenia gravis in childhood. Curr Opin Neurol. 2010 Oct;23(5):536-40. doi: 10.1097/WCO.0b013e32833c32af.
[13] Finnis MF, Jayawant S. Juvenile myasthenia gravis: a paediatric perspective. Autoimmune Dis. 2011;2011:404101. doi: 10.4061/2011/404101.
[14] 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.
[15] 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.
[16] 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.
[17] 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**
[18] 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.
[19] 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**.
[20] Myasthenia Gravis: Treatment & Symptoms. (2021, April 7). Retrieved April 2024 from **https://my.clevelandclinic.org/health/diseases/17252-myasthenia-gravis-mg**.

[21] DRG EPI (2021) & Optum Claims Analysis Jan 2012-December 2020.
[22] 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.
[23] Antozzi, C et al., Nipocalimab in Generalized Myasthenia Gravis: Results from a Double-Blind, Placebo-Controlled, Randomized Phase 3 Study. Abstract at 2024 European Academy of Neurology Congress. June 2024.
[24] **ClinicalTrials.gov** Identifier: NCT04951622. Available at: **https://clinicaltrials.gov/ct2/show/NCT04951622**. Last accessed: January 2025
[25] **ClinicalTrials.gov** Identifier: NCT04951622. Available at: **https://clinicaltrials.gov/ct2/show/NCT04951622**. Last accessed: January 2025
[26] **ClinicalTrials.gov**. NCT03842189. Available at: **https://clinicaltrials.gov/ct2/show/NCT03842189**. Last accessed: January 2025
[27] **ClinicalTrials.gov** Identifier: NCT05327114. Available at: **https://www.clinicaltrials.gov/study/NCT05327114**. Last accessed: January 2025
[28] **ClinicalTrials.gov** Identifier: NCT04119050. Available at: **https://clinicaltrials.gov/study/NCT04119050**. Last accessed: January 2025
[29] **ClinicalTrials.gov** Identifier: NCT05379634. Available at: **https://clinicaltrials.gov/study/NCT05379634**. Last accessed: January 2025.
[30] **ClinicalTrials.gov** Identifier: NCT05912517. Available at: **https://www.clinicaltrials.gov/study/NCT05912517**. Last accessed: January 2025
[31] **ClinicalTrials.gov** Identifier: NCT06028438. Available at: **https://clinicaltrials.gov/study/NCT06028438**. Last accessed: January 2025
[32] **ClinicalTrials.gov** Identifier: NCT04968912. Available at: **https://clinicaltrials.gov/study/NCT04968912**. Last accessed: January 2025
[33] **ClinicalTrials.gov** Identifier: NCT04882878. Available at: **https://clinicaltrials.gov/study/NCT04882878**. Last accessed: January 2025.
[34] 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.
[35] 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.

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