Johnson&Johnson

NEWS RELEASE

Nipocalimab demonstrates sustained disease control in adolescents living with generalized myasthenia gravis in Phase 2/3 study

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First FcRn blocker to demonstrate sustained disease control over 24 weeks in antibody positive adolescents aged 12 – 17 years, broadening the population in which nipocalimab has been studied

SAVANNAH, Ga., Oct. 15, 2024 /PRNewswire/ -- Johnson & Johnson (NYSE: JNJ) today announced positive results from the Phase 2/3 Vibrance-MG study of nipocalimab in anti-AChR[a] positive adolescents (aged 12 – 17 years) living with generalized myasthenia gravis (gMG). Study participants who were treated with nipocalimab plus standard of care (SOC) achieved sustained disease control as measured by the primary endpoint of immunoglobulin G (IgG) reduction from baseline over 24 weeks, and secondary endpoints of improvement in MG-ADL[b] and QMG[c] scores. These Phase 2/3 data will be featured in an oral presentation (Abstract #MG100) at the Myasthenia Gravis Foundation of America (MGFA) Scientific Session during the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) Annual Meeting, where Johnson & Johnson will present **25 abstracts**.

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"Findings from the Vibrance-MG study underscore the potential of this investigational therapy for young individuals aged 12 – 17 living with gMG. Results show a significant reduction in IgG of approximately 70% in adolescents and a clinical benefit that is consistent with the Vivacity-MG3 study in adults," said Jonathan Strober, M.D., Director of Clinical Services for Child Neurology and Director of the Muscular Dystrophy Clinic at UCSF Benioff Children's Hospital.[d] "It is encouraging to see these positive results as there are currently no approved advanced treatment options for this adolescent population in the United States."

About 10% of new cases of myasthenia gravis are diagnosed in adolescents (12 – 17 years of age) and the severity of gMG in pediatric patients is heightened with 43% having experienced over five hospitalizations in their lifetime, 46% having at least one intensive care unit stay and 68% having periods of exacerbated disease.[1,2,3,4]

Treatment with nipocalimab plus SOC met the study's primary endpoint of reduction in total serum IgG (-69%), and the two secondary endpoints of MG-ADL and QMG, which are measures of disease activity.[5,e] Four of five patients achieved minimum symptom expression (MG-ADL score 0-1) by the end of their treatment phase.[f,g] Nipocalimab was well-tolerated over the six-month period, similar to tolerability seen in adult participants in the Vivacity-MG3 study.[5] There were no serious adverse events and no discontinuations due to an adverse event.

Presented for the first time, these open-label Phase 2/3 results in adolescents are consistent with findings from the pivotal study of nipocalimab in adult patients with gMG. Nipocalimab when added to SOC is the **first FcRn blocker** to demonstrate sustained disease control in a registrational trial as measured by improvement in MG-ADL over placebo plus SOC over a period of six months of consistent dosing (Q2 week) among adults living with gMG.

"The Vibrance-MG data add to the expanding clinical profile of nipocalimab and highlight its potential for adolescents living with gMG who are in need of new treatments," said Sindhu Ramchandren, M.D., Executive Medical Director, Neuroscience, Johnson & Johnson Innovative Medicine. "We are committed to developing innovations for autoantibody-driven neurological diseases, like gMG, with the aim of transforming the lives of people living with these conditions."

Earlier this year, Johnson & Johnson announced the submission of applications to the U.S. Food and Drug Administration (**FDA**) and the European Medicines Agency (**EMA**) seeking approval for nipocalimab for the treatment of gMG.

Editor's notes:

a. Patients with a positive blood test for acetylcholine receptor (anti-AChR) antibodies or muscle-specific tyrosine kinase (anti-MuSK) antibodies are eligible for the study.
b. 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.
c. QMG (Quantitative Myasthenia Gravis) is a 13-item assessment by a clinician that quantifies MG disease severity through muscle weakness. The total QMG score ranges from 0 to 39, where higher scores indicated greater disease severity.
d. Dr. Jonathan Strober is a paid consultant for Johnson & Johnson. He has not been compensated for any media

work.

e. Treatment with nipocalimab showed a mean percentage change from baseline to week 24 for total serum IgG of -68.98% (standard error [SE] = 7.561).

f. Adolescents who received nipocalimab plus current SOC had a mean baseline score of 4.29 (SE = 2.430) on the MG-ADL scale and a mean baseline score of 12.50 (SE = 3.708) on the QMG scale.

g. Adolescents who received nipocalimab plus current SOC had a mean change at week 24 of -2.40 (SE = 0.187) on the MG-ADL scale and -3.80 (SE = 2.683) on the QMG scale.

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. [6,7] The disease impacts an estimated 700,000 people worldwide.[6] Approximately 10 to 15% of new cases of MG are diagnosed in adolescents (12 – 17 years of age).[1,2,3] Among juvenile MG patients, girls are affected more often than boys with over 65% of pediatric MG cases in the US diagnosed in girls.[8,9,10]

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.[6,11,12,13,14] Approximately 100,000 individuals in the U.S. are living with gMG.[15] Vulnerable gMG populations, such as pediatric patients, have more limited therapeutic options.[3] Currently, SOC treatments for adolescents with gMG are extrapolated from adult trials.[3] 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.[3]

About the Phase 2/3 Vibrance-MG Study

The Phase 2/3 Vibrance-MG study (**NCT05265273**) is an on-going open-label study to determine the effect of nipocalimab in pediatric participants with gMG.[16] Seven participants aged 12 – 17 years with a diagnosis of gMG as reflected by a Myasthenia Gravis Foundation of America (MGFA) Class of II through IV at screening, and an insufficient clinical response to ongoing, stable SOC therapy, have been enrolled in the trial.[5] Participants must have a positive blood test for either anti-AChR or anti-MUSK autoantibodies. The study consists of a screening period of up to four weeks, a 24-week open-label Active Treatment Phase during which participants receive nipocalimab intravenously every two weeks, and a Long-term Extension Phase; a safety follow-up assessment will be conducted at eight weeks after last dose.[16] The primary outcome of the study is the effect of nipocalimab on total serum IgG, safety and tolerability, and pharmacokinetics in pediatric participants with gMG at 24 weeks. Secondary endpoints

include change in MG-ADL and QMG scores at 24 weeks.[5,16]

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 Prevalent Rheumatology.[17,18,19,20,21,22,23,24,25] Blockade of IgG binding to FcRn in the placenta is also believed to limit transplacental transfer of maternal alloantibodies to the fetus.[26,27]

The U.S. Food and Drug Administration (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 by the FDA
- 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.

[1] 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.

[2] Evoli A. Acquired myasthenia gravis in childhood. Curr Opin Neurol. 2010 Oct;23(5):536-40. doi: 10.1097/WCO.0b013e32833c32af.

[3] Finnis MF, Jayawant S. Juvenile myasthenia gravis: a paediatric perspective. Autoimmune Dis. 2011;2011:404101. doi: 10.4061/2011/404101.

[4] Barraud C, Desguerre I, Barnerias C, Gitiaux C, Boulay C, Chabrol B. Clinical features and evolution of juvenile myasthenia gravis in a French cohort. Muscle Nerve. 2018 Apr;57(4):603-609. doi: 10.1002/mus.25965.

[5] Strober J et al. Safety and effectiveness of nipocalimab in adolescent participants in the open label Phase 2/3 Vibrance-MG clinical study. Presentation at American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) Annual Meeting. October 2024.

[6] 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. **https://www.thelancet.com/action/showPdf?pii=S2666-6065%2820%2930063-8**

[7] 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: October 2024.
[8] 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.
[9] 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.
[10] 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.
[11] 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**
[12] 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.
[13] 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**.
[14] Myasthenia Gravis: Treatment & Symptoms. (2021, April 7). Retrieved April 2024 from **https://my.clevelandclinic.org/health/diseases/17252-myasthenia-gravis-mg**.
[15] DRG EPI (2021) & Optum Claims Analysis Jan 2012-December 2020.
[16] ClinicalTrials.gov. NCT05265273. Available at: **https://clinicaltrials.gov/study/NCT05265273**. Last accessed: October 2024
[17] ClinicalTrials.gov Identifier: NCT04951622. Available at: **https://clinicaltrials.gov/ct2/show/NCT04951622**. Last accessed: October 2024.
[18] ClinicalTrials.gov. NCT03842189. Available at: **https://clinicaltrials.gov/ct2/show/NCT03842189**. Last accessed: October 2024
[19] ClinicalTrials.gov Identifier: NCT05327114. Available at: **https://www.clinicaltrials.gov/study/NCT05327114**. Last accessed: October 2024
[20] ClinicalTrials.gov Identifier: NCT04119050. Available at: **https://clinicaltrials.gov/study/NCT04119050**. Last accessed: October 2024.
[21] ClinicalTrials.gov Identifier: NCT05379634. Available at: **https://clinicaltrials.gov/study/NCT05379634**. Last accessed: October 2024.
[22] ClinicalTrials.gov Identifier: NCT05912517. Available at: **https://www.clinicaltrials.gov/study/NCT05912517**. Last accessed: October 2024
[23] ClinicalTrials.gov Identifier: NCT06028438. Available at: **https://clinicaltrials.gov/study/NCT06028438**. Last accessed: October 2024.
[24] ClinicalTrials.gov Identifier: NCT04968912. Available at: **https://clinicaltrials.gov/study/NCT04968912**. Last accessed: October 2024.
[25] ClinicalTrials.gov Identifier: NCT04882878. Available at: **https://clinicaltrials.gov/study/NCT04882878**. Last

accessed: October 2024.

[26] 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: October 2024.

[27] 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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