# Cemdisiran, Dosed Subcutaneously Every 12 Weeks, Demonstrates Rapid, Deep and Sustained Disease Control in Generalized Myasthenia Gravis (gMG) Phase 3 Trial

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**As published in *The Lancet* and presented at AAN, NIMBLE trial met its primary and key secondary endpoints at week 24, demonstrating potential best-in-class efficacy and convenience in gMG**

**U.S. regulatory application submitted; cemdisiran could be the first siRNA to be approved for the treatment of gMG**

**Regeneron to host virtual 'Regeneron Roundtable: gMG & C5 Complement Program' investor event on Wednesday, April 22 at 8:30 a.m. ET**

TARRYTOWN, N.Y., April 21, 2026 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced detailed positive results from the Phase 3 NIMBLE trial evaluating investigational cemdisiran in adults with generalized myasthenia gravis (gMG) were published in *The Lancet* and presented for the first time in an oral plenary session at the American Academy of Neurology (AAN) Annual Meeting. Cemdisiran is a novel siRNA therapeutic that durably reduces circulating levels of complement factor 5 (C5), allowing for every three months dosing.

"Generalized myasthenia gravis is a chronic debilitating disease with unpredictable symptoms that impact daily life. While current therapies have managed disease activity, there remains an unmet need for options that achieve rapid and sustained efficacy with reduced treatment burden," said Tuan Vu, M.D., Professor of Neurology at the University of South Florida Morsani College of Medicine, Division Director for Neuromuscular Medicine and EMG Laboratories, and a global principal investigator of the NIMBLE trial.* "In the Phase 3 NIMBLE trial, this first-of-its-kind investigational therapy demonstrated rapid, robust efficacy with lasting benefit through 24 weeks. These impressive results suggest cemdisiran can represent a transformative advance in care for people living with gMG, offering compelling efficacy coupled with convenient four-times-a-year subcutaneous administration."

The NIMBLE trial results at AAN provide additional detail to the topline data shared in August 2025. As presented, and as compared to placebo (n=59), patients treated with subcutaneous cemdisiran (n=64) every twelve weeks experienced clinically meaningful improvements within two weeks on two assessment scales that measured changes from baseline in: daily functions impacted by gMG (such as talking, eating, breathing, vision and mobility) and muscle function (such as vision, speaking/swallowing, breathing and limb mobility). These improvements deepened over time across both scales and were sustained through week 24, with no indication of waning efficacy between doses.

Specifically, the results for cemdisiran compared to placebo at week 24 demonstrated a:

- **4.5-point improvement (least squares [LS] mean change) from baseline on the Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score** (0.4 standard error [SE]) compared to a 2.2-point improvement (0.5 SE). This corresponded to a placebo-adjusted improvement of 2.3 points ($p<0.001$), meeting the primary endpoint. Notably, 76.6% of cemdisiran patients had a ≥3-point improvement, versus 44.1% for placebo. Clinically meaningful improvements in MG-ADL total score occurred within 2 weeks in cemdisiran-treated patients (-2.5-point LS mean change from baseline), which was a pre-specified exploratory endpoint. MG-ADL is a patient-reported assessment of daily functions impacted by gMG.
- **4.2-point improvement (LS mean change) from baseline in Quantitative Myasthenia Gravis (QMG) total score (0.6 SE)** compared to a 1.5-point improvement (0.7 SE). This resulted in a placebo-adjusted improvement of 2.8 points ($p=0.002$), meeting the key secondary endpoint. Notably, 48.4% of cemdisiran patients had a ≥5-point improvement, compared to 19% for placebo. Improvements in QMG total scores occurred within 2 weeks in cemdisiran-treated patients (-1.9-point LS mean change from baseline), which was a pre-specified exploratory endpoint. QMG is a physician-administered assessment of muscle function.

Approved C5 inhibitor therapies have demonstrated in previous registrational clinical trials placebo-adjusted improvements in MG-ADL total scores ranging from -1.6 to -2.1 and in QMG total scores ranging from -2.0 to -3.0 at the time of each trial's primary analysis (generally at week 12 or week 26).

One or more treatment-emergent adverse events (AEs) occurred in 69.2% of patients receiving cemdisiran (cemdi) and 77.1% receiving placebo (pbo). Most AEs were mild-to-moderate in severity. The most common AEs (≥5%) in patients receiving cemdisiran or placebo were: worsening of MG (1% cemdi, 17% pbo), upper respiratory tract infection (12% cemdi, 11% pbo), urinary tract infection (5% cemdi, 3% pbo), nasopharyngitis (5% cemdi, 4% pbo), headache (5% cemdi, 10% pbo), rash (5%

cemdi, 1% pbo), and diarrhea (3% cemdi, 7% pbo). Overall, the rates of infection during the double-blind treatment period (DBTP) were 27% in the cemdisiran group and 40% in the placebo group. No serious infections, meningococcal infections, or deaths occurred during the DBTP. There were no treatment discontinuations due to adverse events through week 24 in the cemdisiran arm compared to 3% of patients receiving placebo. Adverse events leading to dose interruption/reduction occurred in 1% and 4% of patients receiving cemdisiran and placebo, respectively. During the extension period, one death due to pneumonia occurred in the cemdisiran arm in a patient with a significant co-morbidity and also receiving concomitant immunosuppressive therapy. Rates of hospitalization, myasthenic crisis and rescue therapy were numerically lower with cemdisiran.

The potential use of cemdisiran for the treatment of gMG is investigational and has not been approved by any regulatory authority. The U.S. regulatory application for cemdisiran was submitted in the first quarter of 2026; additional regulatory filings, including in the European Union, are planned for 2026.

**About the 'Regeneron Roundtable: gMG & C5 Complement Program' Investor Event**
Regeneron will host a virtual investor event to discuss its C5 development program on Wednesday, April 22 at 8:30 a.m. ET. This is the next webcast in a new investor event series called the 'Regeneron Roundtable', intended to highlight programs from the company's innovative investigational pipeline.

Links to the webcast and to register via telephone may be accessed from the 'Investors and Media' page of Regeneron's website at https://investor.regeneron.com/events-and-presentations. Upon registration, all telephone participants will receive a confirmation email detailing how to join the conference call, including the dial-in number along with a unique passcode and registrant ID that can be used to access the call. A replay of the conference call and webcast will be archived on the company's website for at least 30 days.

**About Myasthenia Gravis (MG)**
MG is a rare and chronic autoimmune disease where abnormal acetylcholine receptor (anti-AChR) antibodies activate the complement system including C5, disrupting communication between nerves and muscles that results in debilitating and potentially life-threatening muscle weakness. In the U.S., the disease impacts approximately 85,000 people. Initial manifestations are usually ocular, but approximately 85% of MG patients experience additional advancements to the disease manifestations, which is known as generalized myasthenia gravis (gMG). For these patients, the disease affects muscles throughout the body, resulting in extreme fatigue and difficulties with facial expression, speech, swallowing and mobility. Treatment-related challenges – which include frequent hospital visits, inconsistent symptom control, and lack of durable treatment effects – can further affect quality of life and long-term disease management.

**About the Complement Factor 5 (C5) Clinical Program**
The largest global, interventional gMG trial conducted to date, NIMBLE is a randomized, double-blind, placebo-controlled ongoing trial evaluating cemdisiran, as well as a combination of cemdisiran and pozelimab (C5 antibody), in patients with gMG who have antibodies to the acetylcholine receptor (anti-AChR) and could continue background immunosuppressants based on the investigator's discretion.

Participants were randomized to receive one of four subcutaneous regimens: cemdisiran (600 mg) every 12 weeks; a combination of cemdisiran (200 mg) and pozelimab (200 mg) every 4 weeks; pozelimab (200 mg) every 4 weeks; or placebo every 4 weeks. The primary endpoint assessed changes in the MG-ADL total score from baseline to week 24. The MG-ADL scale is an eight-question patient-reported tool that measures how gMG affects aspects of daily life and provides a total score ranging from 0 to 24. The key secondary endpoint was the change from baseline in QMG total score at week 24. The QMG is a physician-administered 13-item standardized assessment evaluating muscle function that provides a total score ranging from 0 to 39. In both MG-ADL and QMG, a higher total score indicates greater disease severity, and a larger reduction in total score indicates greater improvement in disease symptoms and better treatment effect.

Cemdisiran and pozelimab are also being evaluated in separate Phase 3 trials as both monotherapy and combination therapy for additional complement-mediated disorders, including PNH and geographic atrophy secondary to age-related macular degeneration. For more information, visit the Regeneron clinical trials website, or contact clinicaltrials@regeneron.com or +1 844-734-6643.

Regeneron is solely responsible for the development, manufacturing, and commercialization of cemdisiran as a monotherapy and in combination with C5 antibodies through a worldwide licensing agreement with Alnylam.

In the U.S., pozelimab monotherapy is approved as Veopoz® (pozelimab-bbfg) for adult and pediatric patients 1 year of age and older with CHAPLE disease, also known as CD55-deficient protein-losing enteropathy, which includes a Boxed Warning for life-threatening and fatal meningococcal infections.

**IMPORTANT SAFETY INFORMATION**

**What is the most important information I should know about VEOPOZ?**
**VEOPOZ is a medicine that affects your immune system and can lower the ability of your immune system to fight infections.**

- VEOPOZ increases your chance of getting serious and life-threatening meningococcal infections that may quickly become life-threatening and cause death if not recognized and treated early.

1. You must receive meningococcal vaccines at least 2 weeks before your first dose of VEOPOZ if you have not already had these vaccines.
2. If you had a meningococcal vaccine in the past, you might need additional vaccination before starting VEOPOZ. Your healthcare provider will decide if you need additional meningococcal vaccination.
3. If your healthcare provider decides that urgent treatment with VEOPOZ is needed, and your meningococcal vaccines are not up-to-date, you should receive meningococcal vaccination as soon as possible. You should also receive antibiotics.
4. Meningococcal vaccines reduce the risk of meningococcal infection but do not prevent all meningococcal infections. Call your healthcare provider or get emergency medical care right away if you get any of these signs and symptoms of a meningococcal infection:
    - headache with nausea or vomiting
    - headache with a stiff neck or stiff back
    - fever and a rash
    - muscle aches with flu-like symptoms
    - headache and fever
    - fever
    - confusion
    - eyes sensitive to light

**Your healthcare provider will give you a Patient Safety Card about the symptoms of meningococcal, or other infection.** Carry it with you at all times during treatment and for 3 months after your last VEOPOZ dose. Your risk of meningococcal infection may continue for several weeks after your last dose of VEOPOZ. It is important to show this card to any healthcare provider who treats you. This will help them diagnose and treat you quickly.

**VEOPOZ may also increase the risk of other types of serious bacterial infections.**

- People who take VEOPOZ may have an increased risk of getting infections caused by *Streptococcus pneumoniae and Haemophilus influenzae*.
- Certain people may also have an increased risk of bacterial infection including gonorrhea infection. Talk to your healthcare provider to find out if you are at risk of gonorrhea infection, about gonorrhea prevention, and regular testing.

Call your healthcare provider right away if you have any new signs or symptoms of infection.

**Do not receive VEOPOZ if you** have a meningococcal infection.

**Before you receive VEOPOZ, tell your healthcare provider about all of your medical conditions, including if you:** have an infection or fever, are pregnant or plan to become pregnant, and are breastfeeding or plan to breastfeed. It is not known if VEOPOZ will harm your unborn baby or if it passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby during treatment with VEOPOZ.

**Tell your healthcare provider about all the medicines you take,** including prescription and over-the-counter medicines, vitamins, and herbal supplements. It is important that you have all recommended vaccinations before you start VEOPOZ, receive antibiotics if you start VEOPOZ within 2 weeks of receiving meningococcal vaccination, and stay up to date with all recommended vaccinations during treatment with VEOPOZ.
VEOPOZ and other medicines may affect each other, causing side effects. VEOPOZ may affect the way other medicines work, and other medicines may affect how VEOPOZ works.

**Especially tell your healthcare provider if you take Intravenous Immunoglobulin (IVIg).**
Know the medicines you take and the vaccines you receive. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

**What are the possible side effects of VEOPOZ?**
**VEOPOZ can cause serious side effects including allergic (hypersensitivity) reactions including infusion-related reactions, which may happen during your treatment.** Tell your healthcare provider right away if you develop any of these symptoms or any other symptom during your VEOPOZ treatment that may mean you are having a serious allergic reaction: chest pain, trouble breathing or shortness of breath, swelling of your face, tongue, or throat, and feel faint or pass out.

**The most common side effects of VEOPOZ are** upper respiratory tract infection, fracture, raised, red patches of skin that are often very itchy (hives), and hair loss (alopecia).
Tell your healthcare provider if you have any side effect that bothers you or that does not go away. These are not all of the possible side effects of VEOPOZ. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

**Please see the full Prescribing Information, including Boxed WARNING, and Medication Guide for VEOPOZ.**

**About Regeneron's *VelocImmune* Technology**
Regeneron's *VelocImmune* technology utilizes a proprietary genetically engineered mouse platform endowed with a genetically

humanized immune system to produce optimized fully human antibodies. When Regeneron's co-Founder, President and Chief Scientific Officer George D. Yancopoulos was a graduate student with his mentor Frederick W. Alt in 1985, they were the first to envision making such a genetically humanized mouse, and Regeneron has spent decades inventing and developing *VelocImmune* and related *VelociSuite*® technologies.

Dr. Yancopoulos and his team have used *VelocImmune* technology to create a substantial proportion of all original, FDA-approved or authorized fully human monoclonal antibodies. This includes REGEN-COV® (casirivimab and imdevimab), Dupixent® (dupilumab), Libtayo® (cemiplimab-rwlc), Praluent® (alirocumab), Kevzara® (sarilumab), Evkeeza® (evinacumab-dgnb), Inmazeb® (atoltivimab, maftivimab and odesivimab-ebgn) and Veopoz® (pozelimab).

**About Regeneron**
Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents, develops and commercializes life-transforming medicines for people with serious diseases. Founded and led by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to numerous approved treatments and product candidates in development, most of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, neurological diseases, hematologic conditions, infectious diseases, and rare diseases.

Regeneron pushes the boundaries of scientific discovery and accelerates drug development using our proprietary technologies, such as *VelociSuite*®, which produces optimized fully human antibodies and new classes of bispecific antibodies. We are shaping the next frontier of medicine with data-powered insights from the Regeneron Genetics Center® and pioneering genetic medicine platforms, enabling us to identify innovative targets and complementary approaches to potentially treat or cure diseases.

For more information, please visit www.Regeneron.com or follow Regeneron on LinkedIn, Instagram, Facebook or X.

**Forward-Looking Statements and Use of Digital Media**
*This press release includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron Pharmaceuticals, Inc. ("Regeneron" or the "Company"), and actual events or results may differ materially from these forward-looking statements. Words such as "anticipate," "expect," "intend," "plan," "believe," "seek," "estimate," variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the nature, timing, and possible success and therapeutic applications of products marketed or otherwise commercialized by Regeneron and/or its collaborators or licensees (collectively, "Regeneron's Products") and product candidates being developed by Regeneron and/or its collaborators or licensees (collectively, "Regeneron's Product Candidates") and research and clinical programs now underway or planned, including without limitation cemdisiran (an investigational siRNA therapeutic targeting C5); the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's Product Candidates and new indications for Regeneron's Products, including cemdisiran for the treatment of adults with generalized myasthenia gravis as discussed in this press release as well as cemdisiran as a monotherapy or in combination with pozelimab (a C5 antibody) for the treatment of other complement-mediated disorders (including paroxysmal nocturnal hemoglobinuria and/or geographic atrophy); uncertainty of the utilization, market acceptance, and commercial success of Regeneron's Products and Regeneron's Product Candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary), including the studies discussed or referenced in this press release, on any of the foregoing or any potential regulatory approval of Regeneron's Products and Regeneron's Product Candidates (such as cemdisiran and pozelimab); the ability of Regeneron's collaborators, licensees, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron's Products and Regeneron's Product Candidates; the ability of Regeneron to manage supply chains for multiple products and product candidates and risks associated with tariffs and other trade restrictions; safety issues resulting from the administration of Regeneron's Products and Regeneron's Product Candidates (such as cemdisiran and pozelimab) in patients, including serious complications or side effects in connection with the use of Regeneron's Products and Regeneron's Product Candidates in clinical trials; determinations by regulatory and administrative governmental authorities which may delay or restrict Regeneron's ability to continue to develop or commercialize Regeneron's Products and Regeneron's Product Candidates; ongoing regulatory obligations and oversight impacting Regeneron's Products, research and clinical programs, and business, including those relating to patient privacy; the availability and extent of reimbursement or copay assistance for Regeneron's Products from third-party payors and other third parties, including private payor healthcare and insurance programs, health maintenance organizations, pharmacy benefit management companies, and government programs such as Medicare and Medicaid; coverage and reimbursement determinations by such payors and other third parties and new policies and procedures adopted by such payors and other third parties; changes to drug pricing regulations and requirements and Regeneron's pricing strategy; other changes in laws, regulations, and policies affecting the healthcare industry; competing products and product candidates (including biosimilar products) that may be superior to, or more cost effective than, Regeneron's Products and Regeneron's Product Candidates; the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators or licensees may be replicated in other studies and/or lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its financial projections or guidance and changes to the assumptions underlying those projections or guidance; the potential for any license, collaboration, or supply agreement, including Regeneron's agreements with Sanofi and Bayer (or their respective affiliated companies, as applicable), to be cancelled or terminated; the impact of public health outbreaks, epidemics, or pandemics on Regeneron's business; and risks associated with litigation and other proceedings and government investigations relating to the Company and/or*

*its operations (including the pending civil proceedings initiated or joined by the U.S. Department of Justice and the U.S. Attorney's Office for the District of Massachusetts), risks associated with intellectual property of other parties and pending or future litigation relating thereto (including without limitation the patent litigation and other related proceedings relating to EYLEA® (aflibercept) Injection), the ultimate outcome of any such proceedings and investigations, and the impact any of the foregoing may have on Regeneron's business, prospects, operating results, and financial condition. A more complete description of these and other material risks can be found in Regeneron's filings with the U.S. Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2025. Any forward-looking statements are made based on management's current beliefs and judgment, and the reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update (publicly or otherwise) any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.*

*Regeneron uses its media and investor relations website and social media outlets to publish important information about the Company, including information that may be deemed material to investors. Financial and other information about Regeneron is routinely posted and is accessible on Regeneron's media and investor relations website (https://investor.regeneron.com) and its LinkedIn page (https://www.linkedin.com/company/regeneron-pharmaceuticals).*

*This is not an endorsement from the University of South Florida

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