Cemdisiran Regulatory Submissions Accepted for Review by FDA and EMA for the Treatment of Generalized Myasthenia Gravis (gMG)

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Cemdisiran could be the first siRNA approved for the treatment of gMG and only therapy to be offered subcutaneously with four times a year dosing

FDA accepted NDA under Priority Review with a target action date in November 2026; European Commission decision anticipated in the second half of 2027

TARRYTOWN, N.Y., June 22, 2026 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced that both the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) have accepted the regulatory applications for cemdisiran to treat adult patients with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive. The FDA will review the New Drug Application (NDA) under Priority Review with a target action date in November 2026, following use of a Priority Review Voucher. A decision from the European Commission is anticipated in the second half of 2027.

The submissions are supported by data from the Phase 3 NIMBLE trial evaluating cemdisiran, dosed subcutaneously every 12 weeks, in adults with symptomatic gMG who may be receiving standard of care immunosuppressants based on the investigator's discretion. Full data from NIMBLE, which is one of the largest global, interventional gMG trials conducted to date, were simultaneously published in *The Lancet* and presented at the American Academy of Neurology (AAN) Annual Meeting in April 2026. A regulatory filing in Japan is also planned for early 2027.

MG is a rare and chronic autoimmune disease where abnormal 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. Worldwide, an estimated 150 to 200 out of every million people have MG. In the U.S., the disease impacts approximately 85,000 people. Initial manifestations are usually ocular, but approximately 85% of MG patients experience progression to additional disease manifestations, which is then categorized as generalized MG. For these patients, the disease affects muscles throughout the body, resulting in extreme fatigue and difficulties with facial expression, speech, swallowing and mobility. For patients living with gMG, many continue to experience challenges with disease management including treatments that only address symptoms, long-term burden of immunosuppressants, lack of responsiveness as well as waning effectiveness, which can all affect their quality of life.

The safety and efficacy of cemdisiran, as well as its potential use for the treatment of gMG, are investigational and have not been fully evaluated or approved by any regulatory authority.

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.

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 in the United States and/or European Union 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 secondary to age-related macular degeneration); uncertainty of the utilization, market acceptance, and/or 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, including in connection with Regeneron's April 2026 agreements with the U.S. government; 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 and its Form 10-Q for the quarterly period ended March 31, 2026. 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).

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