Livsayis™ (cemdisiran) Approved by the FDA as First and Only siRNA Therapy in Generalized Myasthenia Gravis and Only Treatment with Four-Times-a-Year Subcutaneous Dosing

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Approval based on Phase 3 NIMBLE results demonstrating rapid, deep and sustained disease control that is maintained throughout three-month dosing intervals

Livsayis is Regeneron's first approved siRNA therapy and neurology medicine

TARRYTOWN, N.Y., Nov. 18, 2026 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced that the U.S. Food and Drug Administration (FDA) has approved Livsayis™ (cemdisiran) for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody-positive, the predominant form of the disease. Livsayis is a novel, complement component factor 5 (C5)-directed small interfering RNA (siRNA) therapeutic that reduces levels of circulating C5 protein, limiting terminal complement activation, a key driver of the disease. It is the first and only approved siRNA therapy in gMG and the only FDA-approved gMG treatment administered subcutaneously four times a year.

“For people living with gMG, every day can be unpredictable, due to fluctuating muscle weakness and fatigue. They may also experience severe exacerbations that can be life-threatening. Many still experience suboptimal disease control, whether from waning of therapeutic effect, inadequate response or high 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, Livsayis delivered rapid, deep disease control – maintained through the end of dosing intervals – and offers a four-times-a-year subcutaneous dosing schedule. These results highlight how a first-of-its-kind approach can address a key driver of disease and provide a compelling treatment option.”

The FDA approval was based on results from the Phase 3 NIMBLE trial. Livsayis demonstrated the following least square mean change from baseline:

- **4.5-point improvement on the Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score at 24 weeks** (0.4 standard error [SE]) compared to a 2.2-point improvement with placebo (0.5 SE). This corresponded to a placebo-adjusted improvement of 2.3 points (p=0.0005). MG-ADL is a clinician-administered patient-reported assessment of daily functions impacted by gMG, such as talking, chewing, swallowing, breathing, vision and mobility. The proportion of MG-ADL responders with at least 3-point improvement at week 24 was greater for Livsayis (76.6%) compared to placebo (44.1%, p<0.001).
- **4.2-point improvement in Quantitative Myasthenia Gravis (QMG) total score at 24 weeks** (0.6 SE) compared to a 1.5-point improvement with placebo (0.7 SE), corresponding to a placebo-adjusted improvement of 2.8 points (p=0.0015). QMG is a clinician-administered assessment of muscle function, including vision, speaking/swallowing, breathing and limb mobility. The proportion of QMG responders with at least a 5-point improvement at week 24 was greater for Livsayis (48.4%) compared to placebo (19.0%, p<0.001).
- **Rapid onset of clinically meaningful benefit in MG-ADL (2.5 points), with improvements seen as early as two weeks** of starting treatment that deepened over time.
- **Sustained disease control and symptom relief through 24 weeks**, with MG-ADL scores maintained and no indication of waning efficacy.

The prescribing information for Livsayis includes a Boxed Warning for serious meningococcal infections in addition to warnings and precautions for other serious infections, consistent with other FDA-approved medicines that reduce C5 activity. The most common adverse reactions (≥5%) in patients treated with Livsayis and more frequently than placebo were injection site reactions, viral infections and urinary tract infections. Livsayis is available only through a restricted program called the Livsayis Risk Evaluation and Mitigation Strategy (REMS), which is also consistent with other FDA-approved medicines that reduce C5 activity. Details of the Important Safety Information are included below.

“Livsayis is Regeneron’s first approved medicine in neurology, and an important new option for people living with generalized myasthenia gravis who need effective disease control without a heavy treatment burden,” said George D. Yancopoulos, M.D., Ph.D., Board Co-Chair, President and Chief Scientific Officer of Regeneron. “As Regeneron’s first approved siRNA therapy, this approval marks a meaningful step forward for our neuroscience pipeline as we relentlessly pursue bold, new approaches to transform care for people living with serious neurological diseases.”

“Individuals living with gMG experience profound muscle weakness that can affect how they talk, see, breathe and move, and with

it their ability to carry out everyday activities," said Samantha Masterson, President & CEO, Myasthenia Gravis Foundation of America. "This approval is a positive milestone for the gMG community, reflecting continued progress on outcomes that matter most to patients. With Livsayis, those living with generalized myasthenia gravis have a new option that may help maintain disease control while reducing the burden treatment can place on daily life."

Regeneron is committed to supporting people living with rare diseases and ensuring patients who have been prescribed Livsayis access to their medication. Regeneron's myRARE™ program offers resources to help patients and healthcare providers, including product information, insurance benefit verification, and information about potential financial support. To learn about myRARE, visit myRARE.com or call 1-833-4my-RARE (1-833-469-7273).

A regulatory submission for Livsayis for the treatment of gMG is currently under review in the European Union, with a decision anticipated in the second half of 2027. Regulatory submissions are planned in additional markets, including Japan.

About gMG
gMG is a rare, chronic autoimmune disease characterized by unpredictable, debilitating and potentially life-threatening muscle weakness. In the most common form of the disease, abnormal anti-AChR antibodies activate the complement system including C5, disrupting communication between nerves and muscles, which contributes to damage at the neuromuscular junction. For these patients, the disease affects muscles throughout the body, resulting in extreme fatigue and difficulties with facial expression, speech, swallowing, mobility, breathing as well as severe exacerbations, including myasthenic crisis that can impair breathing to the point of requiring hospitalization. Worldwide, an estimated 150 to 200 out of every million people have MG. In the U.S., the disease impacts approximately 85,000 people.

About Livsayis
Livsayis is the first approved siRNA therapy for gMG and the only gMG treatment FDA-approved to be administered subcutaneously four times per year (every three months).

Livsayis is a complement C5-directed siRNA that degrades the messenger RNA (mRNA) that encodes the C5 protein, thereby blocking C5 production, reducing circulating levels of C5 and inhibiting terminal complement activation, a key driver of AChR-positive gMG.

Regeneron is solely responsible for the development, manufacturing, and commercialization of Livsayis through a worldwide licensing agreement with Alnylam.

About the NIMBLE trial
NIMBLE is the largest global, interventional gMG trial conducted to date. In this ongoing Phase 3, randomized, double-blind, placebo-controlled trial, Livsayis is being evaluated in patients with anti-AChR antibody-positive gMG who can continue background immunosuppressants based on the investigator's discretion. Participants were randomized to receive one of four subcutaneous regimens: Livsayis every three months; a combination of Livsayis and a C5-inhibiting monoclonal antibody every 4 weeks; C5-inhibiting monoclonal antibody 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 clinician-administered, 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 clinician-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.

IMPORTANT SAFETY INFORMATION

What is the most important information I should know about LIVSAYIS?

LIVSAYIS is a medicine that affects part of your immune system. LIVSAYIS may lower the ability of your immune system to fight certain infections.

- LIVSAYIS increases your chance of getting serious meningococcal infections caused by **Neisseria meningitidis** bacteria. Meningococcal infections may quickly become life-threatening or cause death if not recognized and treated early.
    - You must complete your meningococcal vaccines at least 2 weeks before your first dose of LIVSAYIS.
    - If you have not completed your meningococcal vaccines, and LIVSAYIS must be started right away, you should receive the required vaccines as soon as possible.
    - If you have not been vaccinated and LIVSAYIS must be started right away, you must also receive antibiotics.
    - If you had a meningococcal vaccine in the past, you might need additional vaccination before starting LIVSAYIS. Your healthcare provider will decide if you need additional meningococcal vaccination.
    - Meningococcal vaccines do not prevent all meningococcal infections. **Call your healthcare provider or get emergency medical help right away if you get any of these signs and symptoms of a meningococcal infection:** fever, fever and a rash, fever with high heart rate, headache with nausea or vomiting, headache and a fever, headache with a stiff neck or stiff back, confusion, muscle aches with flu-like symptoms, or eyes sensitive to light

Your healthcare provider will give you a Patient Safety Card about the risk of serious meningococcal infection. Carry it with you at all times during treatment and for 12 months after your last LIVSAYIS dose. Your risk of meningococcal infection may continue for several months after your last dose of LIVSAYIS. It is important to show this card to any healthcare provider who treats you. This will help them diagnose and treat you quickly.

LIVSAYIS is only available through a program called the LIVSAYIS Risk Evaluation and Mitigation Strategy (REMS). Before you can receive LIVSAYIS, your healthcare provider must: enroll in the LIVSAYIS REMS program; counsel you about the risk of serious meningococcal infections; give you the Patient Guide that explains your risk of serious meningococcal infections; give you a Patient Safety Card that has important information about the signs and symptoms of meningococcal infections; make sure that you are vaccinated against serious infections caused by meningococcal bacteria, and that you receive antibiotics if you need to start LIVSAYIS right away and you are not up-to-date on your vaccines.

LIVSAYIS may also increase the risk of other types of serious infections especially those caused by encapsulated bacteria, including **Streptococcus pneumoniae, Haemophilus influenzae,** and **Neisseria gonorrhoeae**. Certain people may be at risk of serious infections with gonorrhea. Talk to your healthcare provider about whether you are at risk for gonorrhea infection, about gonorrhea prevention, and regular testing.

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

Who should not receive LIVSAYIS?

- **Do not receive LIVSAYIS** if you have a serious meningococcal infection when you are starting LIVSAYIS treatment.

Before you receive LIVSAYIS, tell your healthcare provider about all your medical conditions, including if you:

- Have an infection or fever
- Are pregnant or plan to be pregnant. It is not known whether LIVSAYIS will harm your unborn baby
- Are breastfeeding or plan to breastfeed. It is not known if LIVSAYIS passes into your breast milk

Tell your healthcare provider about medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. 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**.**

The most common side effects in people with gMG treated with LIVSAYIS include: injection site reactions, viral infections and urinary tract infections.
Tell your healthcare provider about any side effect that bothers you or that does not go away. These are not all the possible side effects of LIVSAYIS. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Please see full Prescribing Information, including Boxed WARNING, and Medication Guide for LIVSAYIS.

What is LIVSAYIS?
LIVSAYIS is a prescription medicine used to treat a disease called generalized myasthenia gravis (gMG) in adults who are anti-acetylcholine receptor (AChR) antibody positive.

It is not known if LIVSAYIS is safe and effective in children.

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 Livsayis™ (cemdisiran) for the treatment of generalized myasthenia gravis ("gMG"); uncertainty of the utilization, market acceptance, and/or commercial success of Regeneron's Products (such as Livsayis) 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; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's Product Candidates and new indications for Regeneron's Products (such as Livsayis); 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 (such as Livsayis) and Regeneron's Product Candidates 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 (such as Livsayis) 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) as well as Regeneron's license agreement with Alnylam Pharmaceuticals, Inc. referenced in this press release, 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 the Company or other parties and pending or future litigation or patent office proceedings relating thereto (including without limitation patent litigation and other related proceedings relating to Regeneron's Products), 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 September 30, 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).

Contacts:

Media Relations
Tammy Allen
Tel: +1 914-306-2698
tammy.allen@regeneron.com

Investor Relations
Mark Hudson
Tel: +1 914-847-3482
mark.hudson@regeneron.com

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