Vor Completes Enrollment of Global Phase 3 UPSTREAM MG Trial and Expands Telitacicept Global Franchise into Ocular Myasthenia Gravis

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Topline results from UPSTREAM MG's 24-week primary endpoint remain on track for first half of 2027

Global Phase 3 ocular myasthenia gravis (oMG) registrational trial, UPSTREAM oMG, broadens telitacicept development across the myasthenia gravis (MG) spectrum; first patient dosing anticipated in the first half of 2027

BOSTON, Sept. 08, 2026 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage biotechnology company transforming the treatment of autoimmune diseases, today announced the completion of enrollment in UPSTREAM MG, its global Phase 3 registrational trial evaluating telitacicept for the treatment of generalized myasthenia gravis (gMG). Topline results from the 24-week primary endpoint remain expected in the first half of 2027.

Vor Bio also plans to initiate UPSTREAM oMG, a global Phase 3 registrational trial evaluating telitacicept for the treatment of patients with ocular myasthenia gravis, with the first patient dosing anticipated in the first half of 2027. The program is being advanced as a registrational expansion of Vor Bio's existing MG development program, supporting potential future label expansion across the myasthenia gravis spectrum and builds on the broader clinical experience with telitacicept in MG, including RemeGen's ongoing Phase 3 trial in oMG in China.

"Completing enrollment in UPSTREAM MG brings us closer to a defining Phase 3 readout in the first half of 2027," said Jean-Paul Kress, M.D., Chairman and Chief Executive Officer of Vor Bio. "We are deeply grateful to the patients whose participation and commitment made this milestone possible, as well as to our investigators, key opinion leaders, partners, and the Vor team for their exceptional execution. Additionally, our decision to expand into ocular MG reflects our growing conviction that telitacicept has the potential to be a foundational therapy across MG. More broadly, we see expansion in MG as the first step of a much larger campaign for telitacicept. With multiple global programs advancing, we are executing on our near-term priorities while building a broad, enduring autoimmune franchise."

"Telitacicept represents a promising and differentiated approach to the treatment of myasthenia gravis through targeted B-cell modulation," said James F. Howard, Jr., M.D., UNC School of Medicine. "By inhibiting both BAFF and APRIL, telitacicept has the potential to address key drivers of disease biology through a broader mechanism than current downstream therapies focused primarily on circulating IgG reduction and without complete B-cell depletion. The clinical data generated to date provide a strong rationale for continued development. I look forward to the results of this global study and the potential impact they may have for our patients living with myasthenia gravis. The expansion of the development program into ocular myasthenia gravis is also an important step forward. Ocular symptoms can significantly affect daily functioning and quality of life, and there remains a need for treatment options that may reduce reliance on long-term corticosteroid use while helping to effectively manage disease symptoms."

"Individuals living with myasthenia gravis need more treatment options that can provide meaningful and sustained control of a disease that can profoundly affect everyday life. Completing enrollment in UPSTREAM MG is an important milestone, made possible by the commitment of patients, families and the broader MG community for advancing research," said Samantha Masterson, President and CEO of the Myasthenia Gravis Foundation of America. "We look forward to the results and are encouraged by the expansion into ocular MG, which extends the effort to bring new treatment options to patients across the MG spectrum."

About UPSTREAM MG
UPSTREAM MG is a global, randomized, double-blind, placebo-controlled Phase 3 registrational trial evaluating the efficacy and safety of telitacicept in adults with gMG. The trial includes a 24-week placebo-controlled period followed by a 48-week open-label extension designed to evaluate the longer-term efficacy and safety of telitacicept.

The primary endpoint is change from baseline in Myasthenia Gravis Activities of Daily Living (MG-ADL) score at Week 24. Key secondary endpoints include measures of muscle strength, clinical response and patient-reported outcomes.

The global program builds on the Phase 3 trial of telitacicept conducted by RemeGen in China, in which telitacicept demonstrated statistically significant and clinically meaningful improvements in MG-ADL and Quantitative Myasthenia Gravis (QMG) scores at Week 24. In the subsequent open-label extension, clinical improvement continued through Week 48.

About Generalized Myasthenia Gravis
gMG is a rare, chronic autoimmune neuromuscular disorder that disrupts communication between nerves and muscles, leading to muscle weakness that can impact mobility, vision, swallowing, and breathing. The disease is mediated by autoantibodies, most

commonly targeting the acetylcholine receptor (AChR) or muscle-specific kinase (MuSK), which interfere with neuromuscular transmission. While several therapies are available, many patients continue to experience persistent symptoms or intolerable side effects. As a result, there remains a significant unmet need for new therapies that offer durable efficacy, a favorable safety profile, and convenient administration to improve the quality of life for people living with gMG.

About Ocular Myasthenia Gravis
oMG is a rare, chronic autoimmune neuromuscular disorder that disrupts communication between nerves and muscles, leading to weakness of the muscles controlling the eyes and eyelids. The disease commonly manifests as ptosis, or drooping of the eyelid, and diplopia, or double vision, which can significantly impact reading, driving, working, and other activities of daily living. Like gMG, oMG is mediated by autoantibodies, most commonly targeting the acetylcholine receptor (AChR), which interfere with neuromuscular transmission. While several therapies are used to manage the disease, many patients continue to experience persistent symptoms or treatment-related side effects. As a result, there remains a significant unmet need for new therapies that offer durable efficacy, a favorable safety profile, and convenient administration to improve the quality of life for people living with oMG.

About Telitacicept
Telitacicept is a novel recombinant fusion protein designed to treat autoimmune diseases through dual inhibition of BLyS (BAFF) and APRIL - two cytokines essential to B cell and plasma cell survival. This dual-target mechanism reduces autoreactive B cells and autoantibody production, key drivers of autoimmune pathology.

Telitacicept is approved in China for systemic lupus erythematosus (SLE), rheumatoid arthritis (RA), generalized myasthenia gravis (gMG), IgA nephropathy (IgAN), and Sjögren's disease (SjD).

Vor Bio is advancing telitacicept in global Phase 3 trials in gMG and SjD to support potential regulatory approvals in the United States, Europe, and Japan.

About Vor Bio
Vor Bio is a clinical-stage biotechnology company transforming the treatment of autoimmune diseases. The Company is focused on rapidly advancing telitacicept, a novel dual-target fusion protein, through Phase 3 clinical development and potential commercialization to address serious autoantibody-driven conditions worldwide. For more information visit www.vorbio.com. Vor Bio routinely posts information that may be important to investors in the "Investors" section of its website. The Company encourages investors to consult that section of its website regularly.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The words "anticipate," "continue," "could," "design," "expect," "intend," "may," "ongoing," "plan," "potential," "should," "update," "will," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release include Vor Bio's development and commercialization plans, including initiating UPSTREAM oMG and dosing the first patient in the trial in the first half of 2027, and the anticipated timing of topline results from UPSTREAM MG's 24-week primary endpoint in the first half of 2027; telitacicept's potential to be a foundational therapy across myasthenia gravis, including potential label expansion and potential regulatory approvals in the United States, Europe, and Japan; telitacicept's potential to address key drivers of disease biology through a broader mechanism than current downstream therapies focused primarily on circulating IgG reduction and without complete B-cell depletion; telitacicept's potential to reduce reliance on long-term corticosteroid use while helping to effectively manage disease symptoms; Vor Bio's plan to build a broad, enduring autoimmune franchise; and other statements that are not historical fact.

Vor Bio may not actually achieve the plans, intentions, or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various factors, including that the data for our product candidates may not be sufficient for obtaining regulatory approval to commercialize products; we may not be able to execute our business plans, including meeting our planned clinical and regulatory milestones and timelines, and possible limitations of financial and other resources. These and other risks are described in greater detail under the caption "Risk Factors" included in Vor Bio's most recent annual or quarterly report and in other reports it has filed or may file with the Securities and Exchange Commission.

Any forward-looking statements contained in this press release speak only as of the date hereof, and Vor Bio expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise, except as may be required by law.

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