Telitacicept Demonstrates Sustained Efficacy and Favorable Safety Profile in 48-Week China Phase 3 Open-Label Extension Generalized Myasthenia Gravis Data

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Telitacicept delivered consistent quality-of-life improvement across both treatment and placebo crossover arms

100% of patients on telitacicept for 48 weeks achieved ≥2-point Myasthenia Gravis Activities of Daily Living (MG-ADL) improvement, with a mean reduction of -7.5 points

Sustained efficacy and favorable safety extension data support potential global best-in-disease profile in generalized myasthenia gravis (gMG)

BOSTON, Oct. 29, 2025 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage biotechnology company transforming the treatment of autoimmune diseases, today announced that its collaborator, RemeGen Co., Ltd (HKEX: 9995, SHA: 688331), presented 48-week open-label extension (OLE) data from its Phase 3 study in China evaluating telitacicept in patients with gMG. The results will be presented in a late-breaking session on October 29 at 10:50am PT at the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) Annual Meeting.

“The strength and consistency of these results with telitacicept in China mark a major step forward as we look to redefine long-term disease control for patients living with generalized myasthenia gravis. Achieving sustained and meaningful improvements across both treatment and crossover groups sets a new standard of care expectation globally, especially with nearly eighty-seven percent of patients reaching six-point or greater gains in MG-ADL and over seventy-one percent reaching eight-point or greater gains in QMG,” said Jean-Paul Kress, M.D., Chief Executive Officer and Chairman of the Board. “With our global Phase 3 trial now enrolling across 14 countries, we are excited to build on this momentum and work toward delivering the same transformative benefits to patients worldwide.”

The Phase 3 trial in China was a randomized, double-blind, placebo-controlled study in patients with AChR-Ab or MuSK-Ab positive gMG. Following the 24-week double-blind period, all patients entered the OLE, with those previously on placebo crossing over to telitacicept 240mg.

The primary endpoint of the study was change from baseline in MG-ADL at 24 weeks, with secondary endpoints including changes in MG-ADL and QMG (Quantitative Myasthenia Gravis) at 12, 24, 36, and 48 weeks, as well as the proportion of patients achieving clinically meaningful improvements (≥3-point decrease in MG-ADL and ≥5-point decrease in QMG) at 24 and 48 weeks. The initial 24-week double-blind treatment stage data were presented at the American Academy of Neurology (AAN) Annual Meeting 2025.

Key Findings from the 48-Week OLE:

- At week 48, patients on telitacicept throughout achieved a -7.5 mean MG-ADL change, while placebo crossover patients achieved -6.3; 96.2% of continuous patients and 90.2% of crossover patients reached ≥3-point improvement.
- At week 48, patients on telitacicept throughout achieved a -9.8 mean QMG change, while placebo crossover patients achieved -9.3; 94.2% of continuous patients and 90.2% of crossover patients reached ≥5-point improvement.
- Telitacicept demonstrated a favorable profile comparable to placebo and consistent with prior studies across other autoimmune indications, including systemic lupus erythematosus, rheumatoid arthritis, primary Sjögren's disease, and IgA nephropathy. No new safety signals were observed. Most adverse events were mild to moderate in severity.
- No injection site reactions were reported during the OLE in patients previously on telitacicept. Injection site reactions in placebo crossover patients were mild, self-resolving, and did not lead to discontinuation.

About Telitacicept

Telitacicept is a novel, investigational recombinant fusion protein designed to treat autoimmune diseases by selectively inhibiting 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. In a Phase 3 clinical trial in generalized myasthenia gravis in China, telitacicept demonstrated a placebo adjusted 4.83-point improvement in MG-ADL (Myasthenia Gravis Activities of Daily Living scale) at 24 weeks, the primary endpoint of the trial.

Telitacicept is approved in China for systemic lupus erythematosus (SLE), rheumatoid arthritis (RA), and generalized myasthenia gravis (gMG). A global Phase 3 clinical trial in gMG is currently underway across the United States, Europe, South America, and

Asia-Pacific to support potential approval in the United States, Europe, and Japan.

About Generalized Myasthenia Gravis (gMG)
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. There are approximately 90,000 people in the United States, 140,000 in Europe, and 29,000 in Japan living with the disease.

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.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The words “aim,” “anticipate,” “can,” “continue,” “could,” “design,” “enable,” “expect,” “initiate,” “intend,” “may,” “on-track,” “ongoing,” “plan,” “potential,” “should,” “target,” “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 statements regarding the potential of telitacicept to have a global best-in-disease profile in generalized myasthenia gravis (gMG) and to offer a disease modification rather than cyclical symptom management; telitacicept’s safety profile; our goal to redefine long-term disease control for patients living with gMG and deliver the same transformative benefits we see in China to patients worldwide; and telitacicept’s market opportunity.

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 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. The results of the clinical trial described in this press release is based on information reported by RemeGen; Vor Bio has not independently verified this data. 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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