Kyverna Therapeutics Announces Positive Topline Data from Registrational KYSA-8 Trial of Miv-cel (KYV-101) in Stiff Person Syndrome

December 15, 2025

Landmark results could pave the way for miv-cel to become the first FDA-approved CAR T-cell therapy for autoimmune disease; Company on track to submit BLA for stiff person syndrome in 1H 2026

Miv-cel achieved statistically significant clinical benefit across all primary and secondary endpoints, reversing disability and eliminating immunotherapies after a single dose

Miv-cel was generally well-tolerated with no high-grade CRS or ICANS observed

SPS is a debilitating, progressive autoimmune disease with no FDA-approved therapies

Company to host webcast today, December 15, 2025 at 8 am ET

EMERYVILLE, Calif., Dec. 15, 2025 (GLOBE NEWSWIRE) -- Kyverna Therapeutics, Inc. (Nasdaq: KYTX), a clinical-stage biopharmaceutical company focused on developing cell therapies for patients with autoimmune diseases, today announced positive topline data from KYSA-8, its registrational Phase 2 trial of mivocabtagene autoleucel ('miv-cel', formerly KYV-101), a fully human, autologous CD19-targeting CAR T-cell therapy with CD28 co-stimulation, in stiff person syndrome (SPS).

"We are very pleased to share transformative topline data in stiff person syndrome, which could pave the way for miv-cel to become the first and only approved therapy in SPS and CAR T-cell therapy for autoimmune disease," said Warner Biddle, Chief Executive Officer of Kyverna Therapeutics. "Today's results further cement our leadership position in the autoimmune CAR T field and add to the growing body of evidence supporting miv-cel's potential to fundamentally shift the treatment paradigm in autoimmune diseases. We look forward to submitting our BLA for SPS in the first half of 2026 with the goal of bringing this novel therapy to patients and physicians who desperately need an effective treatment for this devastating and progressive disease."

KYSA-8 Clinical Trial Summary and Topline Data Highlights

KYSA-8 is a single-arm registrational Phase 2 trial in which patients with SPS, who had an inadequate response with non-approved treatment options, received a single dose of miv-cel. A total of 26 patients were dosed and followed through the primary analysis time point (Week 16) with additional follow-up thereafter.

"Today's topline data represent a significant breakthrough in the treatment of stiff person syndrome, demonstrating miv-cel's ability to reverse progressive disability in a debilitating disease that has no approved therapies," said Naji Gehchan, Chief Medical and Development Officer of Kyverna Therapeutics. "With a single dose, miv-cel achieved highly statistically significant and sustained improvements in overall disability, mobility, and stiffness, while enabling all patients to remain free of immunotherapies. In addition, miv-cel demonstrated a well-tolerated and manageable safety profile. We believe these unprecedented results, which support our BLA submission, will have a profound impact on patients. We want to thank the patients, their families and the healthcare providers for participating in this important trial."

Efficacy

- After a single dose, miv-cel achieved statistically significant benefits on primary and all secondary efficacy endpoints at Week 16 (the primary analysis time point):
  - **Primary Endpoint:** Miv-cel demonstrated a robust and sustained improvement in mobility with a highly statistically significant improvement in timed 25-foot walk (T25FW) (p=0.0002). The median improvement was 46% at Week 16 as compared to baseline.
  - 81% of patients exceeded a 20% improvement in T25FW, a threshold considered clinically meaningful.
  - **Secondary Endpoints:** highly statistically significant benefit (all p-values <0.0001) was also achieved across all secondary endpoints, including the Modified Rankin Scale (mRS), Distribution-of-stiffness Index (DSI), Hauser Ambulation Index (HAI), and Heightened Sensitivity Scale (HSS).
- Of the 12 patients who required a walking aid-device prior to treatment, 67% no longer needed assistance to walk at Week 16.
- 100% of patients remained free of immunotherapies, and no patients required rescue therapy as of the last follow up, highlighting miv-cel's potential to provide unprecedented clinical benefit while significantly reducing or eliminating chronic treatment burden.

Safety

- Miv-cel was well-tolerated, with no high-grade cytokine release syndrome (CRS) or immune effector cell-associated neurotoxicity syndrome (ICANS) observed.
- Grade 3/4 neutropenia, a known adverse event associated with CAR T treatments, was observed in certain patients and was manageable.

“I’m excited to see this trial bring to light a highly debilitating neurologic autoimmune disease that impacts 6,000 patients in the U.S.,” said Amanda Piquet, M.D., FAAN, Director of Autoimmune Neurology, University of Colorado Anshutz School of Medicine, Céline Dion Foundation Endowed Chair, and lead investigator of the KYSA-8 trial. “Patients with SPS often rely on burdensome, chronic treatments with significant side effects, and the majority still face progressive disease that can lead to loss of independence, diminished quality of life, and, in some cases, permanent disability. For these reasons, miv-cel’s ability to significantly improve mobility and reduce stiffness is both remarkable and unprecedented, bringing hope to patients and their families who deserve better treatment options.”

Based on these data, Kyverna plans to submit a Biologics License Application (BLA) to the FDA for SPS in the first half of 2026. It has received both Regenerative Medicine Advanced Therapy and Orphan Drug designations for miv-cel in this indication. The Company also plans to share the full SPS data set at a medical conference in 2026.

Investor Webcast Details

Kyverna will host a live webcast today, Monday, December 15, 2025, from 8:00-9:00 am ET to review these results.

Participants will need to register at the below-noted URL in order to listen and participate in the call. Once registered, participants will receive a dial-in phone number and unique PIN number which will be needed to join the call. The call can also be accessed via live webcast. The webcast and supporting presentation materials will be available on the "Events & Presentations" section of Kyverna's Investor Relations webpage at ir.kyvernatx.com. An archived replay will also be available on the website.

Dial-In Registration Link:
Conference Call Registration

Webcast Link:
Kyverna Topline Results for SPS Conference Call

About KYSA-8 Trial Design

The registrational Phase 2 KYSA-8 trial is an open-label, single-arm, multicenter study evaluating the safety and efficacy of miv-cel in patients with SPS. A total of 26 adult patients with SPS were dosed in the trial. Key inclusion criteria included a confirmed SPS diagnosis, stiffness index ≥2, and inadequate response to prior immunomodulatory therapies.

Patients received lymphodepletion with cyclophosphamide and fludarabine followed by a single infusion of miv-cel at a target dose of 1×10^8 CAR T cells. The primary endpoint was the change from baseline in the T25FW at Week 16. Secondary endpoints included the change from baseline in mRS, HAI, DSI, and HSS. Patients will be followed for one year.

Primary Endpoint	
Timed 25-Foot Walk (T25FW)	Validated tool capturing improvement in walking ability
Secondary Endpoints	
Modified Rankin Score (mRS)	Reduction in degree of disability
Hauser Ambulation Index (HAI)	Improvement in time and degree of assistance to complete timed 25-foot walk
Distribution of Stiffness Index (DSI)	Reduction in muscle stiffness across body regions
Heightened Sensitivity Scale (HSS)	Reduction in muscle spasms

About Stiff Person Syndrome (SPS)

SPS is a rare, progressive neurologic autoimmune disease characterized by muscle stiffness and painful muscle spasms, impacting mobility and gait. Stiffness, rigidity, and spasms in the torso, arms, and legs lead to progressive disability causing up to 80% of patients to lose mobility[1-3]. SPS has been shown to lead to permanent disability and increased risk of mortality[3]. Most patients with SPS have antibodies to glutamic acid decarboxylase 65 (GAD65) or the glycine receptor, which disrupt normal inhibitory neurotransmission, contributing to the hallmark symptoms of SPS. There are currently no FDA-approved treatments for SPS. Current treatment options include symptomatic treatments, off-label immunotherapies, such as intravenous immunoglobulin (IVIg), rituximab and plasmapheresis, as well as physical, speech, occupational therapy, supportive care, and psychiatric therapy; however, the majority of patients have inadequate or no response to these treatment options.

About mivocabtagene autoleucel (miv-cel)

Mivocabtagene autoleucel (miv-cel’, formerly KYV-101) is a fully human, autologous CD19-targeting CAR T-cell therapy with CD28 co-stimulation, designed for potency and tolerability, which is under investigation for B-cell-driven autoimmune diseases. With a single administration, miv-cel has potential to achieve deep B-cell depletion and immune system reset to deliver durable drug-free, disease-free remission in autoimmune diseases.

About Kyverna Therapeutics

Kyverna Therapeutics, Inc. (Nasdaq: KYTX) is a clinical-stage biopharmaceutical company focused on liberating autoimmune patients through the curative potential of cell therapy. Kyverna’s lead autologous CD19-targeting CAR T-cell therapy candidate, miv-cel (mivocabtagene autoleucel, KYV-101), has demonstrated the potential to fundamentally change the treatment paradigm across multiple B-cell-driven autoimmune diseases.

Kyverna is advancing its potentially first-in-class neuroimmunology franchise with its recently completed registrational trial in stiff person syndrome and an ongoing registrational trial for generalized myasthenia gravis. The Company is also harnessing other KYSA trials and investigator-initiated trials,

including in multiple sclerosis and rheumatoid arthritis, to inform the next priority indications. Additionally, its next generation pipeline includes CAR T-cell therapies deploying novel innovations to improve patient access and experience. For more information, please visit https://kyvernatx.com.

Forward-Looking Statements

Statements in this press release about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute "forward-looking statements." The words, without limitation, "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these or similar identifying words. Forward-looking statements in this press release include, without limitation, those related to: Kyverna's anticipated timing for its live webcast reporting its trial data and results and the topics expected to be discussed on the webcast; that the trial results could pave the way for miv-cel to become the first FDA-approved CAR-T cell therapy for SPS and in autoimmune diseases or set a new treatment standard in SPS; the anticipated timing for its submission of a BLA; potential first-in-class opportunities for miv-cel, including the potential to fundamentally shift the treatment paradigm across various autoimmune diseases; Kyverna's plans to submit the full SPS data set for presentation at a medical conference in 2026; miv-cel's potential, including to reverse progressive disability in SPS, to achieve results that will have a profound impact on patients, to provide unprecedented clinical benefit while significantly reducing or eliminating chronic treatment burden, to become the first and only approved CAR T-cell therapy in SPS and in autoimmune disease and to achieve deep B-cell depletion and immune system reset to deliver durable drug-free, disease-free remission in autoimmune diseases; Kyverna's potential first-in-class class neuroimmunology CAR T franchise and Kyverna's next generation pipeline and innovations to improve patient access and experience. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: uncertainties related to market conditions, the possibility that results from prior clinical trials, named-patient access activities and preclinical studies may not necessarily be predictive of future results; the possibility that the FDA or other regulatory agencies may require additional trials or studies to support its intended BLA submission; intellectual property rights; and other factors discussed in the "Risk Factors" section of Kyverna's most recent Annual Report on Form 10-K and Quarterly Reports on Form 10-Q that Kyverna has filed or may subsequently file with the U.S. Securities and Exchange Commission. Any forward-looking statements contained in this press release are based on the current expectations of Kyverna's management team and speak only as of the date hereof, and Kyverna specifically disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

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