Kyverna Therapeutics Receives U.S. FDA RMAT Designation for KYV-101 in the Treatment of Patients With Progressive Myasthenia Gravis

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The application was evaluated based on the positive clinical outcomes in patients treated in Germany under the named-patient program

The Regenerative Medicine Advanced Therapies designation allows Kyverna to leverage on more expedited meetings and more senior FDA leadership involvement throughout the development cycle for KYV-101

EMERYVILLE, Calif., Aug. 12, 2024 /PRNewswire/ -- Kyverna Therapeutics, Inc. (Kyverna), a patient-centered, clinical-stage biopharmaceutical company focused on developing cell therapies for patients suffering from autoimmune diseases, announced today the designation as Regenerative Medicine Advanced Therapy (RMAT) by the U.S. Food and Drug Administration (FDA) for its autologous, fully human CD19 chimeric antigen receptor (CAR) T-cell product candidate, KYV-101, to be used for the treatment of patients suffering from progressive myasthenia gravis.

"The RMAT designation underscores the attention and interest by the FDA in the development of potentially transforming therapies targeting a severe autoimmune disease such as myasthenia gravis," said Srikanth Muppidi, M.D., a neuromuscular disorder specialist at Stanford Medicine in Palo Alto, CA, and a principal investigator in the KYSA-6 trial. "We are witnessing an era of profound changes in the approach to autoimmune conditions and ultimately, we hope this leads to a symptom-free state for patients."

"We are very happy with the constructive scientific rapport established between Kyverna and the FDA," said Peter Maag, Ph.D., chief executive officer at Kyverna. "We believe the RMAT designation may ultimately add to our rigorous approach to KYV-101 development in the hope of benefitting the most deserving patients."

About Myasthenia Gravis (MG)
Myasthenia gravis is an autoimmune disorder associated with muscle weakness in tissues throughout the body, potentially manifesting in partial paralysis of eye movements, problems in chewing and swallowing, respiratory problems, speech difficulties and weakness in skeletal muscles. MG patients develop antibodies that lead to an immunological attack on critical signaling proteins at the junction between nerve and muscle cells, thereby inhibiting the ability of nerves to communicate properly with muscles. The symptoms of the disease can be transient and in the early stages of the disease can remit spontaneously. However, as the disease progresses, symptom-free periods become less frequent and disease exacerbations can last for months. Disease symptoms reach their maximum levels within two to three years in approximately 80% of patients. Up to 20% of MG patients experience respiratory crisis at least once in their lives[1].

About KYV-101
KYV-101 is an autologous, fully human CD19 CAR T-cell product candidate for use in B cell-driven autoimmune diseases. The CAR in KYV-101 was designed by the National Institutes of Health (NIH) to improve tolerability and tested in a 20-patient Phase 1 trial in oncology. Results were published by the NIH in Nature Medicine[2].

KYV-101 is currently being evaluated in sponsored, open-label, Phase 1/2 and Phase 2 trials of KYV-101 in the United States and Germany across two broad areas of autoimmune disease: rheumatology and neurology.

With 50 patients treated so far with the CAR in KYV-101 in both oncological and autoimmune conditions at more than 15 locations in Europe and the U.S., we believe that the differentiated properties of KYV-101 are critical for the potential success of CAR T cells as autoimmune disease therapies.

KYV-101 is also being evaluated in investigator-initiated trials for multiple indications in multiple geographies.

About Kyverna Therapeutics
Kyverna Therapeutics, Inc. (Nasdaq: KYTX) is a patient-centered, clinical-stage biopharmaceutical company focused on developing cell therapies for patients suffering from autoimmune diseases.

Our lead CAR T-cell therapy candidate, KYV-101 is advancing through clinical development with sponsored clinical trials across two broad areas of autoimmune disease: rheumatology and neurology, including Phase 2 trials for stiff person syndrome, multiple sclerosis and myasthenia gravis, a Phase 1/2 trial for systemic sclerosis, and two ongoing multi-center, open-label Phase 1/2 trials in the United States and Germany for patients with lupus nephritis.

Kyverna's pipeline includes next-generation CAR T-cell therapies in both autologous and allogeneic formats with properties intended to be well suited for use in B cell-driven autoimmune diseases.

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: the potential impact of the clinical outcomes from the named-patient activities; the potential that the results of the KYSA-6 trial could drastically change the treatment landscape for myasthenia gravis; Kyverna's goals to develop certain paradigm-shifting treatment options; the potential for KYV-101 to provide durable, immunosuppressant-free remission for myasthenia gravis patients; Kyverna's beliefs about the differentiated properties of KYV-101; and Kyverna's clinical trials and named-patient activities. 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, 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.

For more information, please visit https://kyvernatx.com.

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[1] Payus et al., *Am J Case Rep*. 2021; 22: e928419-1–e928419-4.

[2] Brudno et al., *Nature Medicine* 2020; 26:270-280.

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