Vanda Pharmaceuticals Announces FDA Rare Pediatric Disease Designation for Investigational Therapy for Charcot-Marie-Tooth Disease Type 2S

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WASHINGTON, July 7, 2026 /PRNewswire/ -- Vanda Pharmaceuticals Inc. (Vanda) (Nasdaq: VNDA) today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to VCA-894A, Vanda's investigational antisense oligonucleotide therapy for the treatment of Charcot-Marie-Tooth disease, axonal, type 2S (CMT2S), a rare, serious, and progressive inherited neurological disorder. The designation was granted by the FDA's Office of Orphan Products Development and Office of Pediatric Therapeutics.

VCA-894A is being developed for a patient who was first diagnosed at an early age with a rare subtype of Charcot-Marie-Tooth (CMT) disease known as CMT2S.[1] CMT2S is an inherited neuromuscular disorder that progressively leads to muscle weakness and loss of motor function, and has an estimated prevalence of less than 1 in 1,000,000 worldwide.[2] VCA-894A's therapeutic target is a unique variant of CMT2S not yet observed in any other patient. The severity and clinical presentations of CMT2S are influenced by the diverse genetic variants associated with CMT disease.

The FDA determined that CMT2S qualifies as a rare pediatric disease because it is a serious or life-threatening condition whose manifestations primarily affect individuals from birth through 18 years of age and it meets the statutory definition of a rare disease.

"CMT2S is a devastating inherited neuropathy for which patients and families have limited treatment options," said Mihael H. Polymeropoulos, M.D., President, Chief Executive Officer and Chairman of Vanda. "The FDA's Rare Pediatric Disease Designation recognizes the significant unmet medical need in this patient population and supports our efforts to develop a potentially transformative therapy for patients affected by this rare disease."

CMT2S is a rare genetic disorder characterized by chronic progressive sensory and motor impairment that can lead to severe disability, including loss of ambulation. The FDA noted in its designation letter that the serious manifestations of the disease primarily affect pediatric patients.

The Rare Pediatric Disease Designation is intended to encourage the development of new therapies for serious or life-threatening rare diseases that primarily affect children. Under the Rare Pediatric Disease Priority Review Voucher program, a sponsor whose product ultimately meets all statutory requirements may become eligible to receive a priority review voucher upon approval of a qualifying marketing application. Eligibility for any future priority review voucher will be determined at the time of a marketing application's review and approval.

Vanda is advancing development of its investigational therapy for CMT2S and will continue to work closely with the FDA regarding the development program.

References:

1. Smieszek, S. *et al. Potential ASO-based personalized treatment for Charcot-Marie-Tooth disease type 2S*. Mol Ther Nucleic Acids 36, 102479 (2025).

2. Charcot-Marie-Tooth disease type 2S. Orpha.net (2025). https://www.orpha.net/en/disease/detail/443073

About Vanda Pharmaceuticals Inc.

Vanda is a leading global biopharmaceutical company focused on the development and commercialization of innovative therapies to address high unmet medical needs and improve the lives of patients. For more on Vanda Pharmaceuticals Inc., please visit www.vandapharma.com and follow us on X @vandapharma.

About Charcot-Marie-Tooth Disease Type 2S (CMT2S)

Charcot-Marie-Tooth disease type 2S is a rare inherited neurological disorder characterized by progressive degeneration of peripheral nerves, resulting in sensory and motor impairment. The disease can lead to significant functional limitations and disability over time.

About VCA-894A

VCA-894A is a 2'-O-methoxyethyl (MOE) phosphorothioate oligonucleotide sodium salt. VCA-894A specifically targets a cryptic splice site variant within IGHMBP2, which causes CMT2S. ASOs may have broad applicability in addressing a number of disorders, from nervous system treatments to systemic treatments.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Various statements in this press release, including but not limited to statements regarding the estimated prevalence of CMT2S, the therapeutic potential of VCA-894A for patients affected by CMT2S, a sponsor's eligibility to receive a priority review voucher, and Vanda's clinical development and regulatory plans and strategies for VCA-894A, are "forward-looking statements" under the securities laws. All statements other than statements of historical fact are statements that could be deemed forward-looking statements. Forward-looking statements are based upon current expectations and assumptions that involve risks, changes in circumstances and uncertainties. Important factors that could cause actual results to differ materially from those reflected in Vanda's forward-looking statements include, among others, the accuracy of the estimates of the prevalence of CMT2S worldwide, the efficacy of VCA-894A in the treatment of patients with CMT2S, Vanda's ability to complete the development and obtain regulatory approval of VCA-894A for the treatment of CMT2S, a product's ability to meet the statutory requirements necessary for its sponsor to become eligible to receive a priority review voucher and the ultimate receipt of the voucher, and the ability of Vanda and the FDA to continue to work closely together with regard to the VCA-894A development program. Therefore, no assurance can be given that the results or developments anticipated by Vanda will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, Vanda. Forward-looking statements in this press release

should be evaluated together with the various risks and uncertainties that affect Vanda's business and market, particularly those identified in the "Cautionary Note Regarding Forward-Looking Statements", "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of Vanda's most recent Annual Report on Form 10-K, as updated by Vanda's subsequent Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other filings with the U.S. Securities and Exchange Commission, which are available at www.sec.gov.

All written and verbal forward-looking statements attributable to Vanda or any person acting on its behalf are expressly qualified in their entirety by the cautionary statements contained or referred to herein. Vanda cautions investors not to rely too heavily on the forward-looking statements Vanda makes or that are made on its behalf. The information in this press release is provided only as of the date of this press release, and Vanda undertakes no obligation, and specifically declines any obligation, to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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