Vanda Pharmaceuticals Announces Orphan Drug Designation Granted for VPO-227, a Novel Candidate for the Treatment of Cholera

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WASHINGTON, Oct. 21, 2022 /PRNewswire/ -- Vanda Pharmaceuticals Inc. (Vanda) (Nasdaq: VNDA) today announced the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation for VPO-227 for the treatment of cholera.

While the incidence of cholera in the U.S. is low, cholera is a widespread infectious diarrheal disease with estimated 1.4 to 4 million cases worldwide, resulting in 21,000 to 143,000 deaths annually.[1] Many of these deaths occur in children, as diarrheal diseases are the second leading cause of death globally among children under 5 years of age.[2] Cholera remains a major public health challenge and recognized unmet medical need despite decades-long public health efforts.

"This designation is an important milestone in the development of VPO-227 and highlights the need for potential new treatment options for patients who suffer from cholera and other conditions of water hyper-excretion," said Mihael H. Polymeropoulos, M.D., Vanda's President, CEO and Chairman of the Board.

VPO-227 is a small-molecule with a novel mechanism of action which blocks the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) ion channel. CFTR plays a pivotal role in cholera-induced intestinal fluid loss as it is affected by the cholera toxin. VPO-227, therefore, has the potential to be an orally administered treatment for cholera. In a recent publication in the journal *Toxins,* the authors showed that oral VPO-227 protects animals from a lethal cholera challenge using two separate cholera strains.[3]

Orphan Drug Designation is granted by the FDA to investigational therapies addressing rare medical conditions and provides benefits to drug developers, including assistance in the drug development process, tax credits for clinical costs, exemptions from certain FDA fees and the potential for seven years of post-approval marketing exclusivity.

Additionally, in order to incentivize development in tropical diseases, including cholera, the FDA has established the Tropical Disease Priority Review Voucher Program, which grants a transferable Priority Review Voucher to sponsors of eligible new drug applications for products treating tropical diseases. Vanda expects to submit an Investigational New Drug (IND) application to the FDA for VPO-227 in 2023.

References

1. Ali, M., Nelson, A. R., Lopez, A. L., & Sack, D. A. (2015). Updated global burden of cholera in endemic countries. *PLOS Neglected Tropical Diseases*, 9(6). https://doi.org/10.1371/journal.pntd.0003832
2. Harris, J. B., LaRocque, R. C., Qadri, F., Ryan, E. T., & Calderwood, S. B. (2012). Cholera. *The Lancet*, 379(9835), 2466–2476. https://doi.org/10.1016/s0140-6736(12)60436-x
3. Rivera-Chávez, F., Meader, B. T., Akosman, S., Koprivica, V, & Mekalanos, J. J. (2022). A potent inhibitor of the cystic fibrosis transmembrane conductance regulator blocks disease and morbidity due to toxigenic vibrio cholerae. *Toxins*, 14(3), 225. https://doi.org/10.3390/toxins14030225

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 Twitter @vandapharma.

About VPO-227

VPO-227 (formerly BPO-27) is a small molecule CFTR inhibitor in development for the treatment of secretory disorders, including cholera. Vanda entered into a license agreement with University of California, San Francisco (UCSF) in 2017, under which Vanda acquired an exclusive worldwide license from UCSF to develop and commercialize a portfolio of CFTR activators and inhibitors, including the activator VPO-227. CFTR activators and inhibitors may have broad applicability in addressing a number of disorders, including chronic dry eye, constipation, polycystic kidney disease, cholestasis, and secretory diarrheas.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Various statements in this press release, including, but not limited to statements regarding Vanda's clinical development and regulatory plans and strategies for VPO-227 and the safety and efficacy of VPO-227, 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, Vanda's ability to complete the IND for VPO-227 and submit it the FDA during 2023, the ability of VPO-227 to safely and effectively treat cholera in humans and Vanda's ability to complete the clinical development of, and obtain regulatory approval for, VPO-227. 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 Annual Report on Form 10-K for the fiscal year ended December 31,

2021, 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.

Corporate Contact:
Kevin Moran
Senior Vice President, Chief Financial Officer and Treasurer
Vanda Pharmaceuticals Inc.
202-734-3400
pr@vandapharma.com

Elizabeth Van Every
Head of Corporate Affairs
Vanda Pharmaceuticals Inc.
202-734-3400
pr@vandapharma.com