Vanda Pharmaceuticals Announces that U.S. Food and Drug Administration Accepts New Drug Application for Tradipitant for the Treatment of Gastroparesis

December 4, 2023

WASHINGTON, Dec. 4, 2023 /PRNewswire/ -- Vanda Pharmaceuticals Inc. (Vanda) (NASDAQ: VNDA) today announced that the U.S. Food and Drug Administration (FDA) has accepted the filing of Vanda's New Drug Application (NDA) for tradipitant for the treatment of symptoms of gastroparesis.

The FDA has set September 18, 2024 as the target date for its decision under the Prescription Drug User Fee Act (PDUFA). If approved, tradipitant will be the first novel drug to be approved by the FDA for the treatment of gastroparesis in over 40 years and to be accepted for review by the FDA for gastroparesis in over 30 years.

Gastroparesis is a serious medical condition characterized by delayed gastric emptying associated with the symptoms of nausea, vomiting, bloating, fullness after meals and abdominal pain, along with significant impairment of social and occupational functioning. The estimated prevalence of gastroparesis in the U.S. is approximately 6 million patients, many of whom remain undiagnosed.[1]

In support of this application, the FDA accepted for review Vanda's non-animal preclinical toxicology data derived from microphysiological systems that was provided by Vanda in the absence of a nine-month toxicity dog study. Vanda believes that this is an important milestone in its multi-year effort to convince the FDA to move away from animal toxicology studies and instead adopt novel and advanced human-relevant technologies.

"We are very pleased with the FDA's acknowledgment of the completeness of our application and we look forward to a substantive review. Tradipitant, if approved, will be the first novel drug for patients with gastroparesis since 1979," said Mihael H. Polymeropoulos, M.D., Vanda's President, CEO and Chairman of the Board. "We are also encouraged by the FDA's decision to accept for filing this application that includes non-animal preclinical toxicity data from advanced human-relevant microphysiological systems in the absence of a nine-month dog study."

The NDA submission includes results from clinical efficacy studies 2301 and 3301, evidence from a large 12-week open label study and data from the Expanded Access program.

The safety database in this application now under review includes over 1,000 patients with exposures of up to 12-weeks, as well as data from the Expanded Access program with patient exposures of over two years.

The FDA's action on this application will mark the third NDA or supplemental New Drug Application (sNDA) decision expected by Vanda in 2024; a sNDA for HETLIOZ® for the treatment of insomnia characterized by difficulties with sleep initiation has a PDUFA target action date of March 4, 2024 and a sNDA for Fanapt® for the acute treatment of manic or mixed episodes associated with bipolar I disorder has a PDUFA target action date of April 2, 2024.

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 Tradipitant

Tradipitant is an NK-1R antagonist licensed by Vanda from Eli Lilly and Company. Tradipitant is currently in clinical development for gastroparesis and motion sickness. The FDA has imposed a partial clinical hold on tradipitant clinical protocols of longer than 12 weeks duration.

About Gastroparesis

Gastroparesis is a serious medical condition characterized by delayed gastric emptying associated with the symptoms of nausea, vomiting, bloating, fullness after meals and abdominal pain, along with significant impairment of social and occupational functioning. The estimated prevalence of gastroparesis in the U.S. is approximately 6 million patients, many of whom remain undiagnosed.[1] Gastroparesis affects mostly women and it can be of diabetic, idiopathic or other etiology. The only FDA approved treatment for gastroparesis is metoclopramide, approved in 1979, which due to its potential of severe side effects carries a black box warning and limitations of use of no more than 3 months. Patients are faced with limited therapeutic options and clinical guidelines recommend, in addition to metoclopramide, the off label use of different drugs including erythromycin, domperidone (not approved in the U.S.), botulinum toxin injections, gastric stimulators and a variety of surgical procedures in an effort to relieve even temporarily some of the symptoms of the disease.[2] Gastroparesis treatment represents a significant unmet medical need as underscored by the testimonies of interested parties and advocacy organizations including the International Foundation for Gastrointestinal Disorders (IFFGD) and Gastroparesis Patient Association for Cures and Treatments, Inc. (G-Pact).

References

1. Rey, E., Choung, R. S., Schleck, C. D., Zinsmeister, A. R., Talley, N. J., & Locke, G. R., 3rd (2012). Prevalence of hidden gastroparesis in the community: the gastroparesis "iceberg". *Journal of neurogastroenterology and motility*, *18*(1), 34–42. https://doi.org/10.5056/jnm.2012.18.1.34
2. Camilleri, M., Chedid, V., Ford, A. C., Haruma, K., Horowitz, M., Jones, K. L., Low, P. A., Park, S. Y., Parkman, H. P., & Stanghellini, V. (2018). Gastroparesis. *Nature reviews. Disease primers*, *4*(1), 41. https://doi.org/10.1038/s41572-018-0038-z

CAUTIONARY NOTE REGARDING FORWARD LOOKING STATEMENTS

Various statements in this press release, including, but not limited to, statements regarding the FDA's review of the NDA for tradipitant and the potential approval of tradipitant for the treatment of symptoms of gastroparesis, the prevalence of gastroparesis in the U.S., the FDA's acceptance for filing of the NDA for tradipitant in the absence of data from a nine-month toxicity dog study and Vanda's expectations regarding the timing of the FDA's decisions with respect to the NDA for tradipitant and the sNDAs for HETLIOZ® and Fanapt® 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 FDA's assessment of the adequacy of the data included in the NDA for tradipitant and its willingness to approve the NDA without data from a nine-month toxicity dog study, the accuracy of the estimates of the number of patients in the U.S. with gastroparesis, the FDA's assessment of the data included in the sNDA's for HETLIOZ® and Fanapt® and the FDA's ability to meet the PDUFA target action dates for the NDA for tradipitant and the sNDAs for HETLIOZ® and Fanapt®. 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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