FDA Accepts and Grants Priority Review of Vanda's Applications for HETLIOZ® (tasimelteon) in the Treatment of Smith-Magenis Syndrome

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WASHINGTON, Aug. 3, 2020 /PRNewswire/ -- Vanda Pharmaceuticals Inc. (Vanda) (Nasdaq: VNDA) today announced that the U.S. Food and Drug Administration (FDA) has accepted for priority review Vanda's applications for Smith-Magenis Syndrome (SMS). The applications include a Supplemental New Drug Application (sNDA) for HETLIOZ® capsules and a New Drug Application (NDA) for the liquid formulation of HETLIOZ® for the treatment of adults and children, respectively, with Smith-Magenis Syndrome (SMS). The FDA has set December 1, 2020 as the target date for its decision under the Prescription Drug User Fee Act (PDUFA-VI).

"The FDA filing of the HETLIOZ® applications for priority review marks a major milestone and brings us closer to providing a critical therapy to patients with SMS," said Mihael H. Polymeropoulos, M.D., Vanda's President and CEO. Currently, there are no approved treatments for patients with SMS, a rare orphan disorder affecting approximately 15,000 people in the U.S.

About Smith-Magenis Syndrome

Smith-Magenis Syndrome (SMS) is a developmental disorder that is caused by a small deletion of human chromosome 17p [1,2]. In more rare cases SMS is caused by a point mutation in the RAI1 gene which resides in the deleted region. SMS is estimated to affect 1/15,000-25,000 births in the U.S.[3] SMS is usually not inherited but rather is due to a de-novo deletion. Patients with SMS present with a number of physical, mental and behavioral problems. The most common symptom of SMS is a severe sleep disorder associated with significant disruption in the lives of patients and their families.

References:

1. Williams, S. R., Zies, D., Mullegama, S. V, Grotewiel, M. S., & Elsea, S. H. (2012). Smith-Magenis syndrome results in disruption of CLOCK gene transcription and reveals an integral role for RAI1 in the maintenance of circadian rhythmicity. Am.J Hum.Genet., 90(1537–6605), 941–949.
2. Gropman, A. L., Duncan, W. C., & Smith, A. C. (2006). Neurologic and developmental features of the Smith-Magenis syndrome (del 17p11.2). Pediatr.Neurol., 34(0887–8994), 337–350.
3. Orphanet ORPHA number 819.

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 HETLIOZ®

HETLIOZ® (tasimelteon) is a melatonin receptor agonist. HETLIOZ® has been granted market authorization by the U.S. Food and Drug Administration and the European Medicines Agency. For full U.S. Prescribing Information for HETLIOZ®, including indication and Important Safety Information, visit www.hetlioz.com.

HETLIOZ® IS NOT CURRENTLY APPROVED BY ANY REGULATORY AUTHORITY FOR THE TREATMENT OF SMS.

CAUTIONARY NOTE REGARDING FORWARD LOOKING STATEMENTS

Various statements in this release, including, but not limited to statements regarding the target completion date of the FDA's review of the sNDA and NDA for HETLIOZ® and Vanda's ability to make HETLIOZ® available to patients with SMS, are "forward-looking statements" under the securities laws. Forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions 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 ability of the FDA to complete its review of the applications on time and make the determination that HETLIOZ® is safe and effective in the treatment of SMS in adults and children. There can be no assurance that the actual 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. Therefore, no assurance can be given that the outcomes stated in such forward-looking statements will be achieved. 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, 2019, 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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