FDA Approves HETLIOZ® (tasimelteon) for the Treatment of Nighttime Sleep Disturbances in Smith-Magenis Syndrome

December 1, 2020

WASHINGTON, Dec. 1, 2020 /PRNewswire/ -- Vanda Pharmaceuticals Inc. (Vanda) (Nasdaq: VNDA) today announced that the U.S. Food and Drug Administration (FDA) has approved HETLIOZ® (tasimelteon) capsule and liquid formulations for the treatment of adults and children, respectively, with nighttime sleep disturbances associated with Smith-Magenis Syndrome (SMS). SMS is a rare neurodevelopmental disorder, a defining feature of which is an "inverted" circadian rhythm, making it extremely difficult for patients with SMS to sleep during the night. HETLIOZ® is the first FDA-approved medication for patients with SMS.

"The FDA approval of HETLIOZ® for the treatment of nighttime sleep disturbances in SMS would not have been accomplished without the heroic efforts of SMS patients and the efforts of their families and advocates supporting the recruitment, design, and conduct of the study," said Mihael H. Polymeropoulos M.D., Vanda's President and CEO. "We remain committed to providing this much needed therapy to patients with SMS."

The approval of HETLIOZ® for the treatment of nighttime sleep disturbances in SMS was based on a single placebo-controlled efficacy study in this rare disorder, which studied both adults with SMS taking the HETLIOZ® capsule and children with SMS taking the liquid formulation. The safety profile of HETLIOZ® in this study was similar to those seen in HETLIOZ® studies previously conducted for the treatment of Non-24-Hour Sleep-Wake Disorder, and was similar between adults and children with SMS.

"We are very excited to see HETLIOZ®, the first ever treatment approved for people with SMS, addressing the significant problem of sleep disturbances and we are happy to see this treatment used in our community," said Maggie Miller, Co-Founder and Vice President of PRISMS (Parents and Researchers Interested in Smith-Magenis Syndrome). "We thank Vanda and the FDA for partnering to help our community and we look forward to continued partnership to bring this important therapy to families with SMS."

HETLIOZ® capsules, for adults with SMS, will be immediately available and the HETLIOZ LQ ™ liquid formulation, for children with SMS, is expected to be available in the first quarter of 2021.

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.

CAUTIONARY NOTE REGARDING FORWARD LOOKING STATEMENTS

Various statements in this release, including, but not limited to statements regarding Vanda's ability to make HETLIOZ® available to patients with nighttime sleep disturbances in 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, market acceptance of HETLIOZ® as a treatment of SMS in adults and children, Vanda's dependence on third-party manufacturers to manufacture HETLIOZ® in sufficient quantities and quality, and Vanda's sales and marketing infrastructure. 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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