Vanda Pharmaceuticals Announces Agreement with FDA on Resubmission of the Application for HETLIOZ® for the Treatment of Patients with Smith-Magenis Syndrome

May 13, 2020

WASHINGTON, May 13, 2020 /PRNewswire/ -- Vanda Pharmaceuticals Inc. (Vanda) (Nasdaq: VNDA) today announced that following the completion of a Type A Meeting with the U.S. Food and Drug Administration (FDA) on May 8, 2020, it has reached agreement with the FDA to resubmit its application for HETLIOZ® (tasimelteon) for the treatment of Smith-Magenis Syndrome (SMS). As previously disclosed, Vanda received a Refusal to File letter from the FDA on March 12, 2020. The Type A Meeting resolved the outstanding issues regarding the filing of the application. Vanda plans to resubmit as soon as possible, seeking approval of the solid capsule formulation of HETLIOZ® for the treatment of adults with SMS, and the liquid formulation of HETLIOZ ® for the treatment of children with SMS*.

"We are very pleased with the outcome of the Type A Meeting," said Dr. Mihael H. Polymeropoulos, President and CEO of Vanda. "This meeting was a great example of collaboration with the agency and we appreciate FDA's thoughtful work in helping us advance the progress of our application and bring this potentially important treatment closer to use by patients with SMS."

In December 2018, Vanda reported results of the largest placebo controlled study ever conducted in patients with SMS, with HETLIOZ® patients seeing significant improvements in sleep. SMS is a developmental disorder that is frequently caused by a small deletion of human chromosome 17p.[1, 2] In some cases, SMS is caused by a point mutation in the RAI1 gene, which resides in the deleted region. SMS is estimated to affect 1 in 15,000-25,000 individuals.[3] Patients with SMS present with a number of physical, mental and behavioral issues. The most common symptom of SMS is a severe sleep disorder, which results in significant disruption in the lives of patients and their families.

*Vanda's resubmission will be in the form of a supplemental New Drug Application for the solid capsule formulation of HETLIOZ® for the treatment of adults with SMS (the sNDA) and a New Drug Application for the liquid formulation of HETLIOZ ® for the treatment of children with SMS (the NDA). Originally, Vanda submitted an sNDA covering both formulations, but the FDA requested that Vanda separate that application into an sNDA and an NDA for the different formulations.

References:

[1] Williams, SR, Zies, D, Mullegama, SV, Grotewiel, MS, & Elsea, SH. 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. 2012; 90(1537–6605), 941–949.

[2] Gropman, AL, Duncan, WC, & Smith, AC. Neurologic and developmental features of the Smith-Magenis syndrome (del 17p11.2). Pediatr Neurol. 2006; 34(0887–8994), 337–350.

[3] Greenberg F, Guzzetta V, Montes de Oca-Luna R et al: Molecular analysis of the Smith –Magenis syndrome: a possible contiguous gene syndrome associated with del(17)(p11.2). Am J Hum Genet. 1991; 49:1207 – 1218.

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 Vanda's Twitter and LinkedIn.

Corporate Contact:
AJ Jones II
Chief Corporate Affairs and Communications Officer
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

CAUTIONARY NOTE REGARDING FORWARD LOOKING STATEMENTS

Various statements in this release, including, but not limited to statements regarding Vanda's HETLIOZ® program for the treatment of SMS and plans to resubmit the sNDA and the NDA related to such program, 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, Vanda's ability to complete the preparation of and submit the sNDA and the NDA; the FDA's acceptance and review of such filings; Vanda's ability to obtain FDA approval of HETLIOZ® for the treatment of SMS in adults and children; and other factors that are described in the "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 and quarterly report on Form 10-Q for the quarter ended March 31, 2020, which are on file with the SEC and available on the SEC's website at www.sec.gov. In addition to the risks described above and in Vanda's annual report on Form 10-K and quarterly reports on Form 10-Q, other unknown or unpredictable factors also could affect Vanda's results. 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 and estimates will be achieved.

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 release is provided only as of the date of this 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.

View original content:http://www.prnewswire.com/news-releases/vanda-pharmaceuticals-announces-agreement-with-fda-on-resubmission-of-the-application-for-hetlioz-for-the-treatment-of-patients-with-smith-magenis-syndrome-301058197.html

SOURCE Vanda Pharmaceuticals Inc.