



Vanda Pharmaceuticals Receives FDA Approval to Proceed with Investigational New Drug VSJ-110 for Allergic Conjunctivitis

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WASHINGTON, Oct. 26, 2020 /PRNewswire/ -- Vanda Pharmaceuticals Inc. (Vanda) (Nasdaq: VVDA) today announced that the U.S. Food and Drug Administration (FDA) has approved the Investigational New Drug (IND) application to evaluate Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) activator VSJ-110 (previously known as CFTR_{act}-K267) for the treatment of allergic conjunctivitis.

"This is an exciting milestone in the course of our collaboration with University of California, San Francisco (UCSF) and Dr. Alan Verkman towards the development of human therapeutics targeting the Cystic Fibrosis Transmembrane Conductance Regulator," said Mihael H. Polymeropoulos, M.D., Vanda's President and CEO. "Initiation of the clinical program for VSJ-110 marks the beginning of Vanda's development of therapeutics in ophthalmology exploring the compound's novel dual anti-inflammatory and prosecretory mechanism of action."

The initial Phase II study in human volunteers will evaluate the acute anti-inflammatory effects of VSJ-110 in an ocular allergic challenge model, and will evaluate the prosecretory effects using standard tear production assessments.

The results from this Phase II study will help guide further development of VSJ-110 to treat a variety of ocular inflammatory conditions, including dry eye, which has an estimated worldwide prevalence of 5-20%, with about 16 million affected individuals in the United States.¹ Other potential indications include chronic inflammatory eye conditions, such as atopic keratoconjunctivitis, which remain poorly addressed with current treatment options.²

"Studies in experimental animal models already provide *in vivo* evidence for the efficacy of CFTR_{act}-K267 (VSJ-110) in stimulating chloride secretion and reversing corneal epithelial injury in dry eye," said Alan Verkman, M.D., Ph.D., Professor of Medicine at UCSF.

"It is exciting to see Vanda pursue a unique and novel therapeutic mechanism of action to address common inflammatory eye conditions that impact such a significant number of patients," said Julie Schallhorn, M.D., M.S., Assistant Professor of Ophthalmology at UCSF. "The mechanism of action of VSJ-110, that could address both the aqueous deficiency and inflammatory components of dry eye disease, has the potential to revolutionize how we care for dry eye patients."

Vanda plans to initiate enrollment in the Phase II study by the end of 2020 and anticipates results of this study in 2021.

About VSJ-110

VSJ-110 is a small molecule nanomolar potency CFTR activator. VSJ-110 has shown efficacy in a dry eye model³ and exhibited anti-inflammatory properties in both *in vitro* and *in vivo* assays.

Vanda entered into a license agreement with 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 VSJ-110. 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.

References

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2. Pedram, H., Reza, D. (2019, September). Vernal keratoconjunctivitis. UpToDate. <https://www.uptodate.com/contents/vernal-keratoconjunctivitis>.
3. Chen, X., Lee, S., Zhang, T., Duan, T., Pasricha, N. D., Schallhorn, J. M., Levin, M. H., Koprivica, V., & Verkman, A. S. (2020). Nanomolar Potency Aminophenyltriazine CFTR Activator Reverses Corneal Epithelial Injury in a Mouse Model of Dry Eye. *Journal of ocular pharmacology and therapeutics: the official journal of the Association for Ocular Pharmacology and Therapeutics*, 36(3), 147–153. <https://doi.org/10.1089/jop.2019.0087>

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.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Various statements in this press release, including, but not limited to statements regarding the VSJ-110 clinical development program, the timeline and anticipated findings of the VSJ-110 Phase II study, the potential for VSJ-110 to be a safe and effective treatment for patients with allergic conjunctivitis, dry eye and atopic keratoconjunctivitis, 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 enroll patients for, and

successfully conduct, the VSJ-110 Phase II study for the treatment of allergic conjunctivitis in the midst of the global COVID-19 pandemic; Vanda's ability to enroll patients for, and successfully conduct, studies of VSJ-110 in additional indications; Vanda's ability to complete the clinical development of and obtain regulatory approval of VSJ-110 for the treatment of allergic conjunctivitis and additional indications. 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. 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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