Vanda Pharmaceuticals Granted Orphan Designation for Imsidolimab for the Treatment of Generalized Pustular Psoriasis by the European Commission

August 24, 2026

WASHINGTON, Aug. 24, 2026 /PRNewswire/ -- Vanda Pharmaceuticals Inc. (Vanda) (Nasdaq: VNDA) today announced that its investigational drug imsidolimab has received Orphan Designation from the European Commission, based on the positive opinion received from the European Medicines Agency's (EMA) Committee for Orphan Medicinal Products (COMP), for the treatment of Generalized Pustular Psoriasis (GPP), which is a severe, chronic, and potentially life-threatening inflammatory skin disease driven by dysregulation in the interleukin-36 (IL-36) signaling pathway.[1] The US Food and Drug Administration (FDA) and Japan's Ministry of Health, Labour and Welfare have previously granted Orphan Drug Designation to imsidolimab for the treatment of GPP.

This marks the first time the European Commission has granted orphan designation recognition for a drug to treat GPP in the European Union (EU). GPP is clinically distinct from plaque psoriasis and is characterized by widespread pustular eruptions, systemic inflammation, and serious complications that can lead to increased mortality.[2,3,4] Imsidolimab inhibits IL-36 receptor signaling, addressing the deficiency in the endogenous IL-36 receptor antagonist commonly observed in patients with GPP.

Orphan Designation in the EU is granted by the European Commission following an opinion from EMA's COMP for medicines intended for the diagnosis, prevention, or treatment of life-threatening or chronically debilitating rare conditions. Among other criteria, the condition must affect fewer than 5 in 10,000 people in the EU. Benefits include protocol assistance, reduced regulatory fees, and market exclusivity provisions in the EU following approval.

"The European Commission's Orphan Designation for imsidolimab represents an important milestone for the GPP community and for our efforts to bring this investigational therapy to patients in Europe," said Dr. Mihael H. Polymeropoulos, Vanda's President, CEO and Chairman of the Board. "With orphan designations now received in Europe, the United States and Japan, we remain focused on advancing imsidolimab for patients in these markets who need new treatment options."

This designation follows similar regulatory recognitions in the United States and Japan. Additionally, the imsidolimab Biologics License Application (BLA) for GPP is currently under review by the FDA with a target action date of December 12, 2026.

References

1. Smieszek, S. et al. Efficacy and Safety of Imsidolimab for Generalized Pustular Psoriasis. NEJM Evidence 5, (2026).
2. Ministry of Health Labour and Welfare (MHLW), "Pustular psoriasis (generalized type) (Designated Intractable Disease 37)," Japan Intractable Diseases Information Center. Accessed: Mar. 10, 2026. [Online]. Available: https://www.nanbyou.or.jp/entry/168
3. H. Fujita, R. Iwasaki, S. Tsuboi, Y. Murashiuma, and M. Akiyama, "Regional differences in the prevalence of generalized pustular psoriasis in Japan," J. Dermatol., vol. 51, no. 3, pp. 380–390, Mar. 2024, doi: 10.1111/1346-8138.17089.
4. H. Miyachi et al., "Treatments and outcomes of generalized pustular psoriasis: A cohort of 1516 patients in a nationwide inpatient database in Japan," J. Am. Acad. Dermatol., vol. 86, no. 6, pp. 1266–1274, Jun. 2022, doi: 10.1016/j.jaad.2021.06.008.

About imsidolimab

Imsidolimab is a fully humanized IgG4 monoclonal antibody that inhibits IL-36 receptor signaling and is being developed for GPP, a rare orphan indication. Regulatory and patent exclusivity for imsidolimab is expected to extend into the late 2030s. Vanda holds an exclusive global license for the development and commercialization of imsidolimab.

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.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Various statements in this press release, including but not limited to statements regarding the potential benefits associated with EMA approval of an orphan drug, the therapeutic potential of imsidolimab for patients with GPP in the EU, the United States and Japan, and the anticipated timing of the completion of the FDA's review of the imsidolimab BLA 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, Vanda's ability to obtain approval of, and to successfully commercialize, imsidolimab in the EU, the United States and Japan for the treatment of patients with GPP, Vanda's ability to satisfy the conditions necessary to receive the benefits associated with EMA approval of an orphan drug, and the FDA's ability to complete its review of, and reach a decision with respect to, the imsidolimab BLA by December 12, 2026. 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.

Corporate Contact:
Kevin Moran
Senior Vice President, Chief Financial Officer and Treasurer
Vanda Pharmaceuticals Inc.
202-734-3400
pr@vandapharma.com

Jim Golden / Jack Kelleher / Dan Moore
Collected Strategies
VANDA-CS@collectedstrategies.com

View original content to download multimedia:https://www.prnewswire.com/news-releases/vanda-pharmaceuticals-granted-orphan-designation-for-imsidolimab-for-the-treatment-of-generalized-pustular-psoriasis-by-the-european-commission-302857847.html

SOURCE Vanda Pharmaceuticals Inc.