Alixorexton Granted Breakthrough Therapy Designation by U.S. FDA for the Treatment of Narcolepsy Type 1

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DUBLIN--(BUSINESS WIRE)--Jan. 6, 2026-- Alkermes plc (Nasdaq: ALKS) today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to alixorexton for the treatment of narcolepsy type 1 (NT1), based on phase 1 and phase 2 clinical data, including positive results from Vibrance-1, a large (n=92) phase 2 study evaluating alixorexton in patients with NT1. Alixorexton is the company's novel, investigational, oral, selective orexin 2 receptor (OX2R) agonist in development for the treatment of NT1, narcolepsy type 2 (NT2) and idiopathic hypersomnia (IH).

The FDA's Breakthrough Therapy designation process is designed to expedite the development and review of drugs that are intended to treat a serious condition, and where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on one or more clinically significant endpoints.[1]

"Alixorexton may offer substantial improvements over available therapy for people living with narcolepsy type 1, a community that has continued to face profound unmet medical needs despite available treatments. This Breakthrough Therapy designation underscores the strength of alixorexton's initial clinical data and supports our conviction that targeting the orexin pathway has the potential to fundamentally shift treatment expectations for central disorders of hypersomnolence. If approved, alixorexton's differentiated profile and compelling efficacy may represent a new standard of care in narcolepsy type 1. We look forward to working closely with the FDA as we plan to advance alixorexton into phase 3 development later this quarter," said Craig Hopkinson, M.D., Chief Medical Officer and Executive Vice President of Research & Development at Alkermes.

In the Vibrance-1 phase 2 study, alixorexton met the primary endpoint across all doses tested, demonstrating statistically significant, clinically meaningful and dose-dependent improvements from baseline compared to placebo in wakefulness on the Maintenance of Wakefulness Test (MWT) in patients with NT1. Alixorexton was generally well tolerated at all doses tested.

Alkermes plans to initiate the alixorexton narcolepsy global phase 3 program in the first quarter of 2026.

About Alixorexton

Alixorexton (formerly referred to as ALKS 2680) is a novel, investigational, oral, selective orexin 2 receptor (OX2R) agonist in development for the treatment of narcolepsy type 1 (NT1), narcolepsy type 2 (NT2) and idiopathic hypersomnia (IH). Orexin, a neuropeptide produced in the lateral hypothalamus, is considered to be the master regulator of wakefulness due to its activation of multiple, downstream wake-promoting pathways that project widely throughout the brain.[2] Targeting the orexin system may address excessive daytime sleepiness across hypersomnolence disorders, whether or not deficient orexin signaling is the underlying cause of disease.[3] Once-daily oral administration of alixorexton was previously evaluated in a phase 1 study in healthy volunteers and patients with NT1, NT2 and IH, and in Vibrance-1 and Vibrance-2, phase 2 studies in patients with NT1 and NT2, respectively. It is currently being evaluated in the phase 2 Vibrance-3 study in patients with IH.

About Alkermes plc

Alkermes plc, a mid-cap growth and value equity, is a global biopharmaceutical company that seeks to develop innovative medicines in the field of neuroscience. The company has a portfolio of proprietary commercial products for the treatment of alcohol dependence, opioid dependence, schizophrenia and bipolar I disorder, and a pipeline of clinical and preclinical candidates in development for neurological disorders, including narcolepsy and idiopathic hypersomnia. Headquartered in Ireland, Alkermes also has a corporate office and research and development center in Massachusetts and a manufacturing facility in Ohio. For more information, please visit Alkermes' website at www.alkermes.com.

Note Regarding Forward-Looking Statements

Certain statements set forth in this press release constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, but not limited to, statements concerning: the potential therapeutic and commercial value of alixorexton (formerly referred to as ALKS 2680) and the company's expectations, including timelines, related to the alixorexton development program. The company cautions that forward-looking statements are inherently uncertain. Although the company believes that such statements are based on reasonable assumptions within the bounds of its knowledge of its business and operations, the forward-looking statements are neither promises nor guarantees and they are necessarily subject to a high degree of uncertainty and risk. Actual performance and results may differ materially from those expressed or implied in the forward-looking statements due to various risks and uncertainties. These risks and uncertainties include, among others: whether initial clinical results for alixorexton will be predictive of results of future stages of ongoing clinical studies, future clinical studies or real-world results; whether ongoing or future clinical studies for alixorexton will be initiated or completed on expected timelines or at all; whether alixorexton could be shown to be ineffective or unsafe; potential changes in the cost, scope and duration of the alixorexton development program; and those risks and uncertainties described under the heading "Risk Factors" in the company's Annual Report on Form 10-K for the year ended Dec. 31, 2024 and in subsequent filings made by the company with the U.S. Securities and Exchange Commission (SEC), which are available on the SEC's website at www.sec.gov. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. Except as required by law, the company disclaims any intention or responsibility for updating or revising any forward-looking statements contained in this press release.

1 U.S. Food and Drug Administration. *Breakthrough Therapy*, available at: https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/breakthrough-therapy.

[2] Buysse, D. Diagnosis and assessment of sleep and circadian rhythm disorders. *Journal of Psychiatric Practice*. 2005; 11(2):102-115

[3] Ten-Blanco M, Flores A, Cristino L, Pereda-Perez I. Targeting the orexin/hypocretin system for the treatment of neuropsychiatric and neurodegenerative diseases: From animal to clinical studies. Frontiers in Neuroendocrinology. 2023;69(101066). https://www.sciencedirect.com/science/article/pii/S0091302223000146

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