Denali Therapeutics Announces First Patient Dosed in Phase 1b Study of DNL201 for Parkinson's Disease

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- *Phase 1b study includes Parkinson's disease patients with and without a genetic LRRK2 mutation*

SOUTH SAN FRANCISCO, Calif., Dec. 10, 2018 (GLOBE NEWSWIRE) -- Denali Therapeutics Inc. (NASDAQ: DNLI), a biopharmaceutical company developing a broad portfolio of product candidates for neurodegenerative diseases, today announced initiation of dosing in a Phase 1b clinical study of DNL201 in patients with Parkinson's disease.

"Based on the positive outcome of our Phase 1 study in 122 healthy volunteer subjects, we are excited to evaluate DNL201 in Parkinson's disease patients," said Carole Ho, M.D., Chief Medical Officer of Denali. "This study will provide additional important safety and biomarker data in patients to support rational dose selection. The results from this study will inform decisions on further clinical testing of DNL201, including potential registrational trials."

DNL201, Denali's lead LRRK2 therapeutic candidate, is a small molecule inhibitor of leucine-rich repeat kinase 2 (LRRK2). LRRK2 is a regulator of lysosomal function, which is impaired in Parkinson's disease and may be restored by LRRK2 inhibition. Inhibition of LRRK2 activity may potentially slow the progression of disease in patients with a genetic LRRK2 mutation as well as in patients with sporadic Parkinson's disease.

Mutations in the LRRK2 gene are among the most frequent genetic causes of Parkinson's disease and a major driver of lysosomal dysfunction, which contributes to the formation of Lewy body protein aggregates and neurodegeneration.

About the DNL201 Phase 1b study

This study (NCT03710707) is a 28-day, randomized, placebo controlled Phase 1b clinical trial in patients with mild to moderate Parkinson's disease, with and without genetic LRRK2 mutations. Its purpose is to evaluate safety, tolerability, pharmacokinetics, pharmacodynamics, and target and pathway engagement biomarkers in multiple oral doses of DNL201. Exploratory endpoints include certain clinical endpoints. The planned 30 patients in the study will be randomized to receive either a low dose of DNL201, a high dose of DNL201, or placebo.

Data readout from this study is expected during Q4 2019. Further details are available at ClinicalTrials.gov.

About Denali

Denali is a biopharmaceutical company developing a broad portfolio of therapeutic candidates for neurodegenerative diseases. Denali pursues new treatments by rigorously assessing genetically validated targets, engineering delivery across the blood-brain barrier and guiding development with biomarker monitoring to demonstrate target engagement and select patients. Denali is based in South San Francisco. For additional information, please visit www.denalitherapeutics.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements expressed or implied in this press release include, but are not limited to, plans and expectations regarding, and implications and purposes of, the Phase 1b clinical study of DNL201 in patients with Parkinson's disease; expectations regarding patient enrollment in, and the timing of results of, such study; plans to progress DNL201 into additional clinical studies in Parkinson's disease patients; Denali's belief that inhibition of LRKK2 may have therapeutic benefit for a broad range Parkinson's disease patients; and statements made by Denali's CMO. Actual results are subject to risks and uncertainties and may differ materially from those indicated by these forward-looking statements as a result of these risks and uncertainties, including but not limited to, risks related to: Denali's early stages of clinical drug development; Denali's ability to complete the development and, if approved, commercialization of its product candidates; Denali's dependence on successful development of its BBB platform technology and product candidates currently in its core program; Denali's ability to enroll patients in, conduct, or complete, clinical trials on expected timelines; the uncertainty that any of Denali's product candidates will receive regulatory approval necessary to be commercialized; Denali's ability to continue to create a pipeline of product candidates or develop commercially successful products; Denali's ability to obtain, maintain, or protect intellectual property rights related to its product candidates; implementation of Denali's strategic plans for its business, product candidates and BBB platform technology; and other risks, including those described in Denali's Annual Report on Form 10-K filed with the SEC on March 19, 2018, Denali's Quarterly Report on Form 10-Q filed with the SEC on November 8, 2018 and Denali's future reports to be filed with the SEC. The forward-looking statements in this press release are based on information available to Denali as of the date hereof. Denali disclaims any obligation to update any forward-looking statements, except as required by law.

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