



Alector Announces Promising Preliminary Data from AL001 Phase 1b and Phase 2 Open-Label Long-Term Dosing Study of People with Frontotemporal Dementia

July 28, 2020

- *AL001 was generally safe and well-tolerated in Phase 1b study and after continuous dosing in 15 asymptomatic and symptomatic FTD-GRN participants in Phase 2 study*
- *Treatment with AL001 in Phase 2 study resulted in sustained restoration of plasma progranulin levels in all FTD-GRN participants back to normal range and the majority of symptomatic FTD-GRN participants showed a decrease in plasma neurofilament light chain (NfL) levels from baseline*
- *Pivotal Phase 3 study initiated in July 2020*

SOUTH SAN FRANCISCO, Calif., July 28, 2020 (GLOBE NEWSWIRE) -- Alector, Inc. (Nasdaq: ALEC), a clinical-stage biotechnology company pioneering immuno-neurology, today announced preliminary data from its Phase 1b and open-label Phase 2 studies of AL001, for the treatment of people with frontotemporal dementia with a progranulin gene (*GRN*) mutation (FTD-*GRN*). AL001 is the company's wholly owned, investigational human monoclonal antibody designed to modulate progranulin, a key regulator of immune activity in the brain. The data were presented today at the virtual 2020 Alzheimer's Association International Conference (AAIC) by Robert Paul, M.D., Ph.D., chief medical officer of Alector.

Frontotemporal dementia is a rapidly progressing and severe form of dementia found most frequently in people less than 65 years old at the time of diagnosis. There are multiple heritable forms of FTD, and FTD-*GRN* represents 5% to 10% of all people with FTD. There are currently no FDA-approved treatment options for FTD.

"We continue to discover and develop therapies in pursuit of our goal to eradicate neurodegenerative diseases," said Arnon Rosenthal, Ph.D., co-founder and chief executive officer of Alector. "The encouraging results from the Phase 1b and Phase 2 studies of AL001 represent substantial progress. With our rapid initiation of a pivotal Phase 3 trial, we hope that it is only a matter of time before AL001 is the first treatment option for people living with this devastating disease."

The ongoing Phase 2, multicenter, open-label, study is evaluating the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD), fluid and imaging biomarkers and the effect on clinical outcome assessments of AL001. The Phase 2 study is expected to enroll up to 40 participants who will receive treatment with AL001 for up to 96 weeks. The study has completed enrollment of 10 symptomatic patients with FTD-*GRN* and 5 asymptomatic individuals carrying a *GRN* mutation. Enrollment for FTD patients with *C9orf72* mutations is currently ongoing.

Due to the COVID-19 pandemic, several clinical sites for the Phase 2 study were temporarily closed or conducted reduced or remote patient assessments during the evaluation period. As a result, some participants missed a dose of AL001 or missed clinical assessments during the treatment period. Alector is working closely with the sites, investigators and participants to manage future effects of the COVID-19 pandemic on the study.

"AL001 was well-tolerated and caused sustained restoration of plasma progranulin levels in all FTD-*GRN* participants back to normal levels. These preliminary findings are encouraging and also suggest that long-term treatment with AL001 has the potential to slow disease progression in patients with FTD-*GRN* as measured by relevant disease biomarkers," said Dr. Paul. "Although the COVID-19 pandemic impacted data collection for this initial analysis, we believe these early results are quite promising. We continue to advance the Phase 2 study and would like to thank the participants, their families and our study collaborators for their support in advancing AL001 forward."

A summary of the preliminary results from the Phase 2 study are as follows:

- A total of 15 participants with symptomatic and asymptomatic FTD-*GRN* have been evaluated in the Phase 2 study as of May 14, 2020.
- AL001 was observed to be generally safe and well-tolerated. There were no treatment-related serious adverse events observed in participants receiving AL001 treatment.
- AL001 led to sustained restoration of plasma progranulin levels in all FTD-*GRN* participants back to the normal range.
- Preliminary data from the symptomatic FTD-*GRN* participants showed a decrease in plasma NfL levels from baseline in the majority of participants at the last measured time point.
- In the presented case study of a symptomatic FTD-*GRN* participant, with the longest continuous treatment with AL001 for over 28 weeks, treatment resulted in a sustained decrease in plasma NfL by 29% from baseline.

These early data suggest that longer term treatment with AL001 could lead to sustained increase in PGRN and sustained reduction in plasma NfL over time. Alector plans to present additional Phase 2 data highlighting clinical and biomarker endpoints in the future.

In July 2020, Alector [announced](#) that the first patient was dosed in INFRONT-3, a global, pivotal Phase 3 trial evaluating the efficacy and safety of AL001 in symptomatic and pre-symptomatic people with FTD-*GRN*. AL001 has received Fast Track and Orphan Drug designations from the U.S. Food and Drug Administration for FTD.

About the AL001 Open-Label Phase 2 Clinical Study

The AL001 Phase 2 clinical study is an ongoing, multicenter study evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of AL001 administered intravenously in participants with a progranulin gene mutation (*GRN*) or *C9orf72* mutation causative of frontotemporal dementia. The study is designed to assess the safety and tolerability of AL001 and measure the levels of progranulin, a disease specific biomarker, in plasma and in cerebrospinal fluid. Up to 40 patients will be enrolled and will receive treatment with AL001 for up to 96 weeks. For more information on the Phase 2 study, please visit www.clinicaltrials.gov.

About Frontotemporal Dementia (FTD)

Frontotemporal dementia is a rapidly progressing and severe form of dementia found most frequently in people less than 65 years old at the time of diagnosis. It affects 50,000 to 60,000 people in the United States and roughly 110,000 in the European Union, with potentially higher prevalence in Asia and Latin America. There are currently no FDA-approved treatments options for FTD.

There are multiple heritable forms of FTD. In one form, FTD-*GRN*, people have a mutation in the progranulin gene. This population represents 5% to 10% of all people with FTD. Mutations in a single copy of a progranulin gene leads to a 50% or greater decrease in the level of progranulin protein and invariably leads to development of FTD. In another form, FTD-*C9orf72*, people with mutations in the chromosome 9 open reading frame 72 (*C9orf72*) gene can develop FTD, which represents approximately 5% to 10% of all people with FTD. FTD-*C9orf72* is associated with abnormal accumulation of the protein TDP-43, which is also a hallmark in FTD-*GRN*. To date researchers have identified more than 120 inherited loss of function mutations in the progranulin gene that lead to FTD.

About Alector

Alector is a clinical stage biotechnology company pioneering immuno-neurology, a novel therapeutic approach for the treatment of neurodegenerative diseases. Immuno-neurology targets immune dysfunction as a root cause of multiple pathologies that are drivers of degenerative brain disorders. Alector is developing a broad portfolio of programs designed to functionally repair genetic mutations that cause dysfunction of the brain's immune system and enable the rejuvenated immune cells to counteract emerging brain pathologies. The Company's product candidates are supported by biomarkers and target genetically defined patient populations in frontotemporal dementia and Alzheimer's disease. Alector is headquartered in South San Francisco, California. For additional information, please visit www.alector.com.

Forward-Looking Statements

This press release contains "forward-looking" statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements are based on our beliefs and assumptions and on information currently available to us on the date of this press release. Forward-looking statements may involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from those expressed or implied by the forward-looking statements. These statements include but are not limited to statements regarding the Company's plans for and anticipated benefits and mechanism of the Company's product candidates, the timing and objectives of the clinical studies and anticipated regulatory and development milestones. Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future. Important factors that could cause our actual results to differ materially are detailed from time to time in the reports Alector files with the Securities and Exchange Commission, including in our quarterly report on Form 10-Q that is filed with the Securities and Exchange Commission ("SEC"). Copies of reports filed with the SEC are posted on Alector's website and are available from Alector without charge.

Contacts

Media

Erica Jefferson

Vice President, Communications and Public Affairs

Alector, Inc.

301-928-4650

erica.jefferson@alector.com

1AB

Dan Budwick

973-271-6085

dan@1abmedia.com

or

Investors:

Alector, Inc.

ir@alector.com



Source: Alector, Inc.