Press Release

Abliva AB (publ), 556595-6538
09 December 2024 08:32:00 CET - Lund, Sweden

Abliva Announces Publication of Results from Phase 1a/b Study of KL1333 in the Scientific Journal Brain

-Results demonstrated that KL1333 was safe and well tolerated, with early signs of efficacy in patients with primary mitochondrial disease-

Abliva AB (Nasdaq Stockholm: ABLI) a clinical-stage company developing medicines for the treatment of rare and severe primary mitochondrial disease, today announced that the Phase 1a/b study with KL1333 in healthy volunteers and patients has been published in the prestigious scientific journal Brain.

The manuscript, entitled *Optimising rare disorder trials: a phase 1a/1b randomized study of KL1333 in adults with mitochondrial disease*, by Dr. Pizzamiglio et al., can be accessed via the following link: http://academic.oup.com/brain/article-lookup/doi/10.1093/brain/awae308.

As previously announced, Abliva completed a clinical Phase 1a/b study with lead candidate KL1333 in healthy volunteers (n=64) and a cohort of patients with primary mitochondrial disease (n=8). The primary aim of the study was to evaluate the safety and pharmacokinetics of KL1333 in healthy volunteers and the safety and efficacy profile in patients. The results showed that KL1333 was safe and well tolerated. In addition, there were signs of efficacy, as well as an exposure-effect relationship, across the two main endpoints studied – fatigue and myopathy (muscle weakness).

> *"A publication in a prestigious journal like Brain highlights the importance of these findings and validates the adaptive trial design, which has informed Abliva's ongoing Phase 2 FALCON study of KL1333 in primary mitochondrial disease," said Magnus Hansson, Chief Medical Officer of Abliva. "We believe it is important to involve patients early, especially in rare disease studies, and we look forward to advancing KL1333 through clinical development as expeditiously as possible."*

These results further support the ongoing global, potentially registrational FALCON study in adult patients with mitochondrial disease who experience fatigue and myopathy. The first patient in the first wave of the study was dosed in June 2023. In July 2024, an interim analysis of the FALCON study evaluated 24-week data for patients included in the first wave of the study. The analysis confirmed that both independent, alternative primary endpoints passed futility, corroborated the strong safety profile of KL1333, and established that the study will recruit a total of 180 patients. See the announcement in full here.

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About Abliva's clinical Phase 1a/b study with KL1333

Abliva's clinical Phase 1a/b study was a double-blind, randomized, placebo-controlled study aiming to primarily assess the safety and pharmacokinetics of KL1333, the company's candidate drug for chronic oral treatment of primary mitochondrial disease. The study was conducted in the UK and was divided into four parts - three with healthy volunteers and one with patients. Patients were recruited in London by the team of Prof. Robert Pitceathly at the UCL Queen Square Institute of Neurology and The National Hospital for Neurology and Neurosurgery, and in Newcastle by the team of Prof. Grainne S. Gorman at the Wellcome Trust Centre for Mitochondrial Research. The study was finalized during spring 2021.

About the FALCON Study

FALCON is a Phase 2, global, randomized, placebo-controlled, potentially registrational study evaluating the safety and efficacy of KL1333 in adult patients with primary mitochondrial disease who experience consistent, debilitating fatigue and myopathy (muscle weakness), the most common and impairing symptoms. A total of 180 patients with mitochondrial DNA mutations who meet the eligibility criteria are randomized 3:2 to receive KL1333 (50mg-100mg) or placebo twice daily for 48 weeks. The two alternative primary endpoints assess consistent fatigue (using the PROMIS Fatigue Mitochondrial Disease Short Form) and myopathy (using the 30 second Sit-to-Stand test), only one of which must be positive to file for marketing approval. An interim analysis evaluating 24-week data from the first wave of patients confirmed the strong safety profile of KL1333, and both primary endpoints passed futility, meaning that both have the potential to demonstrate benefit in the final analysis of the study.

About KL1333

Abliva's lead candidate, KL1333, has been designed to treat chronic fatigue and myopathy (muscle weakness) in genetically confirmed adult patients with primary mitochondrial disease. Diagnoses can include MELAS-MIDD and KSS-CPEO spectrum disorders as well as MERRF syndrome. The drug candidate is intended for long-term oral treatment. KL1333 has the ability to restore the ratio of NAD+ and NADH, and thus leads to the formation of new mitochondria and improved energy levels. In a cohort of mitochondrial disease patients in a Phase 1a/b study, the patients who received KL1333 showed both improvements in symptoms of fatigue as well as functional improvements. KL1333 is currently being evaluated in a global, potentially registrational, Phase 2 study (the FALCON study) and has received orphan drug designation in both the USA and Europe as well as Fast Track designation in the USA.

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About primary mitochondrial disease

Primary mitochondrial disease affects the ability of cells to convert energy. The disease can manifest itself very differently depending on the organs impacted and the number of dysfunctional mitochondria in that organ. Historically viewed as clinical syndromes, our knowledge about the various mutations underlying mitochondrial disease has increased, improving our ability to identify and treat these patients. It is estimated that 1 in 5,000 people have primary mitochondrial disease. It often presents in early childhood and leads to severe symptoms, such as mental retardation, fatigue, myopathy, heart failure and rhythm disturbances, diabetes, movement disorders, stroke-like episodes, and epileptic seizures.

Abliva – Delivering mitochondrial health

Abliva discovers and develops medicines for the treatment of mitochondrial disease. This rare and often very severe disease occurs when the cell's energy provider, the mitochondria, do not function properly. The company has prioritized two projects. KL1333, a powerful regulator of the essential co-enzymes NAD^+ and NADH, has entered late-stage development. NV354, an energy replacement therapy, has completed preclinical development. Abliva, based in Lund, Sweden, is listed on Nasdaq Stockholm, Sweden (ticker: ABLI). For more information, please visit www.abliva.com. Subscribe to our news and follow us on LinkedIn and YouTube.

For more information, please contact:

Catharina Johansson, Deputy CEO, CFO & VP Investor Relations
+46 (0)46-275 62 21, ir@abliva.com

Abliva AB (publ)
Medicon Village, SE-223 81 Lund, Sweden
Tel: +46 (0)46 275 62 20 (switchboard)
info@abliva.com, www.abliva.com

Attachments

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