Press Release

Abliva AB (publ), 556595-6538
16 March 2021 14:45:00 CET - Lund, Sweden

Abliva reports completion of KL1333 Phase 1a /b study

Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage biopharmaceutical company developing medicines for the treatment of rare and severe primary mitochondrial diseases, today announced the completion of its clinical Phase 1a/b study with KL1333, Abliva's drug candidate for chronic oral treatment of primary mitochondrial diseases. No serious adverse events (SAEs) have been reported, and study data will be evaluated during the spring and early summer.

The primary aim of this double-blind, randomized, placebo-controlled Phase 1a/b study is to assess the safety and pharmacokinetics of KL1333, both in healthy volunteers and in patients with primary mitochondrial disease. All study participants have now completed the study. The next step is to process and evaluate data, where biomarkers and clinical outcome measures also will be assessed.

A total of 64 healthy volunteers and eight patients have been included in the study, conducted in the UK.

"On behalf of the Abliva team, I'd like to express our sincere gratitude to the persons who participated in the study and to the clinical teams at Covance in Leeds, in Newcastle led by Prof. Gorman, and in London led by Dr. Pitceathly. They have made this possible despite a serious pandemic, and their efforts will help advance the KL1333 development program substantially and increase the knowledge in this field of medical science", said Abliva's Chief Medical Officer Magnus Hansson.

Abliva plans to start a registrational clinical Phase 2/3 efficacy study during the second half of 2021. Preparatory activities, including a drug-drug interaction study, a patient registry study and a study to validate key patient-reported outcome measures for the efficacy study are either ongoing or being finalized. Results from these studies will ensure that the Phase 2/3 study is positioned for success.

Press Release

Abliva AB (publ), 556595-6538
16 March 2021 14:45:00 CET - Lund, Sweden

For more information, please contact:

Catharina Johansson, CFO & Vice President 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

Subscribe to our news: https://abliva.com/posts/news-subscription/
Follow us on LinkedIn: https://www.linkedin.com/company/abliva
Subscribe to our YouTube channel: https://www.youtube.com/channel/UChqP7Ky5caXtp72CELhD6Mg

About primary mitochondrial diseases

Primary mitochondrial diseases are metabolic diseases that affect the ability of cells to convert energy. The disorders can manifest differently depending on the organs affected and have historically been viewed as clinical syndromes, and more recently as disease spectra caused by genetic defects affecting mitochondrial function. An estimated 125 in every 1,000,000 people suffer from a primary mitochondrial disease. The diseases often present in early childhood and lead to severe symptoms such as mental retardation, fatigue, myopathy, heart failure and rhythm disturbances, diabetes, movement disorders, stroke-like episodes, and epileptic seizures.

About KL1333

KL1333 is a potent modulator of the cellular levels of NAD^+, a central co-enzyme in the cell's energy metabolism. KL1333 has in preclinical models been demonstrated to increase mitochondrial energy output, have long-term beneficial effects on energy metabolism, strengthen muscle function and improve biomarkers of mitochondrial disease. It is in clinical development stage intended to document the use for chronic oral treatment of primary mitochondrial disorders, in particular MELAS-MIDD spectrum disorders, mainly caused by the mutation m.3243A>G in the mitochondrial DNA (mtDNA) which affects about 35 in 1,000,000 people. An additional group is PEO-KSS spectrum disorders caused by a deletion of a large part of mtDNA which affects 15 in 1,000,000. These patients suffer from debilitating symptoms such as metabolic dysfunction, fatigue, muscle weakness, and deafness. KL1333 is currently being evaluated in clinical Phase 1 studies and has been granted orphan drug designation in both the United States and Europe. KL1333 has been in-licensed from Yungjin Pharm, a Korean pharmaceutical company.

Press Release

Abliva AB (publ), 556595-6538
16 March 2021 14:45:00 CET - Lund, Sweden

Abliva – Delivering mitochondrial health

Abliva develops medicines for the treatment of primary mitochondrial diseases. These congenital, rare, and often very severe diseases occur when the cell's energy provider, the mitochondria, do not function properly. The company is focused on two projects. KL1333, a powerful NAD^+ regulator, is in clinical development and has been granted orphan drug designation in Europe and the US. NV354, an energy replacement (succinate) therapy, is in preclinical development. Abliva, based in Lund, Sweden, is listed on Nasdaq Stockholm, Sweden (ticker: ABLI).

Attachments

Abliva reports completion of KL1333 Phase 1a/b study