Press Release

Abliva AB (publ), 556595-6538
19 May 2021 18:10:00 CEST - Lund, Sweden

Abliva reports positive clinical Phase 1a/b results for KL1333 including signs of efficacy in PMD patients

Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage biopharmaceutical company developing medicines for the treatment of rare and severe primary mitochondrial diseases (PMD), today announced positive safety and pharmacokinetic data from the double-blind, randomized, placebo-controlled Phase 1a/b study with KL1333, as well as signals of efficacy in relevant clinical outcome measures in patients with primary mitochondrial diseases.

The primary aim of this double-blind, randomized, placebo-controlled Phase 1a/b study was to assess the safety and pharmacokinetics of KL1333, Abliva's candidate drug for chronic oral treatment of PMD, both in healthy volunteers and in patients. Data from the study confirms single ascending dose (SAD) data from a prior Phase 1 study, and showed, for the first time, multiple-ascending dose (MAD) data from healthy volunteers and multiple day dosing in patients. The study included an analysis on the food effect and split dosing of KL1333 in healthy volunteers as well as a full characterization of pharmacokinetic parameters in both healthy volunteers and patients with primary mitochondrial diseases.

"This is a seminal trial for people with primary mitochondrial diseases. The study findings support progression of an innovative therapeutic agent into the later stages of drug development in a patient population with high unmet medical needs. I look forward to an ongoing partnership with Abliva as they advance their portfolio of novel drug candidates towards regulatory approval," said Dr. Robert Pitceathly, Chief Investigator of Part 1b of the study, MRC Clinician Scientist and Honorary Consultant Neurologist at University College London Queen Square Institute of Neurology and The National Hospital for Neurology and Neurosurgery.

In a cohort of eight patients with primary mitochondrial diseases (six dosed with KL1333, two with placebo), there were signs of efficacy across well-established, relevant clinical endpoints including two different patient-reported fatigue endpoints and a functional endpoint, 30-Second Sit to Stand. Although the study was not primarily designed to evaluate efficacy and the duration of dosing was only 10 days, the mean of change was numerically greater in the patients dosed with KL1333 compared to the placebo group for all three endpoints. In addition, there was an association between levels of KL1333 in the patients and efficacy.

"We were pleased to see the movement of two independent fatigue scales after only 10 days of dosing as it suggests that KL1333, through its mechanism of action, works quickly and it gives us increasing confidence in the use of fatigue as a primary endpoint in our upcoming clinical study. The fact that the patients with the highest exposure of KL1333 had the best effects, with clinically meaningful improvements across all three endpoints, strengthens our clinical program moving into our Phase 2/3 study," said Magnus Hansson, Abliva's CMO.

As Abliva previously communicated, there were no serious adverse events in the study. Further review of the safety data confirms no safety signals and reinforces the strong safety profile of KL1333 after dosing over 100 healthy volunteers and patients throughout development. KL1333 was generally well tolerated across patients and healthy volunteers with the main dose-limiting tolerability of gastrointestinal side effects, an effect that was improved by administering the drug two or three times per day.

Additionally, a second pharmacology study, one evaluating the interaction of KL1333 with seven enzymes (CYPs) that metabolize other drugs, has just completed. In the study there was only mild inhibition of one of the less common enzymes (CYP1A2) across the cohort, further strengthening the program as it moves forward in development.

“This data package increases our confidence in our upcoming Phase 2/3 study as we have confirmed the safety of KL1333, seen early signals of efficacy from relevant clinical outcomes, and identified a good dosing regime”, said Ellen Donnelly, Abliva’s CEO. “The company will now review the totality of the data and work to finalize our clinical protocol as we move towards IND submission later this year.”

This information is information that Abliva AB is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact persons set out below, at 2021-05-19 18:10 CEST.

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About primary mitochondrial diseases

Primary mitochondrial diseases are metabolic diseases that affect the cells' ability to convert energy. The disorders can manifest differently depending on the organs in which the genetic defects are present. They 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 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 diseases. The study was coordinated and conducted by Clinical Research Organization *Covance* in Leeds, UK, and was divided into four parts - three with healthy volunteers and one with patients. Patients were recruited in Newcastle by the team of Prof. Grainne S. Gorman at the Wellcome Trust Centre for Mitochondrial Research, and in London by the team of Dr. Robert Pitceathly at the UCL Queen Square Institute of Neurology and The National Hospital for Neurology and Neurosurgery. The study was finalized during spring 2021.

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.

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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

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