Press Release

Abliva AB (publ), 556595-6538
07 October 2020 08:30:00 CEST - Lund, Sweden

Abliva doses the first patient in its KL1333 Phase Ia/b clinical study

Abliva AB (Nasdaq Stockholm: ABLI) today announced that the first primary mitochondrial disease patient in the company's ongoing KL1333 Phase Ia/b study has been dosed. In this third part of the study, the pharmaceutical properties of KL1333 will, for the first time, be evaluated in patients.

Eight patients with primary mitochondrial disease will receive a daily dose of KL1333 for ten days, primarily for assessment of safety and pharmacokinetics of the candidate drug. In addition, biomarkers and clinical outcome measures will be evaluated ahead of the planned clinical pivotal Phase II/III study. Abliva's Phase Ia/b clinical study is conducted in the UK (London and Newcastle).

"The dosing in the first patient in our KL1333 program is a significant step in the development of a disease modifying oral treatment for patients with primary mitochondrial diseases. This important event follows the recent announcement of our intention to start a pivotal trial with KL1333 in 2021. Primary mitochondrial diseases are devastating diseases for which there are no existing pharmaceutical treatments and we are excited by the opportunity of bringing a novel treatment to the market and to patients with unmet medical needs", said Erik Kinnman, CEO at Abliva.

In parallel with running the patient portion of the Phase Ia/b study, the company will continue to prepare for the upcoming pivotal Phase II/III efficacy study with KL1333 planned to start H2 2021. Abliva will during H2 2020 initiate a qualitative study validating the specific patient-reported outcome measures, as well as a drug-drug interaction study in healthy volunteers. Moreover, long-term toxicology studies will be initiated that will run in parallel with the pivotal study in agreement with the FDA.

For more information, please contact:

Catharina Johansson, CFO, IR & Communications
+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

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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 by the genetic defects and are viewed as clinical syndromes. An estimated 125 in every 1,000,000 people suffer from a primary mitochondrial disease. Primary mitochondrial diseases often present in early childhood and lead to severe symptoms, such as stunted growth, fatigue, muscle weakness, heart failure and rhythm disturbances, diabetes, movement disorders, stroke-like episodes, deafness, blindness, limited mobility of the eyes, and 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 I 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.

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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