Press Release

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Abliva initiates a drug-drug interaction study and paves the way for a KL1333 pivotal study in 2021

Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage biopharmaceutical company developing medicines for the treatment of rare and severe primary mitochondrial diseases, today announces dosing in the first healthy volunteers in the company's drug-drug interaction (DDI) study with KL1333, the study recommended by the US Food and Drug Administration (FDA), which will assist the program to move directly into a pivotal Phase II /III study in patients with primary mitochondrial disease in 2021.

The DDI study is aimed to assess the potential impact of KL1333 on drugs used by treating physicians as part of the current standard of care. A total of 14 healthy volunteers will receive a daily dose of KL1333 together with a cocktail of other drugs for 12 days. The DDI study is part of a preparatory program ahead of the initiation of the Phase II/III study. The preparatory program also includes a qualitative validation study of specific patient-reported outcome measures, a clinical dosing study, a patient registry study, and initiation of long-term *in vivo* toxicology studies.

These activities will run in parallel with the planning of the Phase II/III study and the ongoing Phase Ia/b study. Four out of a total of eight patients have already been dosed in the patient portion of the Phase Ia/b study. Separately, a healthy volunteer portion will be added to the Phase Ia/b study to provide additional insight on the pharmacokinetics of KL1333. In addition, information provided by the MitoCohort UK (Newcastle University) primary mitochondrial disease registry study will be used to enhance the recruitment protocol of the pivotal Phase II /III study.

"The ongoing preparatory activities are important steps towards the start of the planned Phase II/III study in the second half of next year. We are truly excited about accomplishing the required studies and then initiating the pivotal patient study, as these studies may bring a much-needed novel treatment to patients with primary mitochondrial disease", said Erik Kinnman, CEO at Abliva.

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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.

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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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