Press Release

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Abliva receives positive regulatory feedback from UK MHRA on KL1333 Phase II/III study plan

Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage biopharmaceutical company developing medicines for the treatment of rare and severe primary mitochondrial diseases, today announced it has received positive feedback from the UK Medicines and Healthcare products Regulatory Agency (MHRA) on the accelerated clinical development plan of KL1333 in primary mitochondrial disease (PMD). The feedback positions Abliva for a clinical trial approval also in the UK, of its pivotal clinical Phase II/III study, planned to start in the second half of 2021.

The company recently received positive recommendations from the US Food and Drug Administration (FDA) leading to the current plan to conduct a pivotal Phase II/III study.

“The positive feedback from MHRA further validates Abliva’s decision to advance the development of KL1333, our primary mitochondrial disease asset with blockbuster potential, directly into a pivotal clinical study, a decision that will potentially cut down time to market approval by up to two years. The support from both the US and UK regulatory authorities is very beneficial in our efforts to bring a much-needed novel treatment opportunity to PMD patients”, said Abliva´s CEO Erik Kinnman.

The pivotal Phase II/III study will target patients with genetically confirmed MELAS-MIDD or KSS-CPEO spectrum disorder with multi-organ systemic symptoms. Preparatory activities are ongoing, including the patient portion of the Phase Ia/b study, a drug-drug interaction study in healthy volunteers, and collection of data from the UK patient registry study *MitoCohort*. In addition, the Company will perform a study validating patient-reported outcome measures, a dosing study in healthy volunteers, and long-term *in vivo* toxicology studies.

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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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