Press Release

Abliva AB (publ), 556595-6538
09 September 2020 20:30:00 CEST - Lund, Sweden

Abliva intends to start a pivotal KL1333 study second half 2021

Abliva AB (Nasdaq Stockholm: ABLI) today announced that the company's Board of Directors has decided to accelerate the KL1333 clinical program, with the intention to start a pivotal Phase II/III clinical study, during H2 2021. The decision follows the recent positive feedback received from the US Food and Drug Administration ("FDA").

In the Formal Advice feedback, FDA supported the existing documentation and the main elements of the clinical KL1333 development plan. The FDA furthermore recommended the company to extend and redesign the proposed KL1333 proof of concept ("PoC") study, which would be followed by a pivotal study, into one pivotal placebo-controlled study, a recommendation the Board has today acted on. Abliva believes this decision today combined with the FDA feedback reinforces the strategic intent of the Company to focus on orphan drugs and primary mitochondrial diseases ("PMD").

Abliva's management has carefully considered the opportunities and recommended updating the KL1333 clinical development plan in accordance with the FDA feedback. Today, the Board has consequently decided to support the recommendations to simplify and accelerate the KL1333 clinical program by moving directly into one pivotal clinical study towards a possible market approval of a treatment for PMD.

"I am happy that we, with the one pivotal KL1333 study, potentially could reach PMD patients with a high unmet medical need much earlier than would have been the case if we were to conduct a PoC study first. Furthermore, I am delighted that the FDA accepted starting the pivotal trial in parallel with the long-term toxicology studies enabling a H2 2021 start. In view of this good news Abliva will actively pursue financing for this program including targeting international capital markets", said Erik Kinnman, CEO of Abliva.

As a consequence of the updated KL1333 clinical development plan, 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, to enable the start of the pivotal study. These studies will run in parallel with the remaining patient portion of the ongoing phase Ia/b study. Moreover, long-term toxicology studies will be initiated that will run in parallel with the pivotal study in agreement with the FDA.

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 2020-09-09 20:30 CEST.

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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 mental retardation, heart failure and rhythm disturbances, dementia, movement disorders, stroke-like episodes, deafness, blindness, limited mobility of the eyes, vomiting, 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

Abliva intends to start a pivotal KL1333 study second half 2021