Press Release

Abliva AB (publ), 556595-6538
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Abliva receives positive FDA feedback on its KL1333 development plan

Abliva AB (Nasdaq Stockholm: ABLI) today announced that it has received positive feedback from the US Food and Drug Administration ("FDA") on its KL1333 clinical development plan for the treatment of primary mitochondrial disease (PMD) at a pre-Investigational New Drug ("pre-IND") meeting. Feedback was received on the existing KL1333 documentation to date and the remaining development plan, including the design of the clinical efficacy program in primary mitochondrial disease patients.

The FDA Formal Advice feedback supports the existing documentation and the main features of Abliva's plan to develop KL1333 towards approval for primary mitochondrial disease. The clinical KL1333 development plan will target patients with genetically confirmed MIDD-MELAS or KSS-CPEO spectrum disorders with multi-organ systemic symptoms. Moreover, the endpoints in Abliva's proposed clinical efficacy study will include evaluation of efficacy with regard to patient reported outcomes, as well as biomarkers of pharmacodynamic response.

"This is important feedback, for our KL1333 clinical program. It is also reassuring that it is aligned with the external expert input we have received and our internal plans. The next steps in the regulatory process are the submission and approval of an IND that will enable initiation and conduct of a clinical efficacy study with KL1333 in the US, and starting a similar dialogue with European regulators, said Magnus Hansson, Chief Medical Officer at Abliva

"The positive feedback from the FDA validates the preclinical, product development, and clinical documentation to date. Also, it is an additional step forwards in our efforts to deliver a needed new treatment to PMD patients", said Abliva´s CEO Erik Kinnman.

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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

Abliva receives positive FDA feedback on its KL1333 development plan