Press Release

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Abliva's Lead Candidate KL1333 Receives FDA Fast Track Designation

Abliva AB (Nasdaq Stockholm: ABLI) a clinical-stage company developing drugs for the treatment of rare and severe primary mitochondrial disease, today announced that the company's lead candidate KL1333 has received Fast Track designation from the U.S. Food and Drug Administration (FDA), facilitating its clinical development and path forward to market.

Abliva's lead drug candidate KL1333 is currently being evaluated in the FALCON study, a global, potentially registrational, Phase 2 study in mitochondrial disease patients. Dosing was initiated in June this year, and an interim analysis is expected towards the middle of 2024.

The FDA has now granted KL1333 Fast Track designation. This designation gives Abliva the possibility for more frequent meetings and written communication with the FDA. In addition, it will allow Abliva to receive continuous feedback on each section of the New Drug Application (NDA) (for sale and marketing in the U.S.) for KL1333, with expedited review and accelerated approval if certain criteria are met.

"The Fast Track designation is a quality stamp for our lead candidate and will facilitate our regulatory interactions in the U.S. This designation is one more important step forward as we prepare for a favorable interim analysis on the path towards bringing a new therapy to patients in this area of extremely high unmet medical need", said Ellen Donnelly, CEO.

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About KL1333

KL1333 is being developed towards a treatment for a subset of adult primary mitochondrial disease patients suffering from multiple debilitating symptoms, including chronic fatigue and myopathy. Diagnoses can include MELAS-MIDD and KSS-CPEO spectrum disorders as well as MERRF syndrome. The KL1333 compound is a potent modulator of the cellular levels of NAD^+ and NADH, central co-enzymes in the cell's energy metabolism. In a cohort of mitochondrial disease patients in a Phase 1a/b study, the patients who received KL1333 showed both improvements in symptoms of fatigue as well as functional improvements. KL1333 is currently being evaluated in a global, potentially registrational, Phase 2 study (the FALCON study) and has received orphan drug designation in both the USA and Europe.

About primary mitochondrial disease

Primary mitochondrial disease affects the ability of cells to convert energy. The disease can manifest itself very differently depending on the organs impacted and the number of dysfunctional mitochondria in that organ. Historically viewed as clinical syndromes, our knowledge about the various mutations underlying mitochondrial disease has increased, improving our ability to identify and treat these patients. It is estimated that 125 people per million have primary mitochondrial disease. It often presents in early childhood and leads to severe symptoms, such as mental retardation, fatigue, myopathy, heart failure and rhythm disturbances, diabetes, movement disorders, stroke-like episodes, and epileptic seizures.

Abliva – Delivering mitochondrial health

Abliva discovers and develops medicines for the treatment of mitochondrial disease. This rare and often very severe disease occurs when the cell's energy provider, the mitochondria, do not function properly. The company has prioritized two projects. KL1333, a powerful regulator of the essential co-enzymes NAD^+ and NADH, has entered late-stage development. NV354, an energy replacement therapy, has completed preclinical development. Abliva, based in Lund, Sweden, is listed on Nasdaq Stockholm, Sweden (ticker: ABLI).

Attachments

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