Press Release

Abliva AB (publ), 556595-6538
11 October 2023 08:30:00 CEST - Lund, Sweden

Abliva Achieves Important Milestone in the Ongoing FALCON Study

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 has now included the target number of patients required for screening in Wave 1 of the FALCON study. The study therefore remains on track to have all Wave 1 patients commence dosing by the end of the year with the interim analysis towards the middle of 2024.

Abliva is currently running the FALCON study, a global, randomized, placebo-controlled, and potentially registrational clinical Phase 2 study evaluating the efficacy of KL1333 in adult mitochondrial disease patients experiencing fatigue and myopathy.

The target number of patients required for screening in Wave 1 of the study has now been met. Screened patients have been, and will continue to be, evaluated for initiation of dosing in the study, with the goal that all eligible patients will have initiated dosing by the end of 2023. In the FALCON study, patients who meet the initial criteria are evaluated during a screening period, during which time their genetic background and a baseline of consistent fatigue and myopathy is confirmed. Patients that satisfy the necessary criteria commence dosing at the end of their screening period.

“The fact that we were able to quickly identify over 90 rare disease patients for screening in this study, with an intention to dose at least 40, speaks to both the commitment of the sites to this study and to the significant interest by patients to participate in a study that offers a drug that may address both their fatigue and their myopathy”, said Dag Nesse, VP Clinical Operations.

The FALCON trial has an adaptive study design with an interim analysis that will determine the final number of patients to be recruited in the full study (Wave 1+ Wave 2) to support a potentially registrational dataset.

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For more information, please contact:

Catharina Johansson, Deputy CEO, CFO & VP Investor Relations
+46 (0)46-275 62 21, ir@abliva.com

Abliva AB (publ)
Medicon Village, SE-223 81 Lund, Sweden
Tel: +46 (0)46 275 62 20 (switchboard)
info@abliva.com, www.abliva.com

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About the FALCON study

The FALCON study is a global, randomized, placebo-controlled, potentially registrational, clinical Phase 2 study with KL1333. Through the study, the company evaluates the safety and efficacy of KL1333 on primary mitochondrial disease in adult patients with mitochondrial DNA mutations, with a focus on chronic fatigue and muscle weakness, which are the most common and debilitating disease expressions in these patients. The company will recruit 120 – 180 patients, in two waves, who will be given KL1333 or placebo twice daily for 12 months. An interim analysis will take place after the completion of Wave 1 and will give important statistical information on safety and powering in Wave 2.

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.

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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 as well as Fast Track designation in the USA.

Attachments

Abliva Achieves Important Milestone in the Ongoing FALCON Study