Press Release

Abliva AB (publ), 556595-6538
18 July 2024 22:22:00 CEST - Lund, Sweden

Abliva Announces Positive Interim Analysis of the FALCON Study for KL1333 in Primary Mitochondrial Disease

-Independent committee confirms FALCON study is powered for potential success with both alternative endpoints passing futility-

-Committee confirms strong safety profile of KL1333-

-Abliva to host conference call and webcast on Friday 19 July at 4:30 pm CET / 10:30 am ET–

Lund, Sweden, July 18, 2024 — Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage company developing drugs for the treatment of rare and severe primary mitochondrial disease, today announced a positive outcome of the interim analysis for FALCON, the potentially registrational study evaluating KL1333 in patients with primary mitochondrial disease. The study evaluates fatigue and myopathy as alternative independent primary endpoints, only one of which is required for a successful study readout. The independent Data Monitoring Committee (DMC) recommended continuing the study without modification. The favorable recommendation by the DMC to continue the FALCON study with a total of 180 patients validates the overall study design and confirms the strong safety profile of KL1333.

The DMC has reviewed the 24-week interim data from the Wave 1 patient cohort. In their analysis, the independent group conducted a pre-specified analysis of the conditional power of the two primary endpoints after 24 weeks of dosing. Both endpoints passed futility and the DMC recommended to include a total of 180 patients, increasing the probability of a positive readout upon completion of the full study. The DMC emphasized that KL1333 has a strong safety profile. No safety concerns or drug-related serious adverse events (SAEs) were seen after 24 weeks of dosing.

The interim analysis was conducted by an independent committee to maintain the rigor of a blinded study. Statistical significance across the two primary endpoints will be evaluated at the conclusion of the full study.

“We are pleased with the positive outcome of the interim analysis as it highlights the positive risk-benefit of KL1333, de-risking the full study, increasing our confidence in KL1333’s novel mechanism of action and the FALCON study design” said Ellen K. Donnelly, Chief Executive Officer of Abliva. “There are currently no approved therapies for mitochondrial disease and with over 85,000 people affected across the US and Europe, we believe KL1333 is well-positioned to address this high unmet medical need and reach blockbuster potential in key geographies. KL1333 has the potential for an expedited path to market and we are committed to rapidly advancing this important medicine towards approval, with plans to start the final wave of FALCON study recruitment in the second half of 2024.”

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Conference call

Abliva will host an investor call on Friday, July 19, 2024, at 10:30 AM ET / 4:30 PM CET, to discuss the positive outcome of the interim analysis for FALCON. A live question and answer session will follow the formal presentation. To register, click here.

If you would like to ask a question during the live Q&A, please submit your request to questions@lifesciadvisors.com.

About the FALCON Trial

FALCON is a Phase 2, global, randomized, placebo-controlled, potentially registrational trial evaluating the safety and efficacy of KL1333 in adult patients with primary mitochondrial disease who experience consistent, debilitating fatigue and myopathy. Patients with mitochondrial DNA mutations who meet the eligibility criteria are randomized 3:2 to receive KL1333 (50mg-100mg) or placebo twice daily for 48 weeks. The two alternate primary endpoints assess chronic fatigue (using the PROMIS Fatigue Mitochondrial Disease Short Form) and muscle weakness (using the 30 second Sit-to-Stand test), the most common and debilitating disease expressions in mitochondrial disease patients.

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 1 in 5,000 people 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.

About KL1333

Abliva's lead candidate, KL1333, has been designed to treat chronic fatigue and myopathy (muscle weakness) in genetically confirmed adult patients with primary mitochondrial disease. Diagnoses can include MELAS-MIDD and KSS-CPEO spectrum disorders as well as MERRF syndrome. The drug candidate is intended for long-term oral treatment. KL1333 has the ability to restore the ratio of NAD+ and NADH, and thus leads to the formation of new mitochondria and improved energy levels. 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.

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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). For more information, please visit www.abliva.com. Subscribe to our news and follow us on LinkedIn and YouTube.

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 2024-07-18 22:22 CEST.

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Attachments

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