Press Release

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Abliva Announces Completion of 24 Weeks of Dosing in Wave 1 of the FALCON Study of KL1333 in Primary Mitochondrial Disease

-With 24 weeks of dosing complete, Abliva remains on track to report the interim analysis in early Q3 2024-

Abliva AB (Nasdaq Stockholm: ABLI) a clinical-stage company developing medicines for the treatment of rare and severe primary mitochondrial disease, today announced that the Wave 1 cohort has completed 24 weeks of dosing in the ongoing Phase 2 FALCON trial of KL1333 for the treatment of primary mitochondrial disease. These data will be reviewed by the Independent Data Monitoring Committee (DMC), with results from the interim analysis expected in early Q3 2024. In addition to evaluating the interim safety data, the DMC will make a recommendation as to whether to continue the study and advise on the final study size.

"We are pleased that the Wave 1 patients have now been dosed for at least 24 weeks, an important milestone that moves us one step closer to delivering the first medicine for systemic mitochondrial disease and addressing the high unmet needs of these patients," said Ellen Donnelly, Chief Executive Officer of Abliva. "We are now well positioned to report the interim analysis after review by the DMC in early Q3, 2024. The analysis will provide the first view of KL1333's safety profile when dosed twice daily for 24 weeks and determine whether the primary endpoints are predicted to reach statistical significance after 48 weeks of dosing. We believe the planned sample size re-estimation for Wave 2, following the interim analysis, will optimize the trial design and increase the probability of success."

About the FALCON study

The FALCON study is a global, randomized, placebo-controlled, potentially registrational, Phase 2 study testing KL1333 in adult patients with primary mitochondrial disease with mitochondrial DNA mutations who experience chronic fatigue and myopathy. Efficacy will be evaluated with two alternate primary endpoints, a mitochondrial disease-specific fatigue scale and a functional test of myopathy, the 30 second Sit-to-Stand test. The study is designed so that the result at study completion has the potential to be positive both if only one or both of the primary endpoints show clinical benefit. All patients will take KL1333 or placebo twice daily for 48 weeks. The study has an adaptive design and will be run in two waves with 120 – 180 total patients participating.

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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

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.

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.

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For more information, please contact:

Investor Contact:
Joyce Allaire
Life Science Advisors
jallaire@lifesciadvisors.com
+1 617.435.6602

Abliva Company 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

Attachments

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