# Press Release

Abliva AB (publ), 556595-6538
17 September 2021 08:45:00 CEST - Lund, Sweden

# Abliva drug candidate NV354 to progress to clinical development following positive regulatory review

**Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage biopharmaceutical company developing medicines for the treatment of rare and severe primary mitochondrial diseases (PMD), today announced that a second program in the portfolio, NV354, will move to the clinic after having received favorable feedback from the regulatory authority in the UK, the MHRA, on the preclinical package. The team will now continue assembling the documentation necessary to support a Phase 1 clinical start in 2022.**

NV354, a precision-based therapy invented within Abliva's own research laboratories, targets a dysfunctional metabolic pathway which occurs in a number of mitochondrial diseases including Leigh sydrome, a devasating childhood disease. This novel therapy has been designed to provide an alternative source of energy, succinate, to the patient's cells via a unique prodrug strategy that aims to arrest disease progression, reduce mortality and improve the overall quality of life for children with Leigh syndrome.

As a gating mechanism to progress NV354 to clinical development, the Abliva team met with the UK regulatory agency to discuss the preclinical data package. With the success of that meeting the team will now continue assembling the documentation required for a Clinical Trial Application (CTA) to support Phase 1 clinical start in 2022.

"The progression of NV354 to the clinic is exciting as it shows that our team has the ability to make important research discoveries and convert those discoveries into clinical candidates," said Ellen Donnelly, CEO of Abliva. "The advancement of NV354 is important as this candidate will join lead candidate, KL1333, in the clinic next year, giving us a pipeline of products and additional opportunity for newsflow and catalysts to bring additional value to shareholders," Donnelly continued.

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## About primary mitochondrial diseases

Primary mitochondrial diseases are metabolic diseases that affect the cells' ability to convert energy. The disorders can manifest differently depending on the organs in which the genetic defects are present. They have historically been viewed as clinical syndromes and more recently as disease spectra, caused by genetic defects affecting mitochondrial function. An estimated 125 in every 1,000,000 people suffer from a primary mitochondrial disease. The diseases often present in early childhood and lead to severe symptoms such as mental retardation, fatigue, myopathy, heart failure and rhythm disturbances, diabetes, movement disorders, stroke-like episodes, and epileptic seizures.

## About NV354

The NV354 project is based on an Abliva innovation in which the body's own energy substrate, succinate, is made available in the cell via a 'prodrug' technology where the drug becomes active after entering the body. NV354 has been designed to address a common cause of primary mitochondrial disease, complex I dysfunction. When complex I is dysfunctional energy conversion is not sufficient, limiting cellular function with a risk of metabolic crisis. This dysfunction is apparent in disorders including Leigh syndrome and MELAS, both of which are very serious diseases with symptoms such as muscle weakness, seizures and other severe neurological manifestations.

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**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, will enter the clinic in 2022. Abliva, based in Lund, Sweden, is listed on Nasdaq Stockholm, Sweden (ticker: ABLI).

**Attachments**

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