Press Release

Abliva AB (publ), 556595-6538
17 April 2023 09:00:00 CEST - Lund, Sweden

Abliva's Drug Candidate NV354 Granted Orphan Drug Designation for the Treatment of Mitochondrial Disease

Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage company developing medicines for the treatment of rare and severe primary mitochondrial diseases, today announced that the drug candidate NV354 has been granted Orphan Drug Designation (ODD) for the treatment of mitochondrial disease by the United States FDA Office of Orphan Products Development.

Abliva's orally available compound NV354 is being developed for the treatment of severe primary mitochondrial diseases including Leigh syndrome, a rare disease primarily affecting the central nervous system.

"The granting of ODD to NV354 by the U.S. FDA is a validation of the quality of the NV354 pre-clinical program and another important milestone for Abliva. The ODD will be beneficial to us as we work to progress NV354 and evaluate the compound in patients who are in urgent need of therapies to relieve their suffering," said Ellen Donnelly, CEO at Abliva.

In pre-clinical models, NV354 has been shown to be safe and efficacious with optimal pharmacological properties, including high levels of brain penetration. NV354, the second program in the Abliva portfolio, was developed by Abliva scientists and complements the lead asset, KL1333, which also has ODD.

Orphan drug designation will facilitate regulatory and scientific advice meetings with the FDA regarding the NV354 program and may enable a focused development program with an expedited approval process. If approved, ODD also provides market exclusivity for NV354 (seven years in the U.S.) once the drug is approved, which adds to the protection provided by the NV354 patent estate.

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Abliva AB (publ) - the mitochondrial medicine company. The company is listed on Nasdaq Stockholm, Small Cap, under the ticker symbol ABLI.

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About NV354

The NV354 program is based on an innovation by Abliva scientists at Lund University. With NV354, 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. NV354 has completed preclinical development and has now been granted orphan drug designation in the U.S.

About primary mitochondrial diseases

Primary mitochondrial diseases are metabolic diseases that affect the ability of cells to convert energy. The diseases can manifest themselves 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 diseases has increased, improving our ability to identify and treat these patients. It is estimated that 125 people per million have 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 Leigh Syndrome

Leigh Syndrome is the most common childhood presentation of primary mitochondrial disease, typically affecting children in their first years of life. The disease is often triggered when an infection or illness causes an increased demand for energy, leading to severe neurological symptoms such as developmental delay and psychomotor regression, progressive disability, or childhood death. In a majority of cases, Leigh Syndrome is caused by genetic mutations causing dysfunction of the mitochondrial respiratory complex I (CI), which is critical for efficient energy production. It is estimated that Leigh and Leigh-like Syndrome affect every 1: 11,000 – 1:34,000 born, and there are currently no approved medicines.

Abliva – Delivering mitochondrial health

Abliva discovers and develops medicines for the treatment of mitochondrial diseases. These rare and often very severe diseases occur 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, is in clinical trials. NV354, an energy replacement therapy, has completed preclinical development. Abliva, based in Lund, Sweden, is listed on Nasdaq Stockholm, Sweden (ticker: ABLI).

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Attachments

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