Press Release

Abliva AB (publ), 556595-6538
14 December 2023 11:45:00 CET - Lund, Sweden

Abliva Receives Orphan Designation from the European Commission for its Drug Candidate NV354

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 European Commission has granted orphan designation for the company's drug candidate NV354 for the treatment of Leigh syndrome, facilitating its clinical development in neurological mitochondrial disease.

"The orphan designation by both the European Commission and the US FDA is a great recognition of the NV354 program and the first-in-class prodrug technology for the treatment of pediatric Leigh syndrome. This will facilitate the future progression to clinical development of a much-needed treatment for these patients", said Ellen Donnelly, CEO at Abliva.

Abliva's drug candidate NV354 is being developed for mitochondrial disease with neurologic complications, including Leigh syndrome, MELAS, and LHON. The program's preclinical development has been completed, and in April 2023, the company was granted orphan drug designation (ODD) for NV354 in the US for the treatment of mitochondrial disease.

Abliva has now received orphan designation for NV354 also from the European Commission for the treatment of Leigh syndrome. Orphan designation in Europe offers Abliva scientific advice on study protocols, various fee reductions, and access to EU grants. If approved for EU and US orphan (drug) status when authorized for marketing, NV354 would benefit from ten years of market exclusivity within the EU and seven years within the US.

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

Subscribe to our news: https://abliva.com/posts/news-subscription/
Follow us on LinkedIn: https://www.linkedin.com/company/abliva
Subscribe to our YouTube channel: https://www.youtube.com/channel/UChqP7Ky5caXtp72CELhD6Mg

Press Release

Abliva AB (publ), 556595-6538
14 December 2023 11:45:00 CET - Lund, Sweden

About NV354

NV354 is being developed for mitochondrial disease with neurologic complications, in particular at insufficient activity in the mitochondrial protein complex I. The resulting deficiency in energy conversion contributes to clinical signs and symptoms in many types of mitochondrial disease, including neurologic complications seen in Leigh syndrome, MELAS, or LHON. There are also additional expansion opportunities outside of mitochondrial disease. Brain-penetrable NV354 is based on an innovation in which the body's own energy substrate, succinate, is made available in the cell via a prodrug technology. The program has completed preclinical development and has been granted Orphan Drug Designation (ODD) in the EU and US.

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.

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).

Attachments

Abliva Receives Orphan Designation from the European Commission for its Drug Candidate NV354