

Press release

NeuroVive Pharmaceutical AB (publ)
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NeuroVive reports breakthrough in mitochondrial myopathy project

Lund, Sweden, 29 January 2018, NeuroVive Pharmaceutical AB (Nasdaq Stockholm: NVP, OTCQX: NEVPF) today announced a breakthrough in the company's project NVP025 for developing treatment of mitochondrial myopathy. In an experimental study carried out in collaboration with researchers at Karolinska Institutet in Stockholm, Sweden, the project's model substance has shown favourable effects which may counter disease progression in mitochondrial myopathy.

Professor Håkan Westerblad and his research group at Karolinska Institutet have evaluated the effects of NeuroVive's cyclophilin inhibitors in an experimental model of mitochondrial myopathy. At the end of the study's treatment duration, survival rate was 94 % in the group receiving treatment, compared to 50 % in the control group. Furthermore, muscle function was better in the treated group compared to the control group.

"The results from the study is an important milestone in NeuroVive's NVP025 project. The study shows that our cyclophilin inhibitors can be further developed towards the goal of offering mitochondrial myopathy patients a novel treatment option. We are now taking the project into its next phase, where we will optimize a candidate drug suitable for further development for patients with different types of muscle disorders," says Magnus Hansson, M.D., Ph.D, Chief Medical Officer and head of preclinical and clinical development at NeuroVive.

The researchers at Karolinska Institutet have previously shown that cyclophilin D, which is the target molecule in the NVP025 project, is present at abnormally high levels in the muscles of mitochondrial myopathy patients, and that inhibiting cyclophilin D with cyclosporin A counters the muscle weakness present in the experimental models.¹⁾ Cyclophilin inhibitors have also shown favourable effects in experimental models of other muscle disorders, such as Duchenne muscular dystrophy.²⁾

"The effects of NeuroVive's cyclophilin inhibitor are very exciting and are in line with results from previous studies, where cyclophilin inhibitors have been shown to counter disruption of mitochondrial function and muscle weakness in different models of muscular disorders. We are looking forward to continuing the positive collaboration with NeuroVive and taking the project additional steps forward in development," says Håkan Westerblad, Professor, Karolinska Institutet.

This information is information that NeuroVive Pharmaceutical AB (publ) is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact person set out below, at 01:00 p.m. CET on 29 January 2018.

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About Mitochondrial myopathy

Mitochondrial myopathies are a group of neuromuscular diseases caused by damage to the mitochondria, the small energy factories found inside almost all the cells in the body. Some of the more common mitochondrial myopathies include Kearns-Sayre syndrome, MERRF syndrome (myoclonus epilepsy with ragged-red fibers) and mitochondrial MELAS (mitochondrial myopathy, encephalopathy, lactic acidosis and stroke-like episodes). The symptoms of mitochondrial myopathies include muscle weakness, exercise intolerance and fatigue, and are often accompanied by other symptoms of genetic mitochondrial disorders such as heart failure or rhythm disturbances, dementia, movement disorders, stroke-like episodes, deafness, blindness, droopy eyelids, limited mobility of the eyes, vomiting, and seizures. The prognosis for these disorders ranges in severity from progressive weakness to death. There is a high unmet medical need of new and effective treatment options for mitochondrial myopathy.

About Karolinska Institutet

Karolinska Institutet is one of the world's leading medical universities. Its vision is to significantly contribute to the improvement of human health. Karolinska Institutet accounts for the single largest share of all academic medical research conducted in Sweden and offers the country's broadest range of education in medicine and health sciences. The Nobel Assembly at Karolinska Institutet selects the Nobel laureates in Physiology or Medicine.

About NeuroVive

NeuroVive Pharmaceutical AB is a leader in mitochondrial medicine, with one project in clinical phase II development for the prevention of moderate to severe traumatic brain injury (NeuroSTAT®) and one project in clinical phase I (KL1333) for genetic mitochondrial diseases. The R&D portfolio consists of several late stage research programs in areas ranging from genetic mitochondrial disorders to cancer and metabolic diseases such as NASH. The company's strategy is to advance drugs for rare diseases through clinical development and into the market. The strategy for projects within larger indications outside the core focus area is out-licensing in the preclinical phase. NeuroVive is listed on Nasdaq Stockholm, Sweden (ticker: NVP). The share is also traded on the OTCQX Best Market in the US (OTC: NEVPF).

1) Cyclophilin D, a target for counteracting skeletal muscle dysfunction in mitochondrial myopathy. Westerblad H. et al. Human Molecular Genetics, 2015, Vol.24, No 23; 6580-6587.

2) Alisporivir rescues defective mitochondrial respiration in Duchenne muscular dystrophy. Schiavone M. Et al. Pharmacol Res. 2017 Nov;125(Pt B):122-131.