Press release
NeuroVive Pharmaceutical AB (publ)
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NeuroVive's genetic mitochondrial disease program NVP015 reaches significant milestone

Lund, Sweden, 30 November 2017 – NeuroVive Pharmaceutical AB *(Nasdaq Stockholm: NVP, OTCQX: NEVPF), the mitochondrial medicine company, today announced that molecules in its NVP015 program have demonstrated positive effects in experimental models of genetic mitochondrial disease and that a lead compound has been selected. The lead was selected based on tolerability, plasma stability and organ delivery, specifically to the brain, and will now be taken into further in vivo experimental efficacy studies and preclinical development.*

The objective of NeuroVive's NVP015 program is to develop a treatment option for patients with genetic mitochondrial diseases, such as Leigh syndrome and MELAS, when acute cellular energy support is needed, such as during an ordinary infection. These patients are at risk of further deterioration in their disease and symptoms during such energy crisis.

"We are very pleased with the progress in the NVP015 program which, together with our KL1333 program, shows our strong commitment to the development of novel treatments for genetic mitochondrial diseases," commented Magnus Hansson, M.D., Ph.D., Chief Medical Officer and VP of Preclinical and Clinical Development at NeuroVive.

NeuroVive collaborates with Dr. Marni Falk's research lab at the Children's Hospital of Philadelphia (CHOP) to evaluate the NVP015 compound class. Top line data from these studies demonstrates that the compounds exert beneficial effects in experimental *C. elegans* models of genetic mitochondrial disease. Approximately 50 per cent of genetic mitochondrial disease patients have a complex I dysfunction (isolated or in combination with other defects) in the mitochondria. Complex I dysfunction occurs when the first of the five complexes in the mitochondrial respiratory chain fails to function normally, resulting in the mitochondria being unable to generate enough cellular energy, ATP.

"This is a breakthrough which takes us a step closer to our goal of developing a therapy for acute energy crisis situations in genetic mitochondrial disease patients. Currently, patients with these types of genetic mitochondrial disorders can only be offered symptomatic treatment alternatives, whereas the aim of the NVP015 program is to create a drug that can bypass the dysfunction, helping the mitochondria to function properly," said Eskil Elmér, M.D., Ph.D., Chief Scientific Officer and VP of Discovery at NeuroVive.

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 08:30 a.m. CET on 30 November 2017.

For more information please contact:
Daniel Schale, Director of Communications
+46 (0)46-275 62 21, ir@neurovive.com

NeuroVive Pharmaceutical AB (publ)
Medicon Village, SE-223 81 Lund, Sweden
Tel: +46 (0)46 275 62 20 (switchboard)
info@neurovive.com, www.neurovive.com

About NVP015
One of the most common causes of mitochondrial diseases relates to complex I dysfunction, i.e. when energy conversion in the first of the five protein complexes in the mitochondrion that are essential for effective energy conversion does not function normally. This is apparent in disorders including Leigh Syndrome and MELAS, both of which are very serious diseases with symptoms such as muscle weakness, epileptic fits and other severe neurological manifestations. The NVP015 program is based on a concept instigated by NeuroVive's Chief Scientific Officer Dr. Eskil Elmér and his colleagues by which the body's own energy substrate, succinate, is made available in the cell via a prodrug technology. A prodrug is an inactive drug that is activated first when it enters the body by the transformation of its chemical structure. Results from the NVP015 program were published in Nature Communications[1] in 2016. The NVP015 program is focused on enabling a systemic therapy to counteract acute energy crises in patients with genetic mitochondrial disease. NeuroVive has received a grant from the Swedish innovation agency Vinnova's 2017 Swelife call for the NVP015 development. NeuroVive collaborates with Dr. Marni Falk's research lab at the Children's Hospital of Philadelphia (CHOP) to evaluate the NVP015 compound class. Mitochondrial complex I dysfunction is also recognized in several other conditions and in October 2017, Dr. Todd Kilbaugh, also at CHOP, received a grant from the NIH program Countermeasures Against Chemical Threats (CounterACT) to study the NVP015 as a novel treatment against chemical threats.

About Mitochondrial Disorders
Approximately 12 in every 100,000 people suffer from a genetic mitochondrial disorder. Genetic mitochondrial disorders are congenital metabolic diseases that affect cellular energy conversion. The disorders can manifest differently depending on which organs are affected by the gene defects and are viewed as syndromes, depending on the organs affected and the signs and symptoms. The disease usually presents in early childhood, such as Leigh syndrome, a rare genetic neurometabolic disorder, characterized by the degeneration of the central nervous system. Another example of mitochondrial disease is MELAS, which can cause mitochondrial myopathy, stroke like episodes and brain damage with epilepsy. Candidate drugs from NeuroVive's genetic mitochondrial disease programs would qualify for orphan drug designation in the US and Europe during clinical development, enabling a faster and less costly route to market, and a higher sales price. In 2016, the orphan drug market amounted to USD 114 billion and in the same year, the average annual cost for the treatment of a single patient was an estimated USD 140,443 (approx. 1.3 million SEK)[2].

About NeuroVive
NeuroVive Pharmaceutical AB is a leader in mitochondrial medicine, with one program in clinical phase II development for the prevention of moderate to severe traumatic brain injury (NeuroSTAT®) and one program 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 programs 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) Ehinger JK et al. (2016) Nat. Commun.7:12317
2) Evaluate Pharma Orphan Drug Report 2017