

## Press release

NeuroVive Pharmaceutical AB (publ)  
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# NeuroVive's NVP015 program will be accelerated through award of major research grant to Children's Hospital of Philadelphia

**Lund, Sweden, 28 August, 2018, NeuroVive Pharmaceutical AB** (Nasdaq Stockholm: NVP, OTCQX: NEVPF) today announced that scientists at the company's research partner the Children's Hospital of Philadelphia (CHOP) have received a three-year grant (W81XWH-17-PRMRP-TTDA), in total of \$4,090,281 USD, from the U.S. Department of Defense, Office of the Congressionally Directed Medical Research Programs (CDMRP) for studies focused on NeuroVive's NVP015 program for genetic mitochondrial diseases.

The extensive program evaluating preclinical efficacy and exploring viable future clinical endpoints for the NVP015 program, will be conducted by a team of world leading experts under the leadership of Principal Investigator Dr. Todd Kilbaugh, Associate Professor of Anesthesiology, Critical Care and Pediatrics. The objective of the therapeutic development program is to develop a drug candidate for Investigational New Drug (IND) submission to the Food and Drug Administration (FDA) for initiation of Phase I clinical trials.

"We are delighted by the news that our research partners at CHOP are receiving this grant. It further validates the extensive interest our novel succinate prodrug approach to treating mitochondrial diseases has generated. The grant will allow Dr. Kilbaugh and his team to thoroughly explore the therapeutic potential of our succinate prodrug compounds and advance them towards a novel treatment for acute energy crises in patients with genetic mitochondrial diseases for which there currently are only symptomatic treatment options available. The outcomes of the grant will expedite the development of our NVP015 project into first in man studies," said NeuroVive Chief Scientific Officer Dr. Eskil Elmér.

The team of experts at CHOP include co-investigators Professor Douglas C Wallace, Director of the Center for Mitochondrial and Epigenomic Medicine (CMEM), Dr. Marni J Falk, Executive Director of Mitochondrial Medicine, and NeuroVive affiliated Dr. Michael Karlsson. In addition, NeuroVive Chief Scientific Officer Dr. Eskil Elmér and NeuroVive Senior Scientist Dr. Johannes Ehinger are formal consultants to the project.

The studies under the grant from the U.S. Department of Defense CDMRP will be the third, and most extensive, collaboration between NeuroVive and CHOP. The first ongoing NVP015 research collaboration is focused on genetic mitochondrial diseases, headed by Dr. Marni J Falk. The second on the use of NVP015 to mitigate the health effects of toxic chemicals, including traditional chemical warfare agents, toxic industrial chemicals and pesticides, headed by Dr. Todd Kilbaugh and funded by the NIH CounterACT program.

*The information was submitted for publication, through the agency of the contact person set out below, at 08:45 a.m. CEST on 28 August 2018.*

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## **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 project is based on an innovation in 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. Within the project a lead compound, NV354, has been selected for further development in the program based on tolerability, oral bioavailability, plasma stability and organ delivery, specifically to the brain.

## **About genetic mitochondrial diseases**

Genetic mitochondrial diseases are metabolic diseases that affect the ability of cells to convert energy. The disorders can manifest differently depending on the organs affected by the genetic defects and are viewed as syndromes. An estimated 12 in every 100,000 people suffer from a mitochondrial disease. Examples of mitochondrial diseases are: Leigh syndrome, MELAS, KSS, CPEO, PEO, Pearson, MERRF and Alpers syndrome. Mitochondrial diseases often present in early childhood and lead to severe symptoms, such as stunted growth, heart failure and rhythm disturbances, dementia, movement disorders, stroke-like episodes, deafness, blindness, limited mobility of the eyes, vomiting and seizures.

## **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 also consists of projects for genetic mitochondrial disorders, cancer and NASH. The company advances drugs for rare diseases through clinical development into the market. For projects for common indications the goal is out-licensing in the preclinical phase. A subset of compounds under NeuroVive's NVP015 program has been licenced to Fortify Therapeutics, a BridgeBio company, for local treatment development of Leber's Hereditary Optic Neuropathy (LHON). NeuroVive is listed on Nasdaq Stockholm, Sweden (ticker: NVP). The share is also traded on the OTCQX Best Market in the US (OTC: NEVPF).

**NeuroVive Pharmaceutical AB (publ)** - the mitochondrial medicine company. The company is listed on Nasdaq Stockholm, Small Cap, under the ticker symbol NVP. The share is also traded on the OTC Markets Group Inc market in the US. NeuroVive Pharmaceutical (OTC: NEVPF) trades on the OTCQX Best Market. Investors can find Real-Time quotes and market information for the company at [www.otcmartets.com/stock/NEVPF/quote](http://www.otcmartets.com/stock/NEVPF/quote)