Press Release

Pharnext Announces that the DSMB Recommends Continuing the Ongoing Phase 3 Trial of PXT3003 for Charcot-Marie-Tooth Disease Type 1A

Paris, France, 5:45pm, September 6, 2017 (CEST) – Pharnext SA (FR00111911287 - ALPHA), a biopharmaceutical company pioneering a new approach to the development of innovative drugs based on the combination and repositioning of known drugs, today announced that the independent Data Safety Monitoring Board (DSMB) has completed its second pre-specified safety evaluation of PXT3003 in the ongoing PLEO-CMT Phase 3 clinical trial. Based on a review of safety data from all randomized patients, the DSMB recommended to continue the PLEO-CMT study as planned.

PLEO-CMT is a pivotal, multi-center, randomized, double blind, placebo-controlled, three-arm Phase 3 study that was initiated in December 2015 and has enrolled 323 patients with mild-to-moderate CMT1A in 30 sites across Europe, the U.S. and Canada. Diagnosis of CMT1A has been confirmed genetically through detection of PMP22 gene duplication. Over 15 months, Pharnext will compare in parallel groups the efficacy and safety of two orally administered doses of PXT3003 to placebo. Efficacy will be assessed through one primary endpoint: change in the ONLS score at 12 and 15 months of treatment to measure improvement of patients' disability with PXT3003. Additional secondary outcome measures will be assessed including functional and electrophysiological endpoints.

The DSMB is an independent body of experts drawn from the fields of clinical medicine, biostatistics and study methodology, chartered to provide recommendations to Pharnext upon regular pre-specified review of the accumulated data during the conduct of the clinical trial.

"This second positive recommendation from an independent board of experts reinforces PXT3003's safety profile, even in patients who received up to 15 months of treatment. This Phase 3 clinical trial is highly significant for patients suffering from CMT1A where only supportive care is available today," **said Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext.** *"We believe that our PLEODRUG™ PXT3003, if successful, has the potential to transform the treatment of CMT1A in adults. We look forward to completing this clinical trial and sharing top-line results in mid-2018."*

About PXT3003

PXT3003, Pharnext's lead PLEODRUG™ in development for the treatment of Charcot-Marie-Tooth type 1A disease (CMT1A), is a novel, synergistic, low-dose combination of baclofen, naltrexone, and D-sorbitol formulated as an oral solution given twice-daily. PXT3003 has multiple main mechanisms of action: a synergistic inhibition of PMP22 gene overexpression associated with myelination improvement, direct nerve protection and additional positive effects on other cellular types: muscle cells, neuromuscular junctions and immune cells. PXT3003 obtained positive results in a Phase II clinical trial in 80 adult patients with CMT1A. In 2014, the EMA and FDA granted orphan drug designation to PXT3003 for the treatment of CMT1A in adults.

An international pivotal Phase III trial (PLEO-CMT) in over 300 adult patients with CMT1A is now underway at 30 sites across Europe, the U.S. and Canada.

About Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company founded by renowned scientists and entrepreneurs including Professor Daniel Cohen, a pioneer in modern genomics. Pharnext has two lead products in clinical development. PXT3003 is currently in an international Phase 3 trial for the treatment of Charcot-Marie-Tooth disease type 1A and benefits from orphan drug status in Europe and the United States. PXT864 has generated positive Phase 2 results in Alzheimer's disease. Pharnext is the pioneer of a new drug discovery paradigm: PLEOTHERAPY™. The Company identifies and develops synergic combinations of repositioned drugs at new optimal lower doses. These PLEODRUG™ offer several key advantages: efficacy, safety and intellectual property including several product or composition of matter patents already granted. The Company is supported by a world-class scientific team.

The company Pharnext is listed on Euronext Growth Stock Exchange in Paris (ISIN code: FR00111911287).
For more information, visit www.pharnext.com

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