Press release

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

Paris, November 17, 2016 – Pharnext SA (FR00111911287 - ALPHA), a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, today announced that the independent Data Safety Monitoring Board (DSMB) has completed its first pre-specified safety evaluation of PXT3003 in the ongoing PLEO-CMT Phase 3 clinical trial. Based on a review of safety data from 100 patients who completed at least three months of study treatment, the DSMB recommended continuing the PLEO-CMT study as planned.

PLEO-CMT is an international pivotal Phase 3 study that was initiated in December 2015 and is planned to enroll 300 patients with mild to moderate CMT1A from Europe and the U.S. by the end of December 2016. Patients will be randomized in three arms – placebo and two PXT3003 doses – and will receive study treatment over 15 months. PXT3003, developed using Pharnext's R&D platform PLEOTHERAPY®, is a novel oral fixed-low dose combination of (RS)-baclofen, naltrexone hydrochloride and D-sorbitol.

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.

"We believe this clinical trial has the potential to be a crucial turning point in the effort to finally provide an efficacious treatment for patients suffering from CMT1A," said **Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext.** *"Today's therapeutic options are very limited and mostly palliative in nature. Our PLEODRUG® PXT3003 has already demonstrated safety, tolerability and improvements in CMT1A patient disability in a Phase 2 trial. Given this positive safety assessment by the DSMB, we are hopeful to bring this much-needed potential therapy to patients suffering from this debilitating condition upon completion of this Phase 3 trial."*

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About CMT1A

Charcot-Marie-Tooth (CMT) disease encompasses a heterogeneous group of inherited, progressive, chronic peripheral neuropathies. CMT type 1A (CMT1A), the most common type of CMT, is an orphan disease affecting at least 125,000 people in Europe and the U.S. The genetic mutation responsible for CMT1A is a duplication of the PMP22 gene coding for a peripheral myelin protein. Overexpression of this gene causes degradation of the neuronal sheath (myelin) responsible for nerve dysfunction, followed by loss of nerve conduction. As a result of peripheral nerve degradation, patients suffer from progressive muscle atrophy of legs and arms causing walking, running, balance problems and abnormal hand functioning. CMT1A patients end up in wheelchairs in at least 5% of cases. They might also suffer from mild to moderate sensitive disorders. First symptoms usually appear during adolescence and will progressively evolve through patients' life.

To date, no curative or symptomatic medications have been approved and treatment consists of supportive care such as orthotics, leg braces, physical and occupational therapy or surgery.

About PLEO-CMT Trial

PLEO-CMT is a pivotal, multi-center, randomized, double blind, placebo-controlled, three-arm Phase 3 study which will enroll 300 patients aged 16 and older with mild to moderate CMT1A in Europe and the U.S. Diagnosis of CMT1A will be 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 dosage variations 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. A nine month follow-up study is planned thereafter, where all patients who will have completed the first 15 months, will receive the active PXT3003 dose.

For more information about the PLEO-CMT clinical trial, please visit the following website:
U.S. NIH ClinicalTrials.gov website at: https://clinicaltrials.gov/ct2/show/study/NCT02579759

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 focuses on neurodegenerative diseases and 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 low dose. These PLEODRUG® offer several key advantages: efficacy, safety, and intellectual property including several composition of matter patents already granted. The Company is supported by a world-class scientific team.

The company Pharnext is listed on Euronext Alternext Stock Exchange in Paris (ISIN code: FR00111911287).

For more information, visit www.pharnext.com