Press Release

Pharnext Announces Initiation of Phase 3 Extension Study PLEO-CMT-FU of PXT3003 for Treatment of Charcot-Marie-Tooth Disease Type 1A

Paris, France, March 16th, 2017 at 5.45pm CET– Pharnext SA (FR00111911287 - ALPHA), a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, today announced that the first two patients have entered the international Phase 3 Extension Study PLEO-CMT-FU of PXT3003 at La Timone University Hospital (Marseille, France). PXT3003 is Pharnext's lead PLEODRUG™ for the treatment of patients with mild-to-moderate Charcot-Marie-Tooth Disease Type 1A (CMT1A), a rare and debilitating inherited peripheral neuropathy for which there are no satisfactory approved treatments available.

The PXT3003 Phase 3 clinical program consists of two international trials:

- **PLEO-CMT**: a pivotal, multi-center, randomized, double blind, fifteen-month, placebo-controlled, adaptive design Phase 3 study that was initiated in December 2015. In November 2016, the independent Data Safety Monitoring Board (DSMB) recommended continuing the PLEO-CMT study as planned after its review of safety data from 100 patients who completed at least three months of study treatment. Patient enrollment was completed in December 2016 with 323 patients in 30 sites across Europe, the United States and Canada. Top-line results from the PLEO-CMT Phase 3 trial are expected in the second quarter of 2018. These data, if positive, will form the basis of the submission package for market approval to regulatory authorities in Europe and the United States.
- **PLEO-CMT-FU**: a multi-center, double blind, nine-month, Phase 3 follow-up extension study designed to assess the long-term safety and tolerability of PXT3003. This study will include patients who have completed the ongoing Phase 3 PLEO-CMT trial. Patients randomized to PXT3003 in PLEO-CMT will continue in PLEO-CMT-FU at the previously assigned dose, while those who received placebo will be randomized to one of two PXT3003 active doses. Top-line results from the PLEO-CMT-FU trial are expected in the second quarter of 2019.

Pharnext plans to apply for marketing authorization for PXT3003 in Europe and the United States in the first quarter of 2019. Long term safety data from PLEO-CMT-FU would then be submitted to regulatory authorities during their review of the marketing authorization application. This should lead to PXT3003 market approval during the second half of 2019, as scheduled.

PXT3003 is an oral fixed-low dose synergistic combination of (RS)-baclofen, naltrexone hydrochloride and D-sorbitol, developed using Pharnext's research and development platform PLEOTHERAPY™. In 2014, PXT3003 was granted orphan drug designation for the treatment of CMT1A in adults in Europe and in the United States.

"The initiation of this second international Phase 3 trial marks an important milestone for the whole PXT3003 clinical development program as it aims at confirming the long-term safety and tolerability profile of PXT3003 ."

said René Goedkoop, M.D., Chief Medical Officer of Pharnext. *"This advancement underscores once more the ability of our clinical team to deliver on time, while bringing us one step closer to providing PXT3003 as a potential treatment option to patients suffering from CMT1A where only supportive care is available today."*

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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 their legs and arms causing walking, running, balance problems and abnormal hand functioning. CMT1A patients end up in wheelchairs in at least 5% of cases. Patients might also suffer from mild-to-moderate sensitive disorders. First symptoms usually appear during adolescence and progressively evolve through patients' lives. 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 and PLEO-CMT-FU Phase 3 Trials

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.

PLEO-CMT-FU is an international, multi-center, double blind, nine-month, Phase 3 follow-up extension study that was initiated in March 2017. PLEO-CMT-FU is designed to assess the long-term safety and tolerability of PXT3003. All randomized patients who have completed the primary PLEO-CMT trial (i.e. 15-month double-blind treatment with two active doses of PXT3003 versus placebo) will be eligible to continue or initiate treatment with PXT3003 in this nine-month extension study. Patients randomized to PXT3003 in the primary trial will continue in PLEO-CMT-FU at the previously assigned dose while those who received placebo will be randomized to one of two PXT3003 active doses.

For more information about the PLEO-CMT clinical trials, please visit (U.S. NIH ClinicalTrials.gov website):
For PLEO-CMT trial: https://clinicaltrials.gov/ct2/show/study/NCT02579759
For PLEO-CMT-FU trial: https://clinicaltrials.gov/ct2/show/NCT03023540

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, please visit www.pharnext.com