Press release

Pharnext Completes Patient Enrollment in International Pivotal Phase 3 Trial of PXT3003 for Charcot-Marie-Tooth Disease Type 1A

Paris, December 20th, 2016 – Pharnext SA (FR00111911287 - ALPHA), a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, today announced that it has completed patient enrollment for its international pivotal Phase 3 PLEO-CMT trial of PXT3003, Pharnext's lead PLEODRUG©, for the treatment of Charcot-Marie-Tooth Disease Type 1A (CMT1A).

PLEO-CMT is a pivotal, multi-center, randomized, double blind, placebo-controlled, adaptive design 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. Patients have been randomized to receive either placebo or one of two doses of PXT3003 during 15 months. The primary endpoint of this clinical trial is the change in the Overall Neuropathy Limitation Scale (ONLS) in order to determine improvement of patients' disability after 12 and 15 months of treatment with PXT3003. Results of the PLEO-CMT trial are expected in the second half of 2018. Patients will be invited to continue treatment with PXT3003 in a 9-month extension study. 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 previous exploratory Phase 2 trial of PXT3003 demonstrated safety, tolerability and improvements beyond stabilization of CMT1A patient disability as published in the Orphanet Journal of Rare Diseases (http://www.ojrd.com/content/9/1/199).

Quotes:

"Completing enrollment of our pivotal Phase 3 trial of PXT3003 is a significant milestone and highlights the strength of our clinical operations and management teams, as well as the support of our numerous clinical trial sites, our dedicated clinical investigators and the patient community," said **René Goedkoop, M.D., Chief Medical Officer of Pharnext.**

Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext added: *"This clinical trial is highly significant for patients suffering from CMT1A where only supportive care is available today. Our PLEODRUG© represents a great hope for patients suffering from this debilitating disease."*

Michael W. Sereda, M.D., Professor of Neurology at the Max Planck Institute, Göttingen said: *"PXT3003 has shown much promise in previous preclinical and clinical studies, and the data Pharnext has published to date positions PXT3003 as a prominent and safe therapeutic candidate for patients with CMT1A. This Phase 3 trial*

could bring us closer to helping our patients find an efficacious treatment capable of altering the progressive course of the disease."

Allison Moore, Founder and Chief Executive Officer of the Hereditary Neuropathy Foundation (HNF) added: *"We have been pleased to have the opportunity to contribute to this groundbreaking research by providing U.S. clinical site identification and patient recruitment support for this pivotal Phase 3 trial of PXT3003. Our HNF resources have been instrumental such as the Global Registry for Inherited Neuropathies (GRIN), the online CMT Inspire Community or the recently launched CMT Connect program. We are enthusiastic that our joint effort with Pharnext could result in providing people suffering from CMT1A with much-needed new therapies and look forward to our joint work for years to come."*

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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 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. 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