Press Release

Pharnext Provides an Update on its Pivotal Clinical Development Program in Charcot-Marie-Tooth Disease Type 1A ('CMT1A')

PARIS, France, February 1, 2021, 6:00 p.m. CET – Pharnext SA (FR0011191287 - ALPHA) (the 'Company'), an advanced late-stage clinical biopharmaceutical company pioneering new approaches to developing innovative drug combinations based on big genomics data and artificial intelligence using its PLEOTHERAPY™ platform, today provides an update on its pivotal clinical development program for its lead medicine PXT3003 in Charcot-Marie-Tooth type 1A ('CMT1A'). Under its open Investigational New Drug ('IND') Application with the U.S. Food and Drug Administration ('FDA'), Pharnext is on track and expects to begin First-Patient-First-Dose in its clinical Pivotal Phase III Trial entitled PREMIER before March 31, 2021 in the U.S.

The PREMIER trial, which is being conducted in patients with mild-to-moderate CMT1A, is expected to enroll approximately 350 subjects from 50 centers worldwide. The FDA has guided that the pivotal studies required for New Drug Application ('NDA') submission include the PREMIER trial and a pre-clinical combination factorial study in a well-validated CMT1A animal model. Pharnext has also applied for, and is interacting with the FDA regarding a Special Protocol Assessment ('SPA') for the PREMIER Phase III Trial.

Pharnext is also continuing to conduct its ongoing Phase III Extension Study, CLN-PXT3003-03, an open label study that has enrolled 187 patients with mild-to-moderate CMT1A out of the 323 patients enrolled in the first double-blind Phase III Trial (PLEO-CMT). As of today, 130 CMT1A patients are still being treated with high-dose PXT3003 and have been followed for more than 2 years in order to assess not only the safety and tolerability of PXT3003, but also to assess its long-term efficacy. Pharnext will be reporting top-line interim data on the safety and efficacy of PXT3003 collected so far from this open label study during Q2 2021.

Dr. David Horn Solomon, Chief Executive Officer of Pharnext, said: "*Our clinical development and regulatory teams have made significant progress towards gaining final agreement on the pivotal trial requirements for submission with the FDA and EMA, as well as beginning our PREMIER trial by the end of Q1 2021. We are excited to continue advancing PXT3003 towards approval for CMT1A patients. Further, the readout of our phase III extension study before the end of Q2 2021 will permit further understanding and validation of the role PXT3003 may play in assisting these patients and their families with this severe, debilitating, chronic inherited neuropathy.*"

About the PREMIER Trial

The PREMIER trial will be an international, multi-center, randomized, double-blind, two-arm placebo controlled, pivotal Phase III trial. PXT3003 will be tested versus placebo in mild-to-moderate CMT1A patients over a 15-month period. Approximately 350 patients will be enrolled at 50 centers worldwide. This study is planned to be initiated in Q1 2021. As agreed with regulatory agencies, the primary efficacy endpoint will be the ONLS scale which measures functional disability. The secondary endpoints will include the following outcome measures: 1) 10-Meter Walk Test (10mWT), 2) Quantified Muscular Testing (bilateral foot dorsiflexion dynamometry), 3) Patient Global Impression of Change (PGI-S), 4) Patient Global Impression of Change (PGI-C), 5) Charcot-Marie-Tooth Neuropathy Score, version 2 (CMTNS-v2), and 6) Quantified Muscular Testing (hand grip). Safety and tolerability will be monitored throughout the study.

About Special Protocol Assessment ('SPA')

The SPA process is a procedure by which the FDA provides official evaluation and written guidance on the design and size of proposed Phase III protocols that are intended to form the basis for a NDA. For more information on the SPA process, please visit: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/special-protocol-assessment-guidance-industry.

About Charcot-Marie-Tooth Disease Type 1A ('CMT1A')

Charcot-Marie-Tooth ('CMT') disease encompasses a heterogeneous group of inherited, severe, debilitating, progressive and chronic peripheral neuropathies. CMT1A, the most common type of CMT, is an orphan disease with a prevalence of 1/5000 people affecting about 150,000 people in Europe and the U.S. and about 1,500,000 people worldwide. The genetic mutation responsible for CMT1A is a duplication of the PMP22 gene coding for a peripheral myelin protein. The duplication of this gene results in overexpression of the PMP22 protein and failure of Schwann cells to produce normal myelin (neuronal sheath). The lack of a normal myelin structure and function leads to abnormal peripheral nerve conduction and axonal loss. As a result of peripheral nerve degradation, patients suffer from progressive muscle atrophy in both the legs and arms causing problems with walking, running and balance as well as abnormal hand functioning. They might also suffer from mild to moderate sensory disorders. First symptoms usually appear during adolescence and will progressively evolve throughout life. Patients with the most severe form of CMT1A end up in wheelchairs, representing at least 5% of cases. 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. More information can be found at https://pharnext.com/en/disease/charcot-marie-tooth.

About PXT3003

PXT3003 is a novel fixed-dose synergistic combination of baclofen, naltrexone and sorbitol formulated as an oral solution given twice a day. The three individual components of PXT3003 were selected to downregulate the overexpression of PMP22 protein, leading to improvement of neuronal signaling in dysfunctional peripheral nerves that are an essential part of the pathophysiology of this disease. PXT3003 could also have a positive effect on other cellular types of the motor unit such as the axon (direct protection), neuromuscular junctions or muscle cells. PXT3003 has shown promising and consistent results across preclinical and clinical studies in Phase II and Phase III (PLEO-CMT). More information can be found at https://pharnext.com/en/pipeline/pxt3003.

About Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for orphan and common neurodegenerative diseases that currently lack curative and/or disease-modifying treatments. Pharnext has two lead products in clinical development. PXT3003 completed an international Phase III trial with positive topline results 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 encouraging Phase II results in Alzheimer's disease and will be advanced through partnerships. Pharnext has developed a new drug discovery paradigm based on big genomics data and artificial intelligence: PLEOTHERAPY™. Pharnext identifies and develops synergic combinations of drugs called PLEODRUG™. More information can be found at www.pharnext.com.

Pharnext is listed on the Euronext Growth Stock Exchange in Paris (ISIN code: FR0011191287).

Disclaimer

This press release contains certain forward-looking statements concerning Pharnext and its business, including in respect of timing of and prospects for clinical trials and regulatory submissions of the Company's product candidates as well as a potential financing transaction, the use of proceeds therefrom and cash runway. Such forward-looking statements are based on assumptions that Pharnext considers to be reasonable. However, there can be no assurance that the estimates contained in such forward-looking statements will be verified, which estimates are subject to numerous risks including the risks set forth in Pharnext's URD approved by the AMF on November 9, 2020 under number N° R. 20-029 as well as in its annual periodic management reports and press releases (copies of which are available on www.pharnext.com) and to the development of economic conditions, financial markets and the markets in which Pharnext operates. The forward-looking statements contained in this press release are also subject to risks not yet known to Pharnext or not currently considered material by Pharnext. The occurrence of all or part of such risks could cause actual results, financial conditions, performance or achievements of Pharnext to be materially different from such forward-looking statements. Pharnext disclaims any intention or obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events, or otherwise.

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