Press Release

Pharnext Announces First Patient Enrolled in Europe in the PREMIER Trial, its Pivotal Phase III Clinical Study of PXT3003 in Charcot-Marie-Tooth Disease Type 1A ('CMT1A')

PARIS, France, CET, July 12, 2021, 8:00 a.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 announces the enrollment of the first patient in Europe in its pivotal Phase III clinical study (the 'PREMIER trial') of PXT3003 in Charcot-Marie-Tooth disease type 1A ('CMT1A'). This first European enrollment, an important Company milestone, has taken place at the University Hospital La Timone in Marseille (France) and was reached according to prior disclosure of timelines. PXT3003 is the Company's lead program to treat CMT1A, an indication with currently no existing approved therapies.

The trial is currently already underway in the U.S. where the first patient was dosed in March 2021 with multiple sites actively screening and enrolling patients with CMT1A. The trial will enroll approximately 350 subjects with mild-to-moderate CMT1A in 50 centers across the U.S., Canada, Europe, and Israel, and, as of today, is on track to complete enrollment in 2Q 2022 as initially planned.

The main objectives of the PREMIER trial are to evaluate the safety and efficacy of PXT3003 for the treatment of CMT1A. Topline results of this trial are expected to be announced in 3Q 2023. If primary endpoints are met in the PREMIER trial and in the pre-clinical factorial study in the well-established and validated CMT1A transgenic rat model, the results of both studies will form the basis of a marketing authorization dossier to be submitted to the U.S. (FDA) and European (EMA) regulatory agencies potentially in 1H 2024.

Adrian Hepner, MD, PhD, Chief Medical Officer of Pharnext, said: "*We are delighted to see the ongoing progress in the enrollment of the PREMIER trial through the opening of the first European center in Marseille according to plans. The addition of European centers to those already running in the U.S. represents another positive step for PXT3003 as we seek to help patients with CMT1A. Additional centers will also be activated worldwide over the summer."*

Shahram Attarian, MD, PhD, Head of the Neuromuscular Diseases and ALS department at the University Hospital La Timone in Marseille (France), Coordinator of the FILNEMUS Rare Diseases Network and Neuromuscular Diseases Reference Centers in France, and Lead Investigator of the PREMIER trial in Europe, said: *"I'm honored to lead the first site in Europe to enroll patients in this promising pivotal Phase III clinical study, another step towards a potential treatment for those with CMT1A. I look forward to having more sites enrolling subjects in Europe, and globally, in the coming weeks.*

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 and PLEO-CMT-FU). More information can be found at https://pharnext.com/en/pipeline/pxt3003.

About the PREMIER Trial

The PREMIER trial is an international, randomized, double-blind, two-arm placebo-controlled, pivotal Phase III study, evaluating the efficacy and safety of PXT3003 versus placebo in mild-to-moderate CMT1A patients, over a 15-month period. The dose of PXT3003 tested in the PREMIER trial corresponds to the high dose ('HD') tested in the prior Phase III trial ('PLEO-CMT'). As agreed with regulatory agencies, the primary efficacy endpoint will be the Overall Neuropathy Limitations Scale ('ONLS') which measures functional motor disability. The secondary endpoints include the following outcome measures: 1) 10-Meter Walk Test ('10mWT'), 2) Quantified Muscular Testing (bilateral foot dorsiflexion dynamometry), 3) Patient Global Impression of Severity ('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. Further information on the PREMIER trial can be found on the ClinicalTrials.gov website (study identification number: NCT04762758) here.

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 ('CMT1A') and benefits from orphan drug status in Europe and the United States. An international pivotal Phase III study of PXT3003 in CMT1A, the PREMIER trial, is currently ongoing. 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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