Press Release

New Data from the Open-Label PLEO-CMT-FU Trial Shows Sustained Benefit with PXT3003 in Patients with Charcot-Marie-Tooth Disease Type 1A After 5 Years of Total Trial Time

- New data from the ongoing Open-Label Phase III Extension Study of PXT3003, the PLEO-CMT-FU trial, suggest good safety profile and continuous treatment effect of PXT3003 measured on the Overall Neuropathy Limitation Scale after 5 years of total trial time

- 126 patients with mild-to-moderate Charcot-Marie-Tooth Disease Type 1A are still on treatment with PXT3003 High Dose in the PLEO-CMT-FU trial

PARIS, France, May 16th, 2022, 8:30 am CET – Pharnext SA (FR0011191287 – ALPHA) (the "Company"), an advanced late-clinical stage biopharmaceutical company developing novel therapeutics for neurodegenerative diseases with high unmet medical need, today announces new results from the ongoing open-label follow-up extension study of PXT3003 in Charcot-Marie-Tooth Disease Type 1A ('CMT1A'), the PLEO-CMT-FU trial, which followed the first double-blind, placebo controlled Phase III study, the PLEO-CMT trial.

In January 2020 and April 2021, Pharnext reported results, based on previous data extraction from the PLEO-CMT and PLEO-CMT-FU trials, suggesting sustained safety and efficacy of PXT3003 in patients with mild-to-moderate CMT1A. The new results announced today are derived from a data extraction performed on April 25th, 2022, which shows the continuous treatment effect for CMT1A patients treated with PXT3003 High Dose ('HD') in the PLEO-CMT-FU Period 2 trial with a data readout at 60 months of total trial time (15 months of PLEO-CMT trial + 9 months of PLEO-CMT-FU trial period 1 + 36 months of PLEO-CMT-FU trial period 2). Please refer to an illustration of the first PXT3003 Phase III program design in the "About the PLEO-CMT-FU Trial" section below for more details.

Key features of the data analysis are as follows:

- PXT3003 continues to show a good tolerability and safety profile over the course of the first Phase III program (double-blind + ongoing open-label).
- PXT3003 continues to show encouraging efficacy results as measured on the Overall Neuropathy Limitations Scale ('ONLS') which evaluates the patient's functional motor disability.
    - The best efficacy signal was observed in the cohort of patients treated with PXT3003 HD during 5 years of total trial time (double blind + ongoing open-label).
    - Patients treated with placebo declined on ONLS during the double-blind phase but then improved when switched to PXT3003 in the ongoing open-label phase.

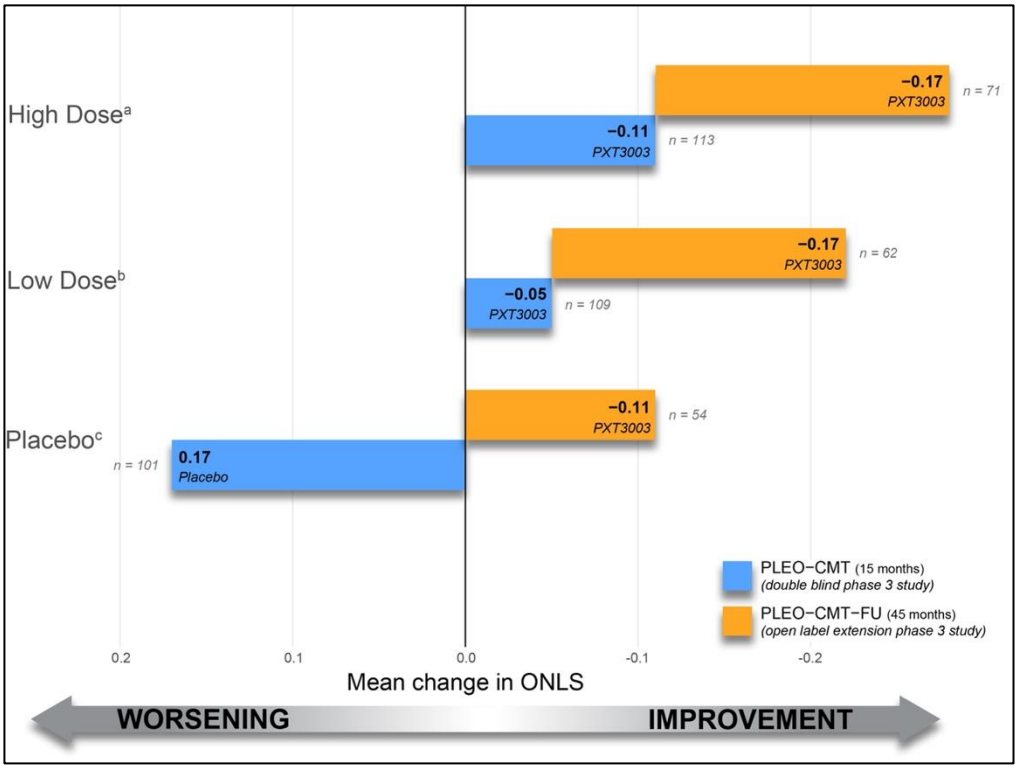
Please refer to the illustration of the ONLS data below for more details.

Dr. Burkhard Blank, Chief Medical Officer at Pharnext commented: "*The data announced today, while generated from an open-label extension study, show a sustained treatment benefit with PXT3003 High Dose for patients with CMT1A. As we approach completion of enrollment in our pivotal Phase III study, the PREMIER trial, in the same mild to moderate CMT1A patients' population, with the same High Dose of PXT3003 and using the same primary efficacy ONLS endpoint, this reinforces our confidence in potentially confirming the safety and efficacy of PXT3003."*

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 PLEO-CMT, PLEO-CMT-FU and PREMIER

trials in Europe, said: *"As an investigator involved since the beginning in the clinical development of PXT3003 in CMT1A, I find these long term safety and efficacy data very encouraging. Being able to stabilize, or even improve, patients with CMT1A is an extremely worthwhile goal particularly as these individuals will inevitably decline following the long-term natural course of the disease with the currently available standard of care. The entire CMT community is hopeful that PXT3003 could be the first approved therapy for this debilitating disease."*

Results of First Double-Blind Phase III (PLEO-CMT) & Open-Label Extension (PLEO-CMT-FU) Studies of PXT3003 with an ONLS Data Readout at 60 Months of Total Trial Time*



*Results based on database extraction done on April 25th, 2022

[a] Cohort of CMT1A patients treated with PXT3003 High Dose during PLEO-CMT and ongoing PLEO-CMT-FU trials

[b] Cohort of CMT1A patients treated with PXT3003 Low Dose during PLEO-CMT + PLEO-CMT-FU Period 1, and then switched to PXT3003 High Dose during PLEO-CMT-FU period 2

[c] Cohort of CMT1A patients treated with placebo during PLEO-CMT, PXT3003 Low Dose or High Dose during PLEO-CMT-FU Period 1 and PXT3003 High Dose during PLEO-CMT-FU Period 2

Please refer to a graphic illustration of the first double-blind Phase III (PLEO-CMT) and open-label extension (PLEO-CMT-FU) studies design in the "About the PLEO-CMT-FU Trial" section below.

About the PLEO-CMT Trial

The PLEO-CMT trial was an international, randomized, double-blind, placebo-controlled, Phase III study evaluating the efficacy and safety of PXT3003 in patients with CMT1A, over a 15-month period. Two dose levels, named low dose ('LD') and high dose ('HD'), of PXT3003 in comparison to placebo were tested in patients diagnosed with mild-to-moderate CMT1A (HD equals double LD). A total of 323 patients were enrolled in 29 centers across Europe, the U.S. and Canada by December 2016 and last-patient-last-visit occurred in March 2018. Due to an unexpected issue in the HD formulation, the HD arm was prematurely stopped in September 2017. A revised statistical analysis plan was developed to take into account the premature HD arm discontinuation. Analysis of the primary endpoint, Overall Neuropathy Limitations Scale ('ONLS') from all investigated populations in the HD arm suggested preliminary efficacy in humans. The study further demonstrated the safety and tolerability of PXT3003. Further information on the PLEO-CMT trial, including study results, can be found on the ClinicalTrials.gov website (study identification number: NCT03023540) here or in the following publication: https://ojrd.biomedcentral.com/track/pdf/10.1186/s13023-021-02040-8.pdf.

About the PLEO-CMT-FU Trial

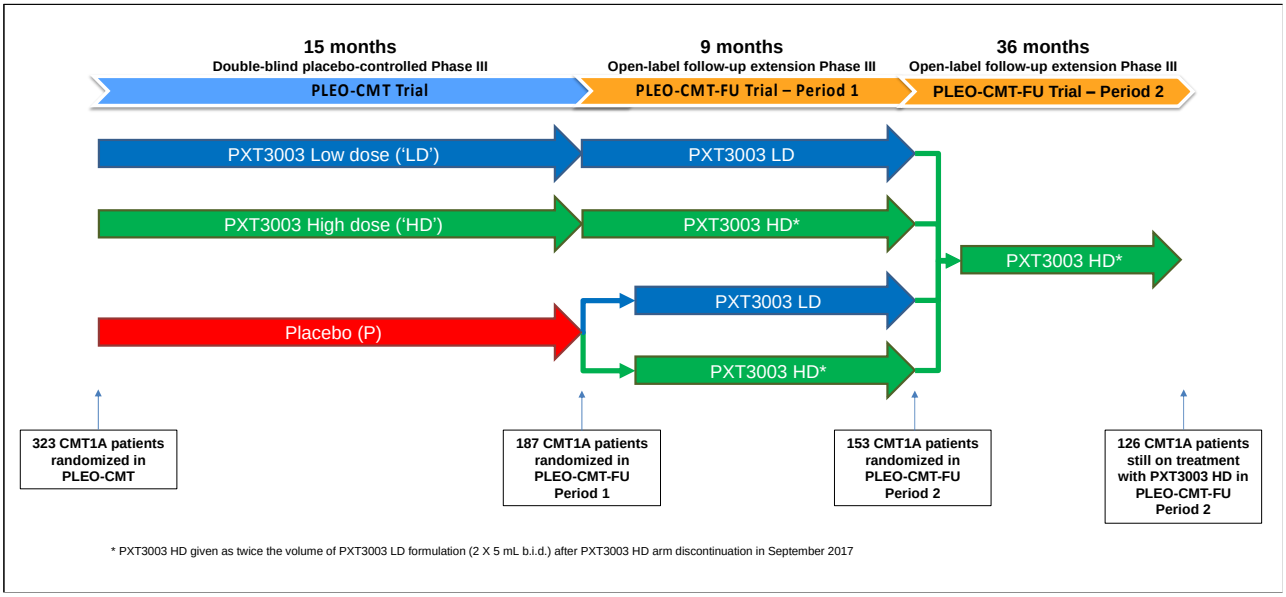
All randomized CMT1A patients who completed the PLEO-CMT trial (treated with PXT3003 or placebo) were eligible to pursue treatment with PXT3003 in the PLEO-CMT-FU trial. This trial enrolled a total of 187 patients and was designed to primarily assess the long-term safety and tolerability of PXT3003. It was initially planned to be a double-blind, nine-month, Phase III follow-up extension study where patients treated with PXT3003 in the PLEO-CMT trial were eligible to continue their treatment at the same dose (High dose 'HD' or Low Dose 'LD'). Patients treated with placebo in the PLEO-CMT trial were randomized in PLEO-CMT-FU to receive LD or HD of PXT3003. Due to the PXT3003 HD formulation issue which occurred during the PLEO-CMT trial, the HD arm was discontinued in September 2017. Consequently, the PLEO-CMT-FU trial became an open-label study divided into two periods:

- Period 1 (9-month treatment period) from March 2017 to April 2019. Patients randomized to PXT3003 LD in PLEO-CMT continued on the same dose. Patients randomized to PXT3003 HD in PLEO-CMT continued on the same dose, but it was given as twice the volume of PXT3003 LD formulation after the PXT3003 HD formulation issue. Patients randomized to placebo in PLEO-CMT continued only on PXT3003 LD after the HD formulation issue.
- Period 2 from July 2018 (still on-going). The 153 patients who entered in PLEO-CMT-FU Period 2 were all switched to PXT3003 HD given as twice the volume of PXT3003 LD formulation.

In PLEO-CMT-FU, on top of safety and tolerability of PXT3003 which is evaluated every 3 months, long-term efficacy is evaluated with the Overall Neuropathy Limitations Scale ('ONLS') measured every 6 months. Results from the PLEO-CMT-FU trial will be reported on a yearly basis.

Further information on the PLEO-CMT-FU trial can be found on the ClinicalTrials.gov website (study identification number: NCT03023540) here.

Design of First Double-Blind Phase III (PLEO-CMT) and Open-Label Extension (PLEO-CMT-FU) Studies of 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 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 Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for 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 encouraging 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. Both of Pharnext's lead assets originated from the Pleotherapy™ R&D approach. Pharnext draws the attention of investors to the financial and other risk factors detailed in its financial reports. More information can be found at https://pharnext.com/en
Pharnext is listed on the Euronext Growth Stock Exchange in Paris (ISIN code: FR0011191287).

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