Press Release

Pharnext announces being at D-18 before publication of topline results from pivotal Phase III clinical trial (PREMIER trial) in Charcot-Marie-Tooth disease type 1A

PARIS, France, November 23, 2023, 08:30 am CET – Pharnext SA (FR001400JXB0 - ALPHA) (the "Company"), an advanced late-clinical stage biopharmaceutical company developing novel therapeutics for neurodegenerative diseases with high unmet medical need, today unveils the calendar for publication of topline results from its pivotal Phase III clinical trial (PREMIER trial) in Charcot-Marie-Tooth disease type 1A (CMT1A), a rare debilitating peripheral neuropathy.

As announced, the PREMIER trial progressed as initially planned. Its 15-month double-blind period ended August 2023[1]. The next step is to lock the database on December 1st. Following this major step, Pharnext expects to receive first study results on December 8th. The Company should then be able to announce topline results of the PREMIER trial on December 11th, after preliminary analysis.

Hugo Brugière, Manager of Pharnext, said: "*We are delighted to successfully complete this Phase III clinical trial, and look forward to receiving topline results in two weeks' time. Our PXT3003 has already shown early signs of efficacy in previous Phase II and Phase III trials in CMT1A. We hope to be able to bring this innovative therapeutic solution to patients who currently have no alternative to palliative options.*"

As a reminder; the PREMIER trial, initiated in March 2021, is an international, randomized, double-blind, two-arm placebo-controlled, pivotal Phase III study, where the primary objective is to evaluate the efficacy and safety of PXT3003 versus placebo in mild-to-moderate CMT1A patients, over a 15-month double-blind period. The dose of PXT3003 tested in the PREMIER trial corresponds to the high dose (HD) tested in the prior Phase III clinical study, the PLEO-CMT trial, and its ongoing open-label extension Phase III study, the PLEOCMT-FU trial. As agreed with regulatory agencies, the primary efficacy endpoint is the Overall Neuropathy Limitations Scale ('ONLS') which measures functional motor disability. A total of 387 patients with mild-to-moderate CMT1A were enrolled in the PREMIER trial.

After completion of the double-blind period, CMT1A patients enrolled in the PREMIER trial were all offered continued treatment with PXT3003 by rolling over an open-label extension period named PREMIER-OLE (Open-Label Extension). This extension study was designed to assess the long-term safety and tolerability of PXT3003 in patients who have completed the PREMIER trial.
The first patient entered the PREMIER-OLE period in September 2022[2]. To date, 310 patients are receiving high-dose PXT3003, and have the opportunity to continue until the treatment is commercially available, should PXT3003 be approved in the US and Europe, respectively by the FDA and the EMA.

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

[1] Pharnext reports the end of double-blind treatment in PREMIER trial, the pivotal Phase III clinical trial of PXT3003 in Charcot-Marie-Tooth disease type 1A
[2] Pharnext Announces First Patient Enrolled in Open Label Extension of the Pivotal Phase III Study of PXT3003 for the Treatment of Charcot-Marie-Tooth Disease Type 1A, the PREMIER Trial

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.

Disclaimer
Pharnext arranged convertible bonds financing (OCEAN-BSA) with Global Tech Opportunities 13 which, after receiving the shares resulting from the conversion or exercise of these instruments, will not remain shareholder of the Company.
The shares resulting from the conversion or exercise of the above-mentioned securities will generally be sold on the market at very short notice, which may create strong downward pressure on the share price.
Shareholders may suffer a loss of their invested capital due to a significant fall in the Company's share price, as well as significant dilution due to the large number of securities issued to Global Tech Opportunities 13.
Investors are advised to exercise extreme caution before deciding to invest in the securities of a listed company that carries out such dilutive financing transactions, particularly when they are carried out in succession. The Company wishes to point out that this is not the first dilutive financing transaction it has undertaken.
Investors are invited to familiarize themselves with the risks associated with these transactions, as mentioned in the press release above.

About Pharnext
Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapies for neurodegenerative diseases currently without satisfactory therapeutic solutions. Pharnext has a first-in-class drug candidate, PXT3003, in development for Charcot-Marie-Tooth disease type 1A (CMT1A), a rare, debilitating, inherited peripheral neuropathy. PXT3003 benefits from orphan drug status in Europe and the United States. In 2018, PXT3003 completed a Phase III clinical trial, the PLEO-CMT trial, with encouraging topline results. This trial was followed by an open-label extension study, the PLEO-CMT-FU trial, with 120 patients continuing treatment with PXT3003. Long-term data suggest a sustained benefit, safety, and efficacy, after 6 years of total trial time. An international pivotal Phase III study of PXT3003, the PREMIER trial, enrolling 387 CMT1A patients was completed in August 2023. PREMIER topline results are expected in December 2023. PXT3003 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 www.pharnext.com.
Pharnext is listed on the Euronext Growth Stock Exchange in Paris (ISIN code: FR001400JXB0).

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