Press Release

Pharnext announces database lock for PXT3003 pivotal Phase III clinical study (PREMIER trial) in Charcot-Marie-Tooth disease type 1A, last step before topline results publication

PARIS, France, December 4, 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 announces the successful completion of database lock for its pivotal Phase III clinical study (PREMIER trial) of PXT3003[1], its drug candidate in Charcot-Marie-Tooth disease type 1A (CMT1A), a rare debilitating peripheral neuropathy.

In the wake of Pharnext's recent announcement[2] marking the last patients' last visit (LPLV) in the pivotal Phase III PREMIER study, we are thrilled to confirm the successful cleaning of the clinical database, with the database lock officially implemented on December 1, 2023. This monumental achievement paves the way for the unblinding of the study, heralding the commencement of the analysis phase for the eagerly anticipated topline results. Pharnext affirms its unwavering commitment to the predetermined schedule, assuring the delivery of topline data from the Phase III PREMIER study on December 11, as previously communicated[3].

Gilbert Wagener, Chief Medical Officer of Pharnext, expressed his exhilaration, stating, "*Being part of the Pharnext team and concluding a Phase III clinical trial that holds the potential to redefine the treatment landscape for CMT1A patients, who currently lack effective options, is nothing short of thrilling. We extend our sincere thanks to all patients, patient advocacy groups, and investigators for their unwavering support throughout the clinical trial. We eagerly anticipate sharing the results with the patient community and the public on December 11, 2023*."

Hugo Brugière, Managing Director of Pharnext, shared his excitement, stating, "*We are overjoyed to be on the cusp of the Phase III clinical data readout. The proximity to the finish line brings an electrifying sense of hope that PXT3003 could emerge as a groundbreaking therapeutic solution for CMT1A patients*."

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.

[1] Marketing of PXT3003 is conditional on a positive pivotal Phase III clinical trial (PREMIER) and the approval of regulatory agencies
[2] 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
[3] 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

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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