Press Release

Pharnext intends to prepare registration and marketing authorization dossiers for PXT3003, its drug candidate in Charcot-Marie-Tooth disease type 1A

PARIS, France, December 19, 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 its intentions after continuing the analysis of pivotal Phase III clinical results (PREMIER trial) for PXT3003, its drug candidate in Charcot-Marie-Tooth disease type 1A (CMT1A), a rare debilitating peripheral neuropathy. Following the publication of topline results on December 11, 2023[1], the analysis is still ongoing and will last through Q1 2024.

REFINE ANAYLSIS OF THE RESULTS

While primary analyses confirm the pivotal Phase III topline results obtained on the Overall Neuropathy Limitation Scale (ONLS) and Charcot-Marie-Tooth Neuropathy Score version 2 (CMTNS-v2), showing no significant difference in clinical response between treatment and placebo, additional subgroup analysis do suggest an efficacy signal. Indeed, in an analysis of patient subgroups, a better response was observed in treated patients with a BMI[2] <25, or in patients under 45 years of age.

Furthermore, analysis of the data revealed that while the study as a whole was randomized, double-blind, two-arm placebo-controlled, each of the clinical investigation centers were not stratified between the two arms in a balanced manner. As a result, in some centers, patients received only placebo, and in others only treatment. However, further analysis shows that when data from centers with fewer than two patients per arm are removed, there is a statistically significant difference between the treated and placebo groups on the CMTNS-v2 endpoint.

FILE PREPARATION FOR REGULATORY PROPOSALS WITH THE FDA AND EMA

From all these new results, results of the Phase II study, first Phase III study and extension studies, one of which is over 6 years, the product's remarkable safety profile, and the absence of any approved treatment or in advanced clinical stage for CMT1A, Pharnext believes there may be a chance to agree on a registration pathway for PXT3003 in CMT1A, with the FDA and the EMA.

From now on, the team will concentrate on putting into perspective all the data accumulated over ten years, in parallel with the medical literature describing the natural history[3] of CMT1A. This analysis will enable the Company's Supervisory Board and Management to decide whether to initiate a meeting with the FDA or wait for the results of the Phase III trial currently being conducted in China by its partner Tasly, which acquired the licensing rights for PXT3003 in China in 2017.

Hugo Brugière, Manager of Pharnext, said: "*I'm pleased to be able to confirm today what I said on December 11: we remain hopeful of finally being able to bring a medical solution to patients with no treatment. The in-depth study of the clinical data reveals more about the possible claims for our drug candidate, and does not contradict the results of previous studies.*
This is an encouraging signal for the future, enabling the Supervisory Board, Management, opinion leaders and our partners to keep moving forward".

[1] Pharnext reports topline results from the pivotal Phase III clinical trial (PREMIER trial) of PXT3003 in Charcot-Marie-Tooth disease type 1A
[2] Body mass index
[3] Description of the different manifestations of a disease and their evolution over time in the absence of any treatment

Disclaimer

Pharnext arranged (I) financing in the form of 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, and (II) financing in OS bonds which were subsequently transferred to a trust, which is now responsible for their equitization.

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. In the specific case of the trust, the shares are sold on the market in accordance with the terms set out in the trust agreement.

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 and/or the trust.

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.

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. More information at www.pharnext.com.
Pharnext is listed on the Euronext Growth Stock Exchange in Paris (ISIN code: FR001400JXB0).

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