Press Release

Pharnext reports topline results from the pivotal Phase III clinical trial (PREMIER trial) of PXT3003 in Charcot-Marie-Tooth disease type 1A

PARIS, France, December 11, 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 topline results of 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.

One of the main challenges in analyzing the results is the slow progression of CMT1A and the subjective nature of some clinical assessments, which can be influenced by placebo and training effects. This makes it difficult to determine the impact of treatment.

In this specific trial, the Overall Neuropathy Limitation Scale (ONLS), which measures functional motor disability, did not confirm findings from our previous clinical trial. Patients with mild-to-moderate CMT1A experienced improvement on both treatment and placebo, rather than the slow deterioration typical of CMT1A's natural progression. This unexpected improvement in the placebo group complicates the interpretation of the results based on this endpoint. It also confirms what the medical literature as a whole has been saying for many years, namely that ONLS is undoubtedly a relevant endpoint over the long term, but not over such a short period of time as a clinical study.

In the meantime, other data from the trial suggest no deterioration in the condition of patients under treatment, which is a positive sign in the context of a degenerative disease such as CMT1A. This suggests that PXT3003 might stabilize the condition of patients, which is an important consideration for a disease where progression is generally inevitable.

Moreover, the trial reaffirmed the high safety profile of the treatment, already established in previous studies. This safety profile is crucial for treatments of chronic diseases such as CMT1A.

Considering these initial results, Pharnext plans to continue analyzing the data, particularly in collaboration with potential partners for licensing or acquisition of PXT3003. This analysis will help determine the next steps toward potential marketing authorization.

Pharnext aims to leverage the clinical data accumulated on PXT3003 over the past decade, as well as the upcoming results from the Phase III trial currently conducted in China by its partner Tasly, who acquired the licensing rights for PXT3003 in China in 2017.

These efforts are part of Pharnext's strategic analysis to provide a medical solution for patients with CMT1A, who currently lack approved treatments. This situation illustrates the challenges of drug development for rare and complex diseases, where traditional clinical trial criteria may not fully capture the potential benefits of a treatment. It also highlights the importance accumulating long-term data and the need for innovative approaches in designing clinical trials for such conditions.

Hugo Brugière, Manager of Pharnext, said: "*Although the results we are sharing today are not exactly what we had hoped for, they are nonetheless very promising. We are now going to make the most of all the data we have accumulated over the last ten years, including our 2 Phase III studies and our 6-year extension study, which tend to demonstrate a beneficial effect on patients.*
I remind that, today, PXT3003 is the only ray of hope for all CMT1A patients, and that no other drug candidate is currently in an advanced stage. It seems to me, therefore, that efficacy results for a risk-free drug could support an application for regulatory approval and market authorization. This is for us the final stage, which we will move forward with patient community".

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

For information, Pharnext will hold a live conference this Monday, December 11, 2023, at 2:00 pm CET, in French. To access:

- Option 1: click on https://us02web.zoom.us/j/88004323605
- Option 2: open the zoom software and enter the meeting ID: 880 0432 3605
- Option 3: from a telephone, dial +33170372246 then enter the meeting ID when prompted: 88004323605 then # to validate

The conference will include a Q&A session based on questions received by email to pharnext@actus.fr no later than Monday December 11, 2023 at 12:00 CET.

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. 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 suggests 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 Q4 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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