Press Release

Pharnext updates on the development program in China for PXT3003 in Charcot-Marie-Tooth disease type 1A (CMT1A)

PARIS, France, 28 September 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 provides an update on the clinical development program in China of its drug candidate PXT3003 in Charcot-Marie-Tooth disease type 1A (CMT1A). As a reminder, PXT3003 is a novel fixed-dose synergistic combination of baclofen, naltrexone and sorbitol that benefits from orphan drug status granted by the EMA and the US FDA.

A SET OF PROMISING AGREEMENTS SIGNED IN 2017

Pharnext announced strategic partnership signed with Tasly Pharmaceutical (Shanghai: 600535) in 2017[1]. These agreements were composed as follows:

1) the creation of a research and development Joint-Venture (JV), GeneNet Pharmaceuticals Co, Ltd, owned 30% by Pharnext, to develop new combinations of molecules;
2) a licensing agreement granting GeneNet with the commercialization rights for PXT3003 in CMT1A in Greater China (Mainland China, Hong Kong, Taiwan, and Macau), as well as exclusive license rights to all PXT3003 patents applied for and authorized in Greater China;
3) an investment of €20 million by Tasly in Pharnext, including €5 million in shares and €15 million in convertible bonds completed in 2017, and an investment of €10 million in convertible bonds completed in 2018[2]. The total subscription of €25 million of convertible bonds being fully converted into ordinary shares in 2019[3].

STATUS OF PHASE III CLINICAL STUDY IN CHINA

In December 2017, GeneNet applied for clinical approval for PXT3003 to be registered as an imported pharmaceutical in China. In June 2018[4], the Chinese National Medical Products Administration (NMPA) granted GeneNet with a priority review of PXT3003 in CMT1A, and in January 2021 with a drug clinical trial approval in China (IND Approval).

In September 2021, GeneNet initiated on its side an independent Phase III clinical study including CMT1A patients in China. By now, the Phase III clinical study for PXT3003 by GeneNet is in process in China, with the plans to file an application for marketing authorization in China.

DEVELOPMENT IN PARALLEL WITH PHARNEXT AND POTENTIAL SYNERGIES

As a reminder, in March 2021[5], Pharnext initiated an international pivotal Phase III study (PREMIER trial), with World Clinical Tiral as a CRO, including 387 CMT1A patients in 52 centers in the United States, Canada, Europe and Israel. The dose of PXT3003 is the 'high' dose tested in the PLEO-CMT trial, but clinical batches are newly manufactured by Unither USA's facility as a 5 mL unit dose oral solution sachet[6]. The PREMIER trial was completed in August 2023[7], with topline results expected in Q4 2023 and Pharnext plans to file an application for marketing authorization in the US in S1 2024.

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[1] Pharnext Announces Strategic Partnership with Tasly, a Leading Chinese Pharmaceutical Group

[2] Pharnext successfully raises €16 million via private placement

[3] Reinforcement of Pharnext equity capital by conversion of convertibles bonds

[4] Pharnext Announces PXT3003 was Granted Priority Review by the China Food and Drug Administration

[5] Pharnext Announces First Patient Enrolled in the PREMIER Trial, its Pivotal Phase III Clinical Development Program of PXT3003 in Charcot-Marie-Tooth Disease Type 1A ('CMT1A')

[6] Pharnext announces successful completion of manufacturing transfer and scale-up of PXT3003 in the United States

[7] 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

Hugo Brugière, Manager of Pharnext, said: "*These two Phase III clinical studies of PXT3003 in CMT1A progressing in parallel for distinct markets could be complementary. We consider, on both sides, that each study can be individually subject to approval on our respective markets. Beyond that, their combined data may later strengthen the marketing authorization filling with the EMA for approval in Europe*."

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 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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