Press Release

Pharnext enters the final stretch for the signature of a licensing agreement for its drug candidate in Charcot-Marie-Tooth disease type 1A (PXT3003), enabling it to capitalize on its assets and put an end to the OCEANE-BSA financing line

PARIS, France, September 20, 2023 at 8:30 am (CET) - Pharnext SCA (FR001400JXB0 - ALPHA) (the "Company"), a late-stage biopharmaceutical company developing novel therapies for neurodegenerative diseases with no satisfactory therapeutic solution, today announces that it is in the final stages of signing a licensing agreement for PXT3003[1], its most advanced drug candidate currently in a pivotal Phase III clinical trial (**PREMIER** trial) in Charcot-Marie-Tooth disease type 1A (CMT1A), a rare disabling peripheral neuropathy.

COMPELLING OFFERS BY EARLY OCTOBER

At the end of the first phase of the partner search, initiated in spring 2023, several pharmaceutical companies expressed their interest in signing a licensing agreement to obtain the commercial rights to PXT3003 in certain geographical areas by submitting a non-binding offer. These highly detailed offers, which already include indicative financial terms for a potential agreement (upfront payment, royalties on sales, exclusivity, etc.), were formulated after a comprehensive due diligence phase based on documentation made available under confidentiality agreements.

Based on the selected non-binding offers, Pharnext invited the candidates to submit a binding offer.

The first offers are expected at 6pm CET on Friday September 29. Pharnext will then review the offers received and select one or more potential partners with a view to signing the definitive agreement(s) and concluding the envisaged transaction by the end of October.

A TOTAL POTENTIAL VALUE OF AROUND 400 M€.

According to the terms of the non-binding bids received to date, the total value of the PXT3003 license agreements - based on offers received to date - would be close to €400 million. This would include a significant proportion of upfront payments, currently estimated at around 10% of the total value of each license.

The signature of such agreements would cover Pharnext's financing needs, and thus put an end to the OCEANE-BSA financing line subscribed by Global Tech Opportunities 13.

Pharnext will communicate on the progress of this final phase in October, once the binding offers have been analyzed and validated by the Company and its advisors.

PERFECTLY ON SCHEDULE

This final step towards a partnership comes just after Pharnext announced, at the end of August, the completion of its **PREMIER** trial, a pivotal Phase III clinical study of its lead drug candidate, PXT3003.

The **PREMIER** trial went according to plan, confirming Pharnext's ability to successfully complete this ambitious program. The announcement of the preliminary results of this study is still scheduled for Q4 2023. This final stage of clinical development will pave the way for the preparation of registration and marketing authorization dossiers.

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

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 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.

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 5 years of total trial time. An international pivotal Phase III study of PXT3003, the PREMIER trial, is currently ongoing with 387 CMT1A patients enrolled. 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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