Press Release

Pharnext received two offers valuing its drug candidate for Charcot-Marie-Tooth disease type 1A at more than €250m, and raises €2.5m

PARIS, France, October 16, 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 it has received two offers to sign an agreement valuing 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 debilitating peripheral neuropathy, at over €250 million.

One offer is a license agreement with geographic exclusivity limited to the United States and Europe, allowing Pharnext to further negotiate in other territories later. The other offer concerns the purchase of all PXT3003 intellectual property and related contracts, for a worldwide exploitation except for China (see below).

In both cases, the estimated total lower value, including revenues indexed to sales of the future drug, is higher than €250m. Initial payments, staggered from signature of the definitive agreement to market authorization, represent at least €60m. The balance will be paid once marketing authorization is obtained. Depending on the various projections of future revenues from drug sales, total revenues could reach between €250m and up to €800m for Pharnext over the duration of the rights.

As a reminder, these potential agreements would complement the licensing agreement already signed in 2017 granting GeneNet (a research and development joint venture controlled by Tasly Pharmaceutical and owned 30% by Pharnext) with the commercialization rights for PXT3003 in CMT1A in Greater China (Mainland China, Hong Kong, Taiwan and Macau), and already generated an investment of €30m by Tasly in Pharnext.

These two binding offers received by Pharnext represent a critical step in the development and commercialization of this promising drug candidate. The names of the parties involved and financial details remain confidential for the time being. In line with the announced process, Pharnext is currently reviewing the offers received and intend to select a potential partner with a view to signing a definitive agreement by the end of October.

The signature of such agreements could cover Pharnext's financing needs, and thus terminate the OCEANE-BSA financing line subscribed by Global Tech Opportunities 13. Pharnext will communicate on the progress of this final phase as soon as possible.

In addition, in order to cover its short-term financing needs and secure the valuation process for its main asset, Pharnext announces that it has asked and obtained GTO 13's agreement to subscribe to additional financing of €2.5m over the next few days by issuing OCEANE-BSA bonds. This additional financing will be announced through usual press releases when drawings are effected.

Hugo Brugière, Manager of Pharnext, said: "*It's a tremendous victory for ambition over pessimism. No one gave us much hope of success when we took over the company in 2022, and even less so when we announced that we would reveal its full value potential in early 2023. Today, after months of thorough analysis of our available data by candidate companies, and before the read-out of top-line results of the Phase III clinical study, we received bids valuing our asset at over a quarter billion euros. We are therefore serene and enthusiastic as we analyze the best options cyrstalize the offers*."

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

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

(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 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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