

Pharnext Amends the Protocol of the International Pivotal Phase 3 Trials of PXT3003 for Charcot-Marie-Tooth Disease Type 1A

- No safety concern and PXT3003 development milestones remain unchanged -

Paris, France, 6:00pm, September 18, 2017 (CEST) – Pharnext SA (FR00111911287 - ALPHA), a biopharmaceutical company pioneering a new approach to the development of innovative drugs based on the combination and repositioning of known drugs, today announced an amendment in the protocol of the ongoing Phase 3 clinical program (PLEO-CMT and PLEO-CMT-FU studies) of PXT3003 for Charcot-Marie-Tooth disease type 1A (CMT1A) in adults to address a stability issue in the high dose formulation of PXT3003.

PLEO-CMT, a pivotal, multi-center, randomized, double blind, placebo-controlled, Phase 3 study, completed enrollment of 323 patients with mild to moderate CMT1A in 30 sites across Europe, the U.S. and Canada in December 2016. Patients have been randomized to receive during 15 months either the placebo or one of two doses of PXT3003: dose 1 (5 mL) or dose 2 (5 mL) with dose 2 equal to twice the dose 1. According to the protocol, all patients were supposed to continue treatment in a 9-month extension study (PLEO-CMT-FU), whilst placebo patients were randomized to dose 1 or dose 2 of PXT3003.

Overtime, after 12 months, a stability issue emerged in some batches of the high dose formulation (dose 2). This finding has raised no safety concern, but to ensure that the high dose patients get full exposure to the dose 2 level, Pharnext decided to switch these patients to receive double the amount of dose 1 (2 X 5 mL) in the 9-month open label extension study (PLEO-CMT-FU). Patients from the placebo and dose 1 arms in the 15-month double blind PLEO-CMT study will continue the Phase 3 clinical trial as planned: then, these patients will have the opportunity to continue treatment with PXT3003 in the PLEO-CMT-FU extension study for 9 months.

Main PXT3003 development milestones remain unchanged: adaptive design and futility analysis still planned by the end of 2017, results of the PLEO-CMT trial still expected in the second half of 2018, most likely in Q3. The statistical analysis plan will take the amendment into consideration. The data will form the basis of the submission package for market approval in the first quarter of 2019. Long-term safety data from PLEO-CMT-FU would then be submitted to regulatory authorities during their review of the marketing authorization application. Pharnext expects PXT3003 market approval during the second half of 2019, as scheduled.

As previously communicated, the independent Data Safety Monitoring Board (iDSMB) evaluated the safety data of all patients on September 5th, 2017, as it had no safety concern for both PXT3003 doses, it recommended study continuation. Of note, in agreement with regulatory agencies, the dose 2 was included in the PLEO-CMT study based on the dose response from the Phase 2 trial. Only the dose 1 was evaluated in the Phase 2 trial and demonstrated safety, tolerability and improvement beyond stabilization of CMT1A patient disability.

Quote:

“We have found a satisfactory solution for this unexpected stability event of the PXT3003 highest dose that was not previously investigated in our Phase 2 trial.” said Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext. *“All our objectives and development milestones remain unchanged and we look forward to bringing this innovative therapy to CMT1A patients.”*

About PXT3003

PXT3003, developed using Pharnext’s R&D platform PLEOTHERAPY™, is a novel oral fixed-low dose combination of (RS)-baclofen, naltrexone hydrochloride and D-sorbitol with Orphan Drug Designation in Europe and the United States.

About Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company founded by renowned scientists and entrepreneurs including Professor Daniel Cohen, a pioneer in modern genomics. Pharnext has two lead products in clinical development. PXT3003 is currently in an international Phase 3 trial for the treatment of Charcot-Marie-Tooth disease type 1A and benefits from orphan drug status in Europe and the United States. PXT864 has generated positive Phase 2 results in Alzheimer’s disease. Pharnext is the pioneer of a new drug discovery paradigm: PLEOTHERAPY™. The Company identifies and develops synergic combinations of repositioned drugs at new optimal lower doses. These PLEODRUG™ offer several key advantages: efficacy, safety and intellectual property including several product or composition of matter patents already granted. The Company is supported by a world-class scientific team.

Pharnext is listed on Euronext Growth Stock Exchange in Paris (ISIN code: FR00111911287).

For more information, visit www.pharnext.com

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