Press Release – Inside Information

Pharnext Provides U.S. Regulatory Update on PLEODRUG™ PXT3003 in Charcot-Marie-Tooth Disease Type 1A

PARIS, France, 8:00 am, August 30, 2019 (CET) – Pharnext SA (FR0011191287 - ALPHA), a biopharmaceutical company pioneering a new approach to the development of innovative drug combinations based on big data genomics and artificial intelligence (the "Company"), today announced the U.S. Food and Drug Administration ("FDA") has recommended that the Company conduct an additional Phase 3 study to evaluate PXT3003 in Charcot-Marie-Tooth Disease Type 1A ("CMT1A").

In the U.S., Pharnext completed its discussions with the FDA concerning the positive results of its pivotal Phase 3 PLEO-CMT study, previously announced in October 2018, in which the high dose arm was prematurely stopped due to an unexpected intercurrent formulation event. Because of this intercurrent event, the FDA advised that Pharnext run an additional Phase 3 study to ultimately support a New Drug Application for PXT3003 in CMT1A. The FDA acknowledged that CMT1A is a disease with high unmet medical need, granting Fast Track designation for PXT3003 in February 2019, and encouraged the Company to return to discuss a proposed study design. The expected calendar of the filing of the dossier for a marketing authorization will be updated accordingly.

"*We remain strongly confident in the potential of PXT3003 as a much-needed therapeutic option for patients with CMT1A, particularly given the consistent signal of efficacy observed across the clinical studies already performed and PXT3003's strong safety profile to-date,"* ***said Daniel Cohen, M.D., Ph.D., co-founder and Chief Executive Officer of Pharnext.*** *"We are fully committed to aligning with the FDA on the design of a clinical study as quickly as possible, in order to bring PXT3003 to U.S. patients with CMT1A."*

In parallel, the Company is continuing the development program for PXT3003 in CMT1A in Europe and in China. Pharnext is actively pursuing its effort to move forward with the registration path in Europe. In China, Pharnext and its Chinese pharmaceutical partner Tasly, are committed to pursuing development and registration path through their joint venture.

Furthermore, the Company aims to keep patients currently enrolled in the Phase 3 extension study on treatment until PXT3003 is commercially available.

About Phase 3 PLEO-CMT Study

In December 2015, Pharnext initiated the PLEO-CMT study, a pivotal 15-month, double-blind Phase 3 study that assessed the efficacy and safety of PXT3003 in 323 CMT1A patients aged 16 to 65 years. In October 2018, the Company announced that PXT3003 met the pre-specified primary endpoint of Overall Neuropathy Limitation Scale (ONLS), with a statistically significant difference compared to placebo (p=0.008). Pharnext also initiated PLEO-CMT-FU, a 9-month, open-label, follow-up extension study in March 2017, for which results are expected in Q4 2019. PLEO-CMT-FU, designed to assess the long-term safety and tolerability of PXT3003, enrolled patients who completed the PLEO-CMT study.

About PXT3003

Pharnext's first-in-class PLEODRUG™ PXT3003, developed using Pharnext's R&D platform, PLEOTHERAPY™, is a novel oral fixed-dose combination of baclofen, naltrexone and sorbitol, with Orphan Drug Designation in EU and the USA. PXT3003, Pharnext's lead PLEODRUG™, has shown positive results both in preclinical and Phase 2 studies for the treatment of CMT1A. These results were published in the Orphanet Journal of Rare Diseases (OJRD) in December 2014. In preclinical studies, PXT3003 inhibited the overexpression of the PMP22 gene, improved myelination of peripheral nerves and motor / sensory impairments. In a Phase 2 clinical study in 80 adult patients with CMT1A, PXT3003 was safe and well tolerated. In addition, PXT3003 showed trends in multiple efficacy endpoints beyond stabilization, particularly the ONLS scale.

About Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for orphan and common neurodegenerative diseases that currently lack curative and/or disease-modifying treatments. Pharnext has two lead products in clinical development. PXT3003 completed an international Phase 3 study with positive topline results 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 encouraging Phase 2 results in Alzheimer's disease. Pharnext has developed a new drug discovery paradigm based on big genomic data and artificial intelligence: PLEOTHERAPY™. Pharnext identifies and develops synergic combinations of drugs called PLEODRUG™. The Company was founded by renowned scientists and entrepreneurs including Professor Daniel Cohen, a pioneer in modern genomics, and is supported by a world-class scientific team.

Pharnext is listed on the Euronext Growth Stock Exchange in Paris (ISIN code: FR0011191287).

For more information, visit www.pharnext.com

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