Press Release

Pharnext Announces FDA Fast Track Designation for PLEODRUG™ PXT3003 for the Treatment of Charcot-Marie-Tooth Disease Type 1A

PARIS, France, 5:45 pm, February 4, 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, announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for the development of PXT3003 for the treatment of patients with Charcot-Marie-Tooth disease Type 1A (CMT1A). PXT3003 is a novel synergistic combination of baclofen, naltrexone and sorbitol, formulated as an oral solution that is given twice a day.

"*We are pleased to receive Fast Track designation for PXT3003 in CMT1A" **said Daniel Cohen, M.D., Ph.D., co-founder and Chief Executive Officer of Pharnext.** "CMT1A is a rare, chronic neuropathy that affects at least 125,000 people across the U.S. and Europe. There is currently no approved treatment for this disease. We look forward to continuing our productive dialogue with the FDA to rapidly progress towards a U.S. New Drug Application for PXT3003, to deliver this therapy to patients as quickly as possible."*

The FDA grants Fast Track designation to facilitate development and expedite the review of drug therapies with the potential to treat a serious condition, where there is an unmet medical need. Fast Track designation allows early and more frequent communications with the FDA to discuss the drug's development plans and review process, and also allows for a rolling review of a company's New Drug Application (NDA).

About Phase 3 PLEO-CMT Trial

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, PXT3003 announced that it met the FDA and European Medicines Agency (EMA) recognized 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, and the study is currently ongoing. 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 trial 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 trial 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

Disclaimer

This press release contains certain forward-looking statements concerning Pharnext and its business. Such forward-looking statements are based on assumptions that Pharnext considers to be reasonable. However, there can be no assurance that the estimates contained in such forward-looking statements will be verified, which estimates are subject to numerous risks including the risks set forth in Pharnext's document de base filed with the AMF on June 2, 2016 under number I.016-0050 as well as in any other periodic report and in any other press release (a copy of which is available on www.pharnext.com) and to the development of economic conditions, financial markets and the markets in which Pharnext operates. The forward-looking statements contained in this press release are also subject to risks not yet known to Pharnext or not currently considered material by Pharnext. The occurrence of all or part of such risks could cause actual results, financial conditions, performance or achievements of Pharnext to be materially different from such forward-looking statements. Pharnext disclaims any intention or obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events, or otherwise.

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