Press Release

Pharnext Announces PXT3003 for the Treatment of Charcot-Marie-Tooth Disease Type 1A has Been Granted Promising Innovative Medicine (PIM) Designation by UK Medicines and Healthcare Products Regulatory Agency

PARIS, France, 8:30 a.m., March 18, 2020 (CET) – Pharnext SA (FR0011191287 - ALPHA), a biopharmaceutical company pioneering a new approach to developing innovative drug combinations based on big genomics data and artificial intelligence, today announced that the United Kingdom's Medicine and Healthcare products Regulatory Agency (MHRA) has granted Promising Innovative Medicine (PIM) designation to its lead drug candidate, PXT3003, for the treatment of Charcot-Marie-Tooth Disease Type 1A (CMT1A) in patients 16 years and older.

A PIM designation is an early indication that a medicinal product is a promising candidate for the Early Access to Medicines Scheme (EAMS) in the treatment, diagnosis or prevention of life-threatening or seriously debilitating conditions with unmet need.

"*We are delighted by the MHRA's decision to award the PIM designation to PXT3003 as it further validates the potential of our lead drug candidate as an innovative treatment approach to address the high unmet medical need in patients with CMT1A,*" **said Daniel Cohen, M.D., Ph.D., co-founder and Chief Executive Officer of Pharnext**. "*All existing data indicate that PXT3003 is a safe and well tolerated drug combination. We look forward to continuing our discussions with U.S. and European regulatory authorities to advance the clinical development of PXT3003 and initiate as quickly as possible an additional pivotal Phase 3 trial in the U.S. and Europe.*"

PXT3003 has also been granted Orphan Drug status in the U.S. and Europe and has received Fast Track designation from the U.S. Food and Drug Administration (FDA).

About Charcot-Marie-Tooth Disease Type 1A (CMT1A)

Charcot-Marie-Tooth (CMT) disease encompasses a heterogeneous group of inherited, progressive, chronic peripheral neuropathies. CMT type 1A (CMT1A), the most common type of CMT, is an orphan disease affecting more than 100,000 people in the U.S. and E.U. The genetic mutation responsible for CMT1A is a duplication of the PMP22 gene coding for a peripheral myelin protein. Overexpression of this gene causes degradation of the neuronal sheath (myelin) and nerve dysfunction. As a result, patients have a significantly altered quality of life, suffering from progressive muscle atrophy of the limbs causing problems with walking, running balance and hand function. They may have loss of sensation, pain and cramps, and at least 5% need wheelchairs. First symptoms usually appear during childhood and progressively evolve throughout patients' lives. 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.

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 the U.S. and E.U. PXT3003, Pharnext's lead PLEODRUG™, has shown positive results both in non-clinical

pharmacology and clinical studies for the treatment of CMT1A. In non-clinical pharmacology 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. These results were published in the Orphanet Journal of Rare Diseases (OJRD) in December 2014. In October 2018, PXT3003 completed an international Phase 3 trial in 323 patients 16 years and older for the treatment of CMT1A, confirming the excellent safety profile of the combination and demonstrating an encouraging efficacy profile. The Phase 3 extension study is currently ongoing.

About the UK Promising Innovative Medicine (PIM) designation and Early Access to Medicines Scheme (EAMS)

The UK's industry-sponsored EAMS aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization when there is a clear unmet medical need. The EAMS is a two-step process: Step I is the Designation as a Promising Innovation Medicine (PIM). The PIM designation is an early indication that a medicinal product is a promising candidate for EAMS and gives reassurance that its clinical development is on track by having an early review of its data by the medicines regulator. Step II is the Scientific Opinion by the Medicines and Healthcare products Regulatory Agency (MHRA, UK regulatory agency). The Scientific Opinion describes the benefits and risks of the medicine and supports the prescriber and patient to make a decision on using the medicine before its license is approved.

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 genomics 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. More information at www.pharnext.com.

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

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 its annual periodic management reports and press releases (copies of which are 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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