*Press Release*

# Pharnext to Announce Top-Line Results from the Pivotal Phase 3 Trial of PXT3003 for the Treatment of Charcot-Marie-Tooth Type 1A Disease by October 2018

**PARIS, France, 7:30 pm, June 21, 2018 (CEST) – 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, today announced an update from its ongoing Phase 3 clinical program (PLEO-CMT and PLEO-CMT-FU studies) evaluating PXT3003 for the treatment of Charcot-Marie-Tooth type 1A disease (CMT1A) in adults. Top-line results from the pivotal PLEO-CMT study are now expected by October 2018.

**Prof. Daniel Cohen, M.D., Ph.D., Pharnext's Co-Founder and Chief Executive Officer said**: "We are thrilled to bring this Phase 3 clinical trial to completion and we now expect to disclose top-line results by October of this year. Our PLEODRUG™ PXT3003 has already shown initial signals of efficacy in our Phase 2 trial in CMT1A. We are hopeful we can bring this much-needed therapy to patients suffering from this debilitating condition, as they currently have limited therapeutic options, most of which are palliative in nature."

Pharnext's first-in-class PLEODRUG™ PXT3003, developed using Pharnext's R&D platform, PLEOTHERAPY™, is a novel oral fixed-low dose combination of baclofen, naltrexone and sorbitol, with orphan drug designation both in Europe and the United States. PXT3003 has already demonstrated safety and tolerability, and the highest dose showed consistent evidence of improvement beyond disease stabilization in CMT1A in the Phase 2 trial where efficacy was assessed using both the Charcot-Marie-Tooth Neuropathy Score (CMTNS) and the Overall Neuropathy Limitations Scale (ONLS) as main endpoints. The results from this Phase 2 trial were published in the Orphanet Journal of Rare Diseases.

PXT3003 is currently being evaluated in a pivotal, multi-center, randomized, 15-month, double-blind, placebo-controlled Phase 3 clinical study ("PLEO-CMT"). The trial was initiated in December 2015 and has enrolled patients aged 16 and older with mild-to-moderate CMT1A in 30 sites across Europe, the U.S. and Canada. The primary endpoint is the change in ONLS score at 12 and 15 months of treatment. Additional outcome measures are being assessed including functional and electrophysiological endpoints.

Patients who had completed the Phase 3 PLEO-CMT trial have been enrolled in a multi-center, 9-month, Phase 3 follow-up extension study ("PLEO-CMT-FU") initiated in March 2016. The study was designed to assess the long-term safety and tolerability of PXT3003 .

Following the successful completion of multiple milestones, including a pre-specified evaluation by the independent Data Safety Monitoring Board in September 2017, a blind variability analysis in November 2017 and a futility analysis in November 2017, Pharnext now expects to announce top-line results from the pivotal PLEO-CMT study by October 2018.

## About 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 at least 125,000 people in Europe and the U.S. 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) responsible for nerve dysfunction, followed by loss of nerve conduction. As a result of peripheral nerve degradation, patients suffer from progressive muscle atrophy of legs and arms causing walking, running, balance problems and abnormal hand functioning. CMT1A patients end up in wheelchairs in at least 5% of cases. They might also suffer from mild to moderate sensitive disorders. First symptoms usually appear during adolescence and will progressively evolve through patients' life.

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 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 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 has developed a new drug discovery paradigm based on big genomic data and artificial intelligence: PLEOTHERAPY™. The Company identifies and develops synergic combinations of drugs called PLEODRUG™ offering several key advantages: efficacy, safety and robust intellectual property. 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 Euronext Growth Stock Exchange in Paris (ISIN code: FR0011191287).
For more information, visit http://www.pharnext.com/

## CONTACTS:

**Pharnext**
René Goedkoop
Chief Medical Officer
contact@pharnext.com
+33 (0)1 41 09 22 30

**Financial Communication (France)**
Actifin
Stéphane Ruiz
sruiz@actifin.fr
+33 (0)1 56 88 11 15

**Investor Relations (U.S.)**
Stern Investor Relations, Inc.
Matthew Shinseki
matthew@sternir.com
+1 212 362 1200

**Investor Relations (Europe)**
MC Services AG
Anne Hennecke
anne.hennecke@mc-services.eu
+49 211 529252 22

**Media Relations (Europe)**
Ulysse Communication
Bruno Arabian
barabian@ulysse-communication.com
+33 (0)1 81 70 96 30

**Media Relations (U.S.)**
RooneyPartners
Kate L. Barrette
kbarrette@rooneyco.com
+1 212 223 0561