*Presse Release*

# Successful Intermediate Analyses for PLEO-CMT Phase III Clinical Trial in Charcot-Marie-Tooth Disease Type 1A

## PLEO-CMT study to continue as planned without increasing the number of patients

**PARIS, France, 6:30pm, November 29, 2017 (CET) – Pharnext SA (FR0011191287 - ALPHA)**, a biopharmaceutical company pioneering a new approach for the development of innovative drugs based on the combination and repositioning of known drugs, today announced successful intermediate analyses for its Phase III clinical trial, PLEO-CMT, in Charcot-Marie-Tooth Disease Type 1A (CMT1A).

PLEO-CMT is a pivotal, multi-center, randomized, 15 months, double blind, placebo-controlled Phase III study that was initiated in December 2015 and has enrolled 323 patients with mild-to-moderate CMT1A in 30 sites across Europe, the U.S. and Canada. As the study will be completed at the end of 2018, two intermediate analyses, a blind variability analysis followed by a futility analysis, were therefore carried out as planned.

According to Professor Philippe Lehert, member of the Independent Data Safety Monitoring Board (DSMB), the variability of tests between patients is indeed within predefined limits. In addition, the futility analysis concludes that PLEO-CMT is sufficiently powered to detect an effect of PXT3003 on the primary efficacy endpoint. These two analyses follow two favorable intermediate safety analyses delivered by the DSMB in November 2016 and September 2017 and indicate therefore that PLEO-CMT can continue according to the original plan without having to increase the trial size.

**Pr. Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext said** *« These two analyses are additional steps successfully reached for the PLEO-CMT study. They allow us to confirm the schedule for final results by the second half of 2018. »*

### About the Data Safety Monitoring Board (DSMB)

The DSMB is an independent body of experts drawn from the fields of clinical medicine, biostatistics and study methodology, chartered to provide recommendations to Pharnext upon regular pre-specified review of the accumulated data during the conduct of the clinical trial.

### 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 III 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: FR0011191287).
For more information, visit www.pharnext.com

**CONTACTS:**

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

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

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

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

**Media Relations (Europe)**
ALIZE RP
Caroline Carmagnol
Aurore Gangloff
pharnext@alizerp.com
+33 (0)1 44 54 36 64

**Media Relations (U.S.)**
RooneyPartners
Marion Janic
mjanic@rooneyco.com
+1 212 223 4017