Press Release

European Medicines Agency Agrees with Pharnext's Pediatric Investigation Plan for PXT3003

This agreement paves the way for the submission of a marketing authorization application in Europe

PARIS, France, 7:30 am, July 10, 2018 (CEST) – Pharnext SA (FR0011191287 – ALPHA) a biopharmaceutical company pioneering a new approach to the development of innovative drug combinations based on big genomic data and artificial intelligence, today announced that the European Medicines Agency (EMA) has agreed with its pediatric investigation plan (PIP) for PXT3003 in Charcot-Marie-Tooth disease type 1A (CMT1A).

"The EMA agreement with our PIP represents a significant step forward in our efforts to bring PXT3003 to patients in Europe", **said Prof. Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext**. *"Given that much of the progression of CMT1A occurs in the first two decades of patients' lives, we believe that by intervening in childhood, we can have a greater impact on patients' disease trajectory. In our Phase 2 study, PXT3003 was observed to stablilize and slow the progression of CMT1A, and also demonstrated clinical improvement in patients. We are dedicated to providing the estimated 14,000 children in Europe living with CMT1A a safe therapeutic option that may offer significant long-term relief."*

As part of the regulatory process for registering new medicines with the EMA, pharmaceutical companies are required to provide a PIP that outlines the clinical development strategy for studying the investigational product in children. EMA agreement with the PIP is required before a company can file a marketing authorization application (MAA) for any new medicinal product in Europe.

PXT3003 is being evaluated in an international pivotal Phase 3 clinical trial in adults with CMT1A, with top-line results expected by October 2018. The clinical study investigating the safety and efficacy of PXT3003 for CMT1A in children will be conducted in Europe, Canada and the United States.

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
medical@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