Press release

**PHARNEXT has completed patient recruitment for its Phase II clinical trial of PXT3003 (the company’s first Pleodrug™) in Charcot-Marie-Tooth disease**

*The trial is praised by the journal Nature Medicine*

**Paris, 22 November 2011** - Pharnext SAS, a biopharmaceutical company specializing in the development of innovative treatments based on Pleotherapy™ for severe, unmet medical needs, today announced the on-schedule completion of the recruitment of 80 patients as part of its Phase II study of PXT3003 (the company’s first Pleodrug™) in type 1A Charcot-Marie-Tooth disease¹ (CMT1A). Pharnext’s combination therapy was developed in just only three years, versus the eight years generally required for novel compounds; the company has been able to move directly from preclinical development to Phase II clinical trials of the Pleodrug™’s efficacy.

"PXT3003 provides a real prospect of improving the status of patients suffering from CMT1A. Recruitment went according to schedule and none of the enrolled patients have dropped out of the study so far, which is very encouraging”, stated Dr Shahram Attarian (the CLN-PXT3003-01 study’s coordinating investigator at La Timone University Medical Center (Marseilles, France)).

"We are delighted to have reached this key milestone with the first Pleodrug™ generated by our proprietary discovery platform. We are getting ever closer to our objective: to provide patients suffering from Charcot-Marie-Tooth disease (which is currently untreatable) with a safe, effective therapy. We are really looking forward to sharing some interesting results (expected in early 2013) with them”, emphasized Professor Daniel Cohen, Pharnext’s founder and CEO.

Patient recruitment started in late December 2010 in six investigating centers across France (Lille, Limoges, Lyons, Marseilles, Nantes and Paris) and completed on schedule. The Phase II clinical trial is a double-blind, placebo-controlled, randomized multicenter study. The patients were randomized between the study’s 4 arms: a placebo arm and 3 arms with the PXT3003 study treatment administered as 3 oral doses twice a day.

PXT3003 is the first drug candidate to be generated by Pharnext’s Pleotherapy™ platform. PXT3003 is composed of a combination of off-patent, approved three drugs – each of which is marketed separately for the treatment of another disease and is administered here at much lower doses than in the usual indication. The study’s objective is to evaluate the pharmacodynamics, pharmacokinetics, safety and efficacy of PXT3003 in CMT1A patients aged 18 to 65 and with genetic confirmation of a duplication on chromosome 17. The results are likely to be published in the first quarter of 2013.

An article in the journal *Nature Medicine* (“Networking for new drugs”, October 2011, vol. 17(10); 1166-1168) described the trial’s novelty in its introduction and commented that whereas many of

¹ Charcot-Marie-Tooth disease is a severe, invalidating, chronic neuromuscular disease that affects 3 million people worldwide and for which there is no treatment.
today's most celebrated drugs are designed to hit only one biological target with great precision, medicine's proverbial “magic bullet” might soon give way to a more sophisticated arsenal when using “network pharmacology” (like Pharmnext's approach) to efficiently tackle severe medical needs.

The Pharmnext approach
Pharmnext focuses on meeting a range of medical needs, including diseases for which there is no satisfactory treatment. This is achieved by moving from conventional, single-drug therapy to Pleotherapy™. In fact, Pleotherapy™ involves a single, patented Pleodrug™ combining mini-doses of several off-patent drugs already approved by the health authorities for the treatment of diseases that are biologically but not clinically linked to the target indication. The company has created a very sophisticated Pleotherapy™ R&D structure and a powerful, proprietary Pleodrug™ discovery engine based on “NEXUS”, a new, protected, genomic process that makes it possible to efficiently create Pleodrugs™ and which is applicable to most diseases.

Pharmnext has particularly solid financial foundations:
- a €3.5-million round of seed fundraising with Truffle Capital (December 2007).
- an agreement with the pharmaceutical group Ipsen, potentially worth up to €94 million in upfront fees and milestone payments (June 2009).
- an initial grant from Oséo (the French state innovation agency) worth 3.4 million Euros (Charcot-Marie-Tooth, 2007).
- a grant from the French Muscular Dystrophy Association (AFM, 2009).
- the Project DIPPAL financed by ISI (OSEO’s Strategic Manufacturing Innovation program) to the tune of €10.4 million over 6 years, in order to deliver diagnostic and therapeutic solutions to patients with Alzheimer’s disease. Pharmnext is leading the DIPPAL project and will receive €7.3 million (November 2010).
- a €4.75 million round of financing (June 2010).
- a €2.50 million round of financing (September 2011).

About Pharmnext
Pharmnext is a biopharmaceutical company founded in Paris in 2007 by Professor Daniel Cohen MD, PhD, his team of research scientists (comprising pioneers in the field of genomics) and Philippe Pouletty, MD (General Partner, Truffle Capital). Pharmnext specializes in the discovery and development of novel pharmaceutical treatments. Based on its founders' extensive experience (over 20 years) in human genetics and systems biology, Pharmnext has perfected an innovative, proprietary process for reconstructing the complex biological networks associated with a given disease. By understanding these networks, Pharmnext identifies and develops Pleodrugs™, a cocktail of low-dose, off-patent drugs already approved for other diseases. The Pleodrugs™ target different nodes of the complex biological networks associated with the disease. Pharmnext performs all aspects of Pleodrug™ R&D up to the end of Phase II clinical trials, before licensing the product to the pharmaceutical industry. Although this approach can (in principle) be applied to any disease, the company is concentrating on neurodegenerative disease, metabolic disease and other serious, chronic diseases.

Disclaimer: Certain statements contained in this press release regarding matters that are not historical facts may be qualified as forward-looking statements. Such statements are subject to risks and uncertainties.

Contacts:

PHARNEXT
Daniel Cohen
+33 1 41 09 22 30
daniel.cohen@pharnext.com

ALIZE RP
Caroline Carmagnol
+33 6 64 18 99 59
caroline@alizerp.com

Anne-Sophie Cosquéric
+33 1 42 68 86 41
anne-sophie@alizerp.com