Press Release

Pharnext announces the registration of its *document de base* as part of its planned IPO on Euronext's Alternext market in Paris

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Paris, France, 3 June 2016 – Pharnext, a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, announces today the registration of its *document de base* by the French *Autorité des marchés financiers* (AMF) under number I.16-050 on June 2, 2016 as part of its planned IPO on Euronext's Alternext market in Paris.

Registration of the *document de base* constitutes the first stage of Pharnext's planned IPO on Euronext's Alternext market in Paris, which is contingent on market conditions and regulatory requirements including the AMF's approval of a prospectus to be prepared as part of the transaction.

Pharnext is developing new treatments targeting **severe neurodegenerative diseases** – both rare and common – which currently have no satisfactory therapeutic solution, such as Charcot-Marie-Tooth type 1A disease and Alzheimer's disease. These treatments, called **pleodrugs, are synergistic lower dose combinations of drugs that have already been approved, but for other diseases.**

◉ Pharnext, a unique approach in the development of new drugs

The pharmaceutical sector is now facing numerous challenges such as higher prices of drugs or higher failure rates in the development of a new drug. Faced with these challenges, Pharnext is proposing **a new breakthrough technology/therapeutic approach to better treat an ever increasing number of patients at an affordable cost: Pleotherapy.**

Developed over the last 30 years, this concept differs from monotherapy, which focuses on a single therapeutic target, by allowing the treatment of **several targets at the same time** thanks to **pleodrugs**. These are **synergistic lower dose combinations of drugs that have already been used** for other diseases and have a known tolerance profile.

◉ Pleotherapy would offer considerable advantages compared to monotherapy

- An **excellent innocuity profile**: the molecules used have already been approved and are used at lower doses,

- **Optimised efficiency**: the molecules consisting of pleodrugs have a **synergistic therapeutic effect** making it possible to focus on several targets within the biological network of a single disease,
- A potentially pleiotropic effect, i.e. having multiple therapeutic effects, allowing a single candidate drug **to target multiple pathological conditions,**
- A **considerably reduced process and development cost**: the technology developed by Pharnext enables to start clinical development only 3 years after initiating work on a new disease. In addition, since the molecules have a known tolerance profile, the Phase 1 clinical trial is not always obligatory. Overall, the gain is estimated to be of 5 years,
- Considerable **flexibility regarding the price of drugs, due to the reduced development costs,**
- **Far-reaching and solid intellectual property**: pleodrugs are protected by patents equivalent to those of conventional drugs,

To demonstrate the suitability of this approach, Pharnext has chosen diseases with high unmet medical needs and no satisfactory treatment to date: Charcot-Marie-Tooth type 1A disease and Alzheimer's disease.

◉ PXT3003, the first candidate drug for Charcot-Marie-Tooth type 1A disease, currently in Phase 3, following very encouraging Phase 2 results for the candidate drug at this stage of clinical development

Type 1A Charcot-Marie-Tooth disease is a hereditary genetic disease causing both motor and sensory neuron damages.

It affects roughly 170,000 patients in Europe and North America. To date, no treatment is capable of slowing, stabilising or reversing the natural development of the disease.

Having obtained positive results in Phase 2, PXT3003 is currently in pivotal Phase 3 international clinical trials in Europe and the United States. The results of these trials should be available in the second half of 2018.

◉ PXT864, the second candidate drug currently under development for Alzheimer's disease, has just completed Phase 2a, having demonstrated a significant improvement - for the candidate drug at this stage of clinical development - in the cognitive functions of patients during treatment

Alzheimer's disease is a chronic neurodegenerative disorder leading to the slow and gradual destruction of neurons. More than 30 million people are currently affected by this disease globally. The prevalence of the disease is rapidly increasing and according to estimates between 70 and 100 million people may be affected around the world between now and 2050.

Currently, there is no drug capable of curing the disease or slowing its development.

PXT864 has just **completed Phase 2a** with **encouraging preliminary results.** A new Phase 2 international trial is scheduled for the first half of 2017 with a larger number of patients, with results expected at the end of 2019/beginning 2020.

◉ Considerable potential for other opportunities in areas of neurodegenerative diseases

Thanks to its knowledge of the biological networks of diseases and of the repositioning of drugs, Pharnext is developing a unique platform which can systematically discover synergistic combinations of drugs. The aim of this platform is to target **major markets** in areas of neurodegenerative diseases: to date **the biological networks of 26 diseases with unmet medical needs** have been developed by Pharnext.

◉ Pharnext is supported by an internationally renowned scientific team and first tier investors

This new paradigm pioneered by Pharnext is recognized by internationally renowned scientists, including several **Nobel Prizes**. The Company has also been supported by **Truffle Capital** since its creation, a renowned investor specialising in the health care sector and since 2011 by **Pierre Bastid** who has been also invested in several biotechnology companies such as Carmat and Cellectis of which he is a board member.

◉ How to obtain the registration document

Pharnext's *document de base* is available on the company website (www.pharnext.com) and the AMF's website (www.amf-france.org), and free of charge on request from the company headquarters: 11, rue des Peupliers, 92130 Issy-les-Moulineaux, France.

◉ Risk factors

The company draws attention to Chapter 4 'Risk Factors' particularly the liquidity risk in the *document de base* registered with the AMF.

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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 focuses on neurodegenerative diseases and has two lead products in clinical development: PXT-3003 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. PXT-864 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 low dose. These "pleodrugs" offer several key advantages: efficacy, safety, and intellectual property including several composition of matter patents already granted. The Company is supported by a world-class scientific team.

For more information please visit www.pharnext.com

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