

## Pharnext Presents Positive Exploratory Phase 2 Data From PXT864 at the 9<sup>th</sup> Clinical Trials on Alzheimer's Disease (CTAD) Conference in San Diego, United States

*Data from the Phase 2 trial suggest promising efficacy of PXT864 in mild Alzheimer's Disease  
The trial also confirmed excellent safety and tolerability of PXT864 over 36 weeks of treatment*

**Paris, December 13, 2016 – Pharnext SA (FR00111911287 - ALPHA)**, a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, presented positive exploratory Phase 2 data for PXT864, a PLEODRUG<sup>®</sup> being developed for the treatment of Alzheimer's disease (AD), at the 9<sup>th</sup> Clinical Trials on Alzheimer's Disease (CTAD) conference, on December 8-10, 2016 in San Diego (United States).

Data collected during the PLEODIAL<sup>®</sup> clinical trial suggests that:

- **PXT864 is well tolerated and safe for use in patients with AD:** excellent compliance rate above 90%, without serious or unexpected Adverse Events (AE) and no AE led to medical intervention or study treatment discontinuation.
- **PXT864 may slow the progression of cognitive disability in patients with mild AD.** The primary endpoint of the study – AD Assessment Scale Cognitive Subscale test (ADAS-Cog-11) – appeared to decline less in the pooled PXT864 dose 1 and 2 groups as compared to historical placebo for mild to moderate patients with AD at week 36.
- **PXT864 may be used concomitantly with low-dose donepezil treatment (5 mg daily):** no new safety concern arose when donepezil was administered concomitantly with PXT864 at week 24 and onwards. In addition, concomitant PXT864-donepezil administration pointed towards an improvement or at least a stabilization of ADAS-Cog-11 score at week 36.

These findings show promising efficacy of PXT864 to be further explored in future studies, such as an international multicenter adaptive-design Phase 2 study.

*“We believe that these findings bring new evidence of PXT864 safety and efficacy in patients suffering from mild stages of Alzheimer's disease and this clinical trial has the potential to be a key turning point in the effort to finally provide an efficacious treatment for these patients,”* said **Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext**. *“These data also further validate our PLEOTHERAPY<sup>®</sup> research and development approach and we hope to provide novel treatments for many diseases via such synergic drug combinations.”*

**René Goedkoop, M.D., Chief Medical Officer of Pharnext** added, *“The data presented provide encouraging evidence for PXT864 as an entirely new approach in the treatment of Alzheimer’s disease. Today there are no treatments capable of altering the progressive course of this terrible disease. We look forward to the continuation of PXT864 clinical development and hope that we can bring an efficacious treatment to people suffering from Alzheimer’s disease and to improve their quality of life.”*

PXT864 is a novel rational-design synergic fixed combination of baclofen and acamprosate given orally as a capsule twice a day. PXT864 is thought to restore the disrupted excitatory – inhibitory balance in the brain of patients suffering from neurodegenerative diseases (disrupted by toxic factors such as A $\beta$  oligomeric peptides in Alzheimer’s disease). PXT864’s most advanced target indication is Alzheimer’s disease. Clinical development of PXT864 in other neurodegenerative diseases including Parkinson’s disease and amyotrophic lateral sclerosis (ALS) is also planned.

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#### About Alzheimer’s Disease

Alzheimer’s disease is an irreversible, progressive neurodegenerative dementia. It is characterized by neuronal death in brain structures implicated with memory leading to cognitive deficits such as thinking, memory, personality and behavior disorders. The disease typically develops and worsens gradually over the course of several years and ultimately leads to death. It affects around 25 million people worldwide. Unfortunately, three out of four patients are only diagnosed once the disease is at a severe stage. Alzheimer's disease has no cure and current existing therapies provide only short and modest symptomatic relief.

#### About PLEODIAL<sup>®</sup> Trial

The PLEODIAL<sup>®</sup> trial was an exploratory multicenter single blind Phase 2 study that evaluated 3 doses of PXT864 in 45 patients with mild Alzheimer’s disease who were naïve to anti-dementia treatment: the first two doses were based on the same ratio of baclofen / acamprosate [dose 1 and 2] and the third dose used another ratio [dose 3]. The main objectives were to assess safety, compliance and preliminary efficacy on cognitive and behavioral impairment. The study was designed over a period of 36 weeks: “PLEODIAL-I” for the first 12 weeks

followed by “PLEODIAL-II” for the remaining 24 weeks. During PLEODIAL-I, patients received PXT864 during the first 4 weeks (“challenge”) followed by 4 weeks of placebo (“dechallenge”) and then 4 weeks of PXT864 (“re-challenge”). During PLEODIAL-II, patients were invited to continue on PXT864 with the dose they had received during PLEODIAL-I. During the last 12 weeks of the study, physicians were authorized to co-administrate donepezil 5 mg with PXT864. In total, patients were treated with PXT864 for 32 out of 36 weeks in the PLEODIAL-I and II studies. This clinical trial was conducted in 7 French memory centers (CMRR: Centre Mémoire de Ressource et de Recherche) from February 2013 (first patient in) to December 2015 (last patient out).

### **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: 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 is the pioneer of a new drug discovery paradigm: PLEOTHERAPY®. The Company identifies and develops synergic combinations of repositioned drugs at low dose. These PLEODRUG® 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.

The company Pharnext is listed on Euronext Alternext Stock Exchange in Paris (ISIN code: FR00111911287).

For more information, visit [www.pharnext.com](http://www.pharnext.com)