

Press release

Pharnext to Present Phase 2 Data From PXT864 at the 9th Clinical Trials on Alzheimer's Disease (CTAD) Conference in San Diego, United States

Data from Phase 2 study accepted as oral and poster presentations

Paris, December 1, 2016 – Pharnext SA (FR00111911287 - ALPHA), a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, today announced that Phase 2 clinical trial data related to PXT864, a PLEODRUG® developed for the treatment of Alzheimer's disease (AD), will be presented at the 9th Clinical Trials on Alzheimer's Disease (CTAD) conference, December 8-10, 2016 in San Diego (United States).

Details are as follows:

Date	Time	Event
Friday		Poster Session 2, P2-12
December 9		
		Neurophysiological Effect of PXT864 in mild
		Alzheimer's Disease Patients
		Presenter:
		Karim Bennys, MD, Memory Research Resource
		Center for Alzheimer's disease, University
		Hospital, Montpellier, France
Saturday	7:30 am	Oral Communications Sessions, OC 54
December 10		
		36 Weeks of Treatment with PXT864 in Mild
		Alzheimer's disease: Results from the PLEODIAL
		Extension Study
		Presenter:
		René Goedkoop, MD, Chief Medical Officer,
		Pharnext, Issy-les-Moulineaux, France

Safety and clinical efficacy of PXT864 were assessed in 45 treatment-naïve patients with mild AD in a multicenter, exploratory, single blind Phase 2 study (PLEODIAL-I). Three doses of PXT864 were administered for 4 weeks

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followed by 4 weeks of placebo and then a 4-week re-challenge with PXT864. Subjects were then invited to continue study treatment (same dose received in PLEODIAL-I) for a further 24 weeks in the PLEODIAL-II extension study. Patients were treated with PXT864 for 32 out of 36 weeks in the PLEODIAL-I and II studies, and were allowed to use donepezil 5 mg, if their condition warranted treatment with a symptomatic agent, during the last 12 weeks of the study.

PXT864 is a novel synergic fixed combination of baclofen and acamprosate given orally as a capsule twice a day. PXT864 was developed 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. Development in other neurodegenerative diseases including Parkinson's disease and amyotrophic lateral sclerosis (ALS) are 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 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 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

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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 <u>www.pharnext.com</u>