Press Release

# Pharnext refocuses its clinical trial programs on PXT3003, its most promising drug candidate, to optimize financial resources allocation

**PARIS, France, February 14th, 2023, 08:30 am CET – Pharnext SA (FR001400BV89 - ALPHA)** (the "Company"), an advanced late-clinical stage biopharmaceutical company developing novel therapeutics for neurodegenerative diseases with high unmet medical need, announces today its strategic decision to focus all resources on its lead drug candidate, PXT3003, in Charcot-Marie-Tooth disease type 1A (CMT1A), a rare, debilitating, and inherited neuropathy with no specific therapeutic solution. The Company believes that PXT3003 in CMT1A is its best near-term opportunity for value creation, with results from the pivotal Phase III clinical trial, the PREMIER trial, expected in Q4 2023.

In this context, the Company's Board of Directors has decided to stop the development of its drug candidate PXT864 in Alzheimer's disease. Although PXT864 has generated interesting and encouraging preclinical and clinical results, these remain insufficient for commercialization in the short or medium term. Further development of PXT864 in Alzheimer's disease would require the implementation of a considerable additional clinical development plan that the Company does not wish to finance at this time.

Nonetheless, data from clinical studies conducted with PXT864 are encouraging and the medical needs of Alzheimer's disease remain significant. PXT864 has generated promising in vitro and in vivo preclinical results[1] (available here), and has conducted Phase I and IIa clinical studies in healthy volunteers and in patients with Alzheimer's disease[2] (CTAD conference poster available here). Further development of PXT864 would therefore be justified, and Pharnext will continue to seek an industrial partner with the resources to pursue the development of PXT864 in Alzheimer's disease. Some companies have already expressed interest in licensing PXT864 from Pharnext and pursuing its development. Other companies interested in licensing the product may contact Pharnext directly (contact@pharnext.com).

Meanwhile, Pharnext reminds that PXT864 has also been tested for the treatment of Amyotrophic Lateral Sclerosis ("ALS" also known as Charcot's disease or Lou-Gehrig's disease), an indication for which the medical needs are also consequential. Very encouraging in vitro and in vivo preclinical results have been obtained in this indication[3] (available here). Pharnext is currently considering the best strategy to pursue the development of PXT864 in ALS (preclinical and clinical): in-licensing to an industrial partner or resuming a more advanced development program internally, after the potential success of the pivotal Phase III of PXT3003 in CMT1A.

**Hugo Brugière, Chairman and CEO of Pharnext, said**: "*As I stated when joining Pharnext, I set myself three priorities: to ensure the Company's ability to complete clinical trials with PXT3003, to reduce the financing needs as much as possible in order to limit the use of financing lines and to enhance the value of assets. Today's decision is perfectly in line with these three objectives. However, the results obtained with PXT864 in Alzheimer's disease are far from anecdotal, making it essential to find the right partner, capable of giving this product every chance of success. This is what Pharnext teams are now working on*".

[1] Chumakov I, et al. Combining two repurposed drugs as a promising approach for Alzheimer's disease therapy. Scientific Reports (Nature) 2015 Jan 8 ;7608.

[2] Touchon J, et al. Direct double-blind analysis arguing for synergistic therapeutic effect of a fised low-dose combination of acamprosate and baclofen in patients with AD. 2017 Nov 1-4. STAD Poster #LBP32BIS.

[3] Boussicault L, et al. Combination of acamprosate and baclofen (PXT864) as a potential new therapy for amyotrophic lateral sclerosis. J NeuroScience Res, 2020;98(12):2435-2450.

**About PXT864**

PXT864 is a novel fixed-dose synergistic combination of baclofen and acamprosate formulated as a pill given twice a day. PXT864's assumed main mechanism of action in neurodegenerative diseases is the restoration of balance between excitatory (glutamate activity) and inhibitory (GABA activity) pathways.

**About Pharnext**

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapies for neurodegenerative diseases currently without satisfactory therapeutic solutions. Pharnext has a first-in-class drug candidate, PXT3003, in development for Charcot-Marie-Tooth disease type 1A (CMT1A), a rare, disabilitating, inherited peripheral neuropathy. PXT3003 benefits from orphan drug status in Europe and the United States. In 2018, PXT3003 completed a Phase III clinical trial, the PLEO-CMT trial, with encouraging topline results. This trial was followed by an open-label extension study, the PLEO-CMT-FU trial, with 120 patients continuing treatment with PXT3003. Long-term data suggest a sustained benefit, safety, and efficacy, after 5 years of total trial time. An international pivotal Phase III study of PXT3003, the PREMIER trial, is currently ongoing with 387 CMT1A patients enrolled. PREMIER topline results are expected in Q4 2023. PXT3003 originated from the Pleotherapy™ R&D approach. Pharnext draws the attention of investors to the financial and other risk factors detailed in its financial reports. More information can be found at www.pharnext.com.
Pharnext is listed on the Euronext Growth market in Paris (ISIN code: FR001400BV89).

**Contacts**

**Press Relations (International)**
Consilium Strategic Communications
Mary-Jane Elliott
Sukaina Virji
Alexandra Harrison
pharnext@consilium-comms.com

**Financial Press Relations**
ACTUS finance & communication
Deborah Schwartz
dschwartz@actus.fr
+33 (0)1 53 67 36 35

**Investor Relations**
ACTUS finance & communication
Jérôme Fabreguettes Leib
pharnext@actus.fr
+33 (0)1 53 67 36 78