Press Release

Pharnext confirms details for 2021 Hybrid Research & Development Day

Event to be held in person in New York, or virtually via a simultaneous live webcast, on Wednesday October 27, 2021 at 8:30 am ET/ 2:30 pm CET

PARIS, France, October 21, 2021, 8:30 a.m. CET – Pharnext SA (FR0011191287 - ALPHA) (the "Company"), an advanced late-stage clinical biopharmaceutical company pioneering new approaches to developing innovative drug combinations based on big genomics data and artificial intelligence using its PLEOTHERAPY™ platform, will be hosting a hybrid Research and Development (R&D) Day, on Wednesday, October 27, 2021, from 8:30 to 10:30 a.m. ET / 2:30 to 4:30 p.m. CET at Convene, 530 Fifth Avenue, New York, NY 10036, which can also be accessed online here.

The Pharnext 2021 R&D Day will focus on its lead clinical asset, PXT3003, in Charcot-Marie-Tooth disease type 1A (CMT1A) and will provide both the opportunity to hear about the Company from the management team and key thought leaders' perspectives on the late-stage clinical development program.

Agenda:

- **08:30 a.m. CET: Welcome & Introduction**
  Dr. David H. Solomon, CEO, Pharnext

- **08:40 a.m. CET: CMT1A Disease Overview**
  Disease basics, unmet need, burden of the disease, patient journey, disease evaluation, treatment expectations
  Mario Saporta, MD, PhD, Associate Professor of Neurology and Human Genetics and Director of CMT Center of Excellence at Miller School of Medicine at the University of Miami (FL, USA) and lead investigator of the PREMIER trial in North America

- **09:00 a.m. CET: CMT1A – The Patient Perspective**
  The impact of CMT1A
  Allison Moore, Founder and CEO, Hereditary Neuropathy Foundation

- **09:15 a.m. CET: PXT3003: Candidate Treatment for CMT1A**
  Discovery, mechanism of action, animal pharmacology study, data from Phase II and first Phase III clinical studies, pivotal Phase III study: the PREMIER trial
  Adrian Hepner, MD, PhD, CMO & Head of R&D, Pharnext
  Florian P. Thomas MD, PhD, Founding Chair & Professor, Department of Neurology, Hackensack University Medical Center & Hackensack Meridian School of Medicine (NJ, USA) and U.S. lead investigator of the PLEO-CMT trial
  Mario Saporta, MD, PhD, Associate Professor of Neurology and Human Genetics and Director of CMT Center of Excellence at Miller School of Medicine at the University of Miami (FL, USA) and lead investigator of the PREMIER trial in North America

- **09:45 a.m. CET: PXT3003 Business Opportunity**
  Xavier Paoli, COO, Pharnext

- **09:55 a.m. CET: Closing remarks**
  Dr. David H. Solomon, CEO, Pharnext

- **10:05 a.m. CET: Questions & Answers Session**

Registration to attend is now open and can be accessed on the same link as the webcast here or by visiting the "Events" section of the Pharnext website (www.pharnext.com). An archived webcast will be made available on Pharnext's website after the event.

About Charcot-Marie-Tooth Disease Type 1A ('CMT1A')

Charcot-Marie-Tooth ('CMT') disease encompasses a heterogeneous group of inherited, severe, debilitating, progressive and chronic peripheral neuropathies. CMT1A, the most common type of CMT, is an orphan disease with a prevalence of 1/5000 people affecting about 150,000 people in Europe and the U.S. and about 1,500,000 people worldwide. The genetic mutation responsible for CMT1A is a duplication of the PMP22 gene coding for a peripheral myelin protein. The duplication of this gene results in overexpression of the PMP22 protein and failure of Schwann cells to produce normal myelin (neuronal sheath). The lack of a normal myelin structure and function leads to abnormal peripheral nerve conduction and axonal loss. As a result of peripheral nerve degradation, patients suffer from progressive muscle atrophy in both the legs and arms causing problems with walking, running and balance as well as abnormal hand functioning. They might also suffer from mild to moderate sensory disorders. First symptoms usually appear during adolescence and will progressively evolve throughout life. Patients with the most severe form of CMT1A end up in wheelchairs, representing at least 5% of cases. To date, no curative or symptomatic medications have been approved and treatment consists of supportive care such as orthotics, leg braces, physical and occupational therapy or surgery. More information can be found at https://pharnext.com/en/disease/charcot-marie-tooth.

About PXT3003

PXT3003 is a novel fixed-dose synergistic combination of baclofen, naltrexone and sorbitol formulated as an oral solution given twice a day. The three individual components of PXT3003 were selected to downregulate the overexpression of PMP22 protein, leading to improvement of neuronal signaling in dysfunctional peripheral nerves that are an essential part of the pathophysiology of this disease. PXT3003 could also have a positive effect on other cellular types of the motor unit such as the axon (direct protection), neuromuscular junctions or muscle cells. PXT3003 has shown promising and consistent results across preclinical and clinical studies in Phase II and Phase III (PLEO-CMT and PLEO-CMT-FU). More information can be found at https://pharnext.com/en/pipeline/pxt3003.

About Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for orphan and common neurodegenerative diseases that currently lack curative and/or disease-modifying treatments. Pharnext has two lead products in clinical development. PXT3003 completed an international Phase III trial with positive topline results for the treatment of Charcot-Marie-Tooth disease type 1A ('CMT1A') and benefits from orphan drug status in Europe and the United States. An international pivotal Phase III study of PXT3003 in CMT1A, the PREMIER trial, is currently ongoing. PXT864 has generated encouraging Phase II results in Alzheimer's disease and will be advanced through partnerships. Pharnext has developed a new drug discovery paradigm based on big genomics data and artificial intelligence: PLEOTHERAPY™. Pharnext identifies and develops synergic combinations of drugs called PLEODRUG™. More information can be found at www.pharnext.com.

Pharnext is listed on the Euronext Growth Stock Exchange in Paris (ISIN code: FR0011191287).

Disclaimer

This press release contains certain forward-looking statements concerning Pharnext and its business, including in respect of timing of and prospects for clinical trials and regulatory submissions of the Company's product candidates as well as a potential financing transaction, the use of proceeds therefrom and cash runway. Such forward-looking statements are based on assumptions that Pharnext considers to be reasonable. However, there can be no assurance that the estimates contained in such forward-looking statements will be verified, which estimates are subject to numerous risks including the risks set forth in Pharnext's URD approved by the AMF on November 9, 2020 under number N° R. 20-029 as well as in its annual periodic management reports and press releases (copies of which are available on www.pharnext.com) and to the development of economic conditions, financial markets and the markets in which Pharnext operates. The forward-looking statements contained in this press release are also subject to risks not yet known to Pharnext or not currently considered material by Pharnext. The occurrence of all or part of such risks could cause actual results, financial conditions, performance or achievements of Pharnext to be materially different from such forward-looking statements. Pharnext disclaims any intention or obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events, or otherwise.

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