Press Release

Pharnext and the Charcot–Marie–Tooth Association Enter Biomarker Research Collaboration

Collaboration focused on identifying and characterizing key biomarkers in Charcot-Marie-Tooth disease type 1A

PARIS, France, 08:30 am, September 3rd, 2020 (CET) – Pharnext SA (FR0011191287 - ALPHA), an advanced clinical-stage biopharmaceutical company pioneering new approaches to develop innovative drug combinations based on big genomics data and artificial intelligence, today announced a research collaboration with the United States patient advocacy group, the **Charcot–Marie–Tooth Association (CMTA)**, to investigate novel biomarkers associated with the Charcot-Marie-Tooth disease type 1A (CMT1A), the most common subtype of the disease.

The primary objective of this collaboration is to identify and validate potential treatment responsive CMT1A biomarkers that could be further explored in future clinical studies, in particular the upcoming Phase III study of PXT3003, Pharnext's lead drug candidate. Pharnext intends to investigate blood samples collected from mild to moderate CMT1A patients enrolled in the first Phase III study of PXT3003. Notably, this collaboration will evaluate the potential of TMPRSS5, a recently identified Schwann cell-specific biomarker in CMT1A patients, to confirm if it can be used to assess treatment response in future clinical trials. As TMPRSS5 is actually part of a broader neurology panel that tests many additional potential biomarkers, the collaboration between Pharnext and the CMTA could lead to the identification of additional biomarkers in CMT1A that are not described yet.

"This exciting collaboration between Pharnext and the Charcot-Marie-Tooth Association underscores the importance of involving patient advocacy organizations in better understanding the disease and working to bring new therapies to CMT1A patients," **said Dr. David Horn Solomon, Chief Executive Officer of Pharnext.** "*Through this collaboration, we aim to further assess blood samples collected during our first Phase III trial of PXT3003 for novel biomarkers and notably confirm the potential of TMPRSS5 in CMT1A. Results of this research collaboration might inform the addition of new exploratory endpoints in our next Phase III trial of PXT3003 to be initiated in Q1 2021. We believe this alliance will enable us to accelerate our efforts in bringing a safe and effective therapeutic for this disease that currently has no viable treatment options."*

"As advocates for the CMT patient population, we are thrilled Pharnext is focusing its clinical development effort on CMT1A," **said Gilles Bouchard, Chairman of CMTA.** *"Our collaboration with Pharnext aims to identify potential biomarkers for CMT1A which is crucial to better understand the pathophysiology of the disease, as well as to evaluate new therapeutic agents in future clinical trials. The development of TMPRSS5 evolved from a STAR (Strategy to Accelerate Research) collaboration involving CMTA Board members Michael Shy and John Svaren, and the Inherited Neuropathy Consortium. This could bring the CMT patient community a step closer to finding a treatment for this serious and debilitating disease for which there is currently no treatment available."*

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 and benefits from orphan drug status in Europe and the United States. PXT864 has generated encouraging Phase II results in

Alzheimer's disease. 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™. The Company was founded by renowned scientists and entrepreneurs including Professor Daniel Cohen, a pioneer in modern genomics, and is supported by a world-class scientific team. More information at www.pharnext.com.

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

About CMTA

The CMTA is aggressively leading the fight to fund the development of treatments and, ultimately, a cure for CMT. Through its Strategy to Accelerate Research (STAR), the CMTA brings together the best CMT researchers, clinicians and experts in therapy evaluation to partner with pharmaceutical and biotechnology companies and patients to expedite the development of treatments for CMT. The CMTA is also actively working to help improve the quality of life for all families living with CMT by offering educational programs and materials, hosting patient and professional conferences, providing support to families through its nationwide branch system through North America and more. More information at www.cmtausa.org.

Disclaimer

This press release contains certain forward-looking statements concerning Pharnext and its business. 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 document de base filed with the AMF on June 2, 2016 under number I.016-0050 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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