Shareholder Letter

PARIS, France, 8:30 a.m. CET, June 17, 2021 – Pharnext SA (FR0011191287 - ALPHA), an advanced late-stage clinical biopharmaceutical company pioneering new approaches to developing innovative drug combinations based on big genomics data and artificial intelligence ("Company") using its PLEOTHERAPY™ platform, today published a Letter to Shareholders from its Chief Executive Officer, Dr. David Horn Solomon.

Dear Fellow Shareholders,

2021 has already seen our Company accomplish a significant key milestone: enrolling the first patient in March in our pivotal Phase III study of PXT3003, the PREMIER trial. As you are familiar, PXT3003 is our lead asset for the treatment of Charcot-Marie-Tooth disease type 1A (CTM1A), a rare and highly debilitating disease. In April, we announced topline results of the interim analysis from the ongoing open-label Phase III extension study (PLEO-CMT-FU), which followed the first double-blind Phase III (PLEO-CMT), suggesting a good safety profile and sustained efficacy of PXT3003 as measured by the Overall Neuropathy Limitation Scale ('ONLS'), after 4.5 years of total trial time. More recently, we secured have contracted a financing for a total amount of up to €81million over the next thirty-six months through a convertible bond program to extend the cash runway of the company and fund the PREMIER trial through to the data readout in Q3 2023.

Financing

This long-term financing was necessary to fund and complete our pivotal Phase III in CMT1A and more globally extend the cash runaway of Pharnext to ensure its growth and development. We understand the frustration around the dilution associated with the convertible bond financing, but given the timing, the current development stage of our company and non-optimal market conditions, we feel the financing in this way benefits the company for several reasons:

Flexibility: The financing has been structured to ensure Pharnext is in control of the timing of drawing down the various tranches based on our cash needs (for more details, please refer to the press release issued on June 7, 2021) . In addition, depending on circumstances, we may not need to draw down the full amount, meaning the overall dilution could be reduced.

Dilution: An equity raise would have created a significant one-time dilution for shareholders instead of spreading the dilution over several years with the current financing. Morevover, it cannot be guaranteed that subcribers of such an equity raise would not have traded their shares in the stock market.

Opportunity: This financing does not prevent us from pursuing deals and partnership discussions, or new opportunities that could come up from our PLEOTHERAPY platform which we are currently working on, to expand and diversify our pipeline and thus create value. We can, in these instances, withhold the convertible bond financing.

Our Focus on PXT3003 and our Pivotal Phase III Clinical Study in CMT1A, the PREMIER Trial

We continue to focus a substantial proportion of our resources on developing PXT3003 with the ultimate goal of obtaining marketing approval from the FDA and EMA to improve everyday life of patients with CMT1A and reduce the burden on their relatives. Currently, there are no approved therapies to treat CMT1A, and PXT3003, which has Orphan Drug Designation from both the FDA and EMA, is the most advanced pharmaceutical product candidate in development for this disease. Given there are over 100,000 CMT1A patients across the US and EU5 markets, there is little competition in the CMT1A therapeutic field and our current understanding of the market, we believe that the worldwide annual peak sales opportunity for PXT3003 could exceed $1 billion.

We have high hopes of success in our PREMIER trial for several reasons:

- The Phase II and the first double-blind Phase III (PLEO-CMT) studies have shown encouraging and consistent safety and efficacy data.
- As recently announced in April 2021, the interim analysis topline data from the ongoing open-label Phase III Extension Study (PLEO-CMT-FU) shows sustained benefits for CMT1A patients after 4.5 years of total trial time. Pharnext will continue reporting long-term data from the ongoing extension study on an annual basis.
- The design of the PREMIER trial has the same primary endpoint (ONLS measuring patients' functional motor disability), same patient population (adult mild-to-moderate CMT1A patients) and same PXT3003 formulation (oral solution) as those used in the previous and ongoing Phase III program (PLEO-CMT and PLEO-CMT-FU trials).

The roll out of the PREMIER trial is still ongoing as planned, following enrollment of the first patient in the US in March 2021 despite the COVID-19 pandemic. Ten sites so far have been activated in North America which are actively screening dozens of CMT1A patients. Additional sites are planned to be activated in the US and Canada during the summer 2021, and we are still on track to announce the activation of the first EU and Israeli sites in Q3 2021. We are confident that we will complete enrollment in the PREMIER trial in Q2 2022 as previously announced which will allow our company to disclose topline results of the pivotal Phase III study in Q3 2023.

The Future for Pharnext

We now have the financing in place to fund and complete our pivotal Phase III clinical study of PXT3003, the PREMIER trial, which, if successful, will ultimately form the basis of a marketing authorization and together with the data from a pre-clinical factorial combination study in the CMT1A rat model. All this could potentially bring a new therapeutic option for CMT1A patients. Being granted marketing authorization in Europe and the US for PXT3003 in CMT1A would represent a tremendous value creation opportunity for both patients and our shareholders in the coming years, and this is what we are entirely committed to achieving.

In parallel, we will pursue the evaluation of growth opportunities for our company to potentially enrich our R&D pipeline, either internally by advancing drug candidates from our PLEOTHERAPY™ platform through clinical development; or through R&D partnership and/or deals with other biopharmaceutical companies or renowned academic research institutions.

I will keep you informed on the progress of our business plan on a regular basis, and I sincerely thank you again for your continued support of our Company.

With Best Regards,

David Horn Solomon
Chief Executive Officer

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 the PREMIER Trial

The PREMIER trial is an international, randomized, double-blind, two-arm placebo-controlled, pivotal Phase III study, evaluating the efficacy and safety of PXT3003 versus placebo in mild-to-moderate CMT1A patients, over a 15-month period. The dose of PXT3003 tested in the PREMIER trial corresponds to the high dose ('HD') tested in the prior Phase III

trial ('PLEO-CMT'). As agreed with regulatory agencies, the primary efficacy endpoint will be the Overall Neuropathy Limitations Scale ('ONLS') which measures functional motor disability. The secondary endpoints include the following outcome measures: 1) 10-Meter Walk Test ('10mWT'), 2) Quantified Muscular Testing (bilateral foot dorsiflexion dynamometry), 3) Patient Global Impression of Severity ('PGI-S'), 4) Patient Global Impression of Change ('PGI-C'), 5) Charcot-Marie-Tooth Neuropathy Score, version 2 ('CMTNS-v2'), and 6) Quantified Muscular Testing (hand grip). Safety and tolerability will be monitored throughout the study. Further information on the PREMIER trial can be found on the ClinicalTrials.gov website (study identification number: NCT04762758) here.

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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