

PARIS, France, 08:30 a.m. CET, October 29, 2020 – 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 Shareholder,

2020 is a pivotal year for Pharnext’s development, and we look forward to continued growth and progress in the remainder of the year and ahead. Over the course of this year, we have made several operational and clinical advances, including important leadership appointments, addition of highly qualified independent directors to our Board, and most importantly, we received guidance from the FDA on the development path forward for our lead asset, PXT3003, for the treatment of Charcot-Marie-Tooth disease type 1A (CTM1A), a rare and highly debilitating disease. We believe that we are well-positioned to initiate and execute our second pivotal Phase III clinical trial for our lead program, PXT3003, in CMT1A.

Our Focus on PXT3003 and Upcoming Pivotal Phase III Clinical Trial in CMT1A

We continue to focus most of our resources on developing PXT3003 for CMT1A with the goal of obtaining marketing approval from FDA and EMA. PXT3003 is a novel combination of naltrexone, baclofen and sorbitol, taken orally, and has Orphan Drug Designation from both FDA and EMA. Currently, there are no approved therapies to treat CMT1A, and PXT3003 is the most advanced pharmaceutical product candidate in development. Given there are over 100,000 CMT1A patients across the US and EU5 markets with little competition, we believe that the global annual commercial revenue opportunity for PXT3003 exceeds \$1 billion. Our Company has been transformed and is dedicated to successfully bringing PXT3003 to the market to treat CMT1A patients, improving their quality of life and reducing the burden on their families and caregivers.

Key Events in 2020:

Strengthened senior management with key hires

I joined the Pharnext team as the Chief Executive Officer in April 2020. Having spent almost 30 years in the pharmaceutical industry and serving as the Chief Executive Officer of Silence Therapeutics and Zealand Pharma previously, I have a tremendous appreciation for Pharnext’s vision and rich pipeline assets.

I am honored to have been given the opportunity to lead Pharnext through this next phase of growth. Our PLEOTHERAPY™ platform, by leveraging genomic big data and artificial intelligence, truly offers a novel and innovative approach to identifying potential treatments for a variety of indications. I am grateful to Daniel Cohen who, as a visionary founder, explored the opportunities to leverage the PLEOTHERAPY™ platform to continue to build our pipeline.

In August 2020, Dr. Adrian Hepner joined Pharnext as Chief Medical Officer and Head of R&D. Dr. Hepner has more than 30 years of industry experience in biomedical research, clinical drug development and medical affairs. Prior to joining Pharnext, he held executive positions at Eagle Pharmaceuticals, Avanir Pharmaceuticals, BioDelivery Sciences International (BDSI) and UCB BioSciences, Inc. Dr. Hepner received an M.D. and Ph.D. in Psychiatry and Neurology from the University of Buenos Aires. He also completed a post-doctoral fellowship in Neuro-Psychopharmacology at the University of Ottawa and additional training at Harvard University. Adrian’s drive and passion for drug development has led to 8 NDAs resulting in approved medicines.

The senior management team also includes Peter Collum, our Chief Financial and Business Officer, who joined Pharnext in July 2019 after a 17-year career in life sciences investment banking with both Bank of America and MTS Health Partners, as well as a 5-year career in the pharmaceutical industry with Roche as an engineer. Peter brings to our company a remarkable knowledge of financial markets and the pharmaceutical industry.

Together with the other talented members of management, we believe we now have a strong and complete team of executives and employees with expertise and invaluable experience to position the Company to meet our strategic objectives and ultimately develop therapies for patients in need.

Interaction with the U.S. Food and Drug Administration (“FDA”)

In June 2020, we had a productive meeting with the FDA to discuss the regulatory path forward for PXT3003 and appreciate the agency’s continued collaborative approach in our clinical advancement of PXT3003. Based on FDA guidance, Pharnext is preparing to conduct an additional pivotal Phase III trial for which the FDA has recommended that the primary endpoint be the Overall Neuropathy Limitations Scale (“ONLS”). This study will have two arms to compare high dose and placebo. I would like to point out that in our original Phase III study, the high dose patients showed encouraging responses using the same primary endpoint, ONLS. We expect to launch this trial before the end of Q1 2021. In the meantime, we are running an open label study for patients enrolled in the original Phase III trial.

We submitted a Special Protocol Assessment (SPA) to the FDA on September 15 in order to further define the new Phase III study protocol.

If successful, the data results from the upcoming Phase III trial will be used to support the New Drug Application (“NDA”) for PXT3003 in CMT1A.

Appointment of new directors to the Board

As part of our 2020 Annual General Meeting, the Company proposed a transformation of the Board consistent with the Company’s vision of developing and commercializing PXT3003 in the U.S. and Europe for CMT1A. The newly appointed directors have strong biopharmaceutical and business expertise focused on clinical development, neurology, strategy, transactions, value creation and governance. The newly appointed directors include:

- Alexandre Berda – Managing Director of CB Lux, Pharnext’s largest shareholder.
- Dr. Jean Combalbert – Founder and CEO of Epics Therapeutics SA, Chairman of the Board of Syndesi Therapeutics SA.
- Dr. Elisabeth Svanberg – Chief Development Officer at Ixaltis SA, Board-certified general surgeon and Associate Professor of Surgery.
- Joshua Schafer – Chief Strategy and Business Development Officer of Mallinckrodt Pharmaceuticals and Board member of Shuttle Pharmaceuticals.
- Dr. David Horn Solomon – Chief Executive Officer of Pharnext SA, Chairman of the Board for Advicenne Pharma (PARIS: ADV) and Rexgenero in London.
- Dr. Lawrence Steinman – Zimmerman Professor of Neurology and Neurological Sciences, Pediatrics and Genetics at Stanford University.

In addition to the newly appointed directors, the Pharnext Board of Directors also includes Michel de Rosen (Chairman), Pierre Bastid, Kenneth Lee and Dr. Philippe Pouletty. Full biographies of Board Members can be found on Pharnext’s website at <https://pharnext.com/about>. We truly believe we have the right Board in place to oversee our value creation opportunity and effectively execute under their expertise.

Entered a Research Collaboration with Charcot–Marie–Tooth Association

In September, we signed a research collaboration with the Charcot–Marie–Tooth Association (CMTA), a United States patient advocacy group, to investigate novel biomarkers associated with CMT1A.

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. 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. We are very excited about this

collaboration and look forward to conducting research that will potentially bring valuable insights for further development of PXT3003.

The Future for Pharnext

2020 has been a very productive and event-driven year for Pharnext. Our plan is to build on this momentum by advancing drug candidates from our PLEOTHERAPY™ pipeline through clinical development and, subject to regulatory approval, ultimately bring them to the market to fulfil the significant unmet medical need in our target patient population. Pharnext estimates that the funding requirements to complete our Phase III trial in CMT1A are approximately €70 million. Pharnext intends to fund its internal clinical development efforts through one or more equity raises, anticipated to be targeted in whole or in part at US and European institutional life science investors, at times and on terms that will be subject to prevailing market conditions. Pharnext expects to also benefit from the participation of existing shareholders in these equity raises further evidencing their support of the Company's strategy and future development path.

With Best Regards,

David Horn Solomon
Chief Executive Officer

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 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™. 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 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 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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