

Pharnext provides regulatory and clinical update on PXT3003 Phase III study for the treatment of Charcot-Marie-Tooth Type 1A

US Food and Drug Administration has agreed with Pharnext and provided clear guidance on the regulatory pathway to approval for PXT3003, including key design elements of a single pivotal Phase III study

PARIS, France, 7:00 a.m., June 10, 2020 (CET) – Pharnext SA (FR0011191287 - ALPHA), a biopharmaceutical company pioneering new approaches to developing innovative drug combinations based on big genomics data and artificial intelligence, today provided an update on the regulatory and clinical status of PXT3003, its lead program in Charcot-Marie-Tooth Type 1A (“CMT1A”).

Regulatory Update

PXT3003 is a novel drug candidate for the treatment of CMT1A and has been granted both Orphan Drug Designation and Fast Track Designation by the US Food and Drug Administration (“FDA”). In Pharnext’s previous interactions with FDA, the agency provided guidance that an additional Phase III study would be required. Based on our most recent interactions with FDA through a Type C meeting, FDA has now provided a very clear path to NDA submission for approval of our lead clinical program.

The FDA has agreed with the key elements of Pharnext’s approach for the developmental pathway to approval for PXT3003. Specifically, the FDA has indicated that a single pivotal Phase III study in CMT1A delivering robust results could be sufficient for approval of PXT3003. This study design will be similar to the earlier Phase III study of PXT3003 that yielded encouraging top line results in October 2018. The FDA has agreed that the primary endpoint will again be Overall Neuropathy Limitations Scale (ONLS). Notably, the FDA has also agreed that the factorial study requirement for combination drugs can be carried out in a preclinical CMT1A disease animal model, and not in a human Phase III clinical trial as typically required. The animal factorial study, a requirement for NDA filing, will be done under GLP or GLP-like conditions and will have a similar study design and use the same CMT1A model as our previously successful preclinical factorial study.

Phase III Trial Design Update

For the upcoming Phase III pivotal study, as recommended by FDA, Pharnext will use ONLS as the primary endpoint, as was used in the previous Phase III study. We will have two arms in the study which will compare the high dose vs placebo. The high dose showed encouraging results in the earlier Phase III study. In addition, we have resolved the past manufacturing issue with our high dose oral solution and will now use the earlier successful low dose oral solution formulation in a higher volume to deliver the high dose in our upcoming Phase III trial. We will also be utilizing a more convenient new unit dose package format, stick packs (liquid sachets), that will assure optimal patient compliance and more accurate dosing.

Pharnext plans to initiate the final pivotal Phase III clinical study before the end of Q1 2021.

Dr David Horn Solomon, Chief Executive Officer commented, “*We are grateful that the FDA has provided strong and specific guidance to complete pivotal studies towards NDA submission and approval for PXT3003 in CMT1A. Both the Phase III clinical trial as well as the preclinical animal study will be informed in their design by the earlier Phase III clinical trial that provided encouraging top-line results, and the earlier animal study that was successful, respectively. We have addressed earlier manufacturing issues and look forward to*

initiating the Phase III clinical trial before the end Q1 2021. Our goal at Pharnext is to provide CMT1A patients and their caregivers a new therapeutic to treat this disease where no therapy currently exists.”

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

Charcot-Marie-Tooth (CMT) disease encompasses a heterogeneous group of inherited, progressive, chronic peripheral neuropathies. CMT type 1A (CMT1A), the most common type of CMT, is an orphan disease affecting more than 100,000 people in the U.S. and E.U. The genetic mutation responsible for CMT1A is a duplication of the PMP22 gene coding for a peripheral myelin protein. Overexpression of this gene causes degradation of the neuronal sheath (myelin) and nerve dysfunction. As a result, patients have a significantly altered quality of life, suffering from progressive muscle atrophy of the limbs causing problems with walking, running balance and hand function. They may have loss of sensation, pain and cramps, and at least 5% need wheelchairs. First symptoms usually appear during childhood and progressively evolve throughout patients' lives. 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.

About PXT3003

Pharnext's first-in-class PLEODRUG™ PXT3003, developed using Pharnext's R&D platform, PLEOTHERAPY™, is a novel oral fixed-dose combination of baclofen, naltrexone and sorbitol, with Orphan Drug Designation in the U.S. and E.U. PXT3003, Pharnext's lead PLEODRUG™, has shown positive results both in non-clinical pharmacology and clinical studies for the treatment of CMT1A. In non-clinical pharmacology studies, PXT3003 inhibited the overexpression of the PMP22 gene, improved myelination of peripheral nerves and motor / sensory impairments. In a Phase II clinical study in 80 adult patients with CMT1A, PXT3003 was safe and well tolerated. In addition, PXT3003 showed trends in multiple efficacy endpoints beyond stabilization, particularly the ONLS scale. These results were published in the Orphanet Journal of Rare Diseases (OJRD) in December 2014. In October 2018, PXT3003 completed an international Phase III trial in 323 patients 16 years and older for the treatment of CMT1A, confirming the excellent safety profile of the combination and demonstrating an encouraging efficacy profile. The Phase III extension study is currently ongoing. An additional international pivotal Phase III trial is planned to be initiated in the first quarter of 2021.

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 3 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 2 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).

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-

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