Press release

Pharnext Announces Findings Evaluating Charcot-Marie-Tooth Neuropathy Score

The CMTNS remains an appropriate measure of impairment, although with some limitations

PARIS, February 2nd, 2017 at 8:00am (CET) – Pharnext SA (FR00111911287 - ALPHA), a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, announced today publication in *PLoS One* of statistical findings evaluating the clinical scale Charcot-Marie-Tooth Neuropathy Score (CMTNS) for application in Charcot-Marie-Tooth Disease Type 1A (CMT1A).

CMTNS is the first clinical scale dedicated to quantify impairment and measure disease progression in CMT patients. It has been used as the primary endpoint in most completed clinical trials for CMT1A to date. However, its ability to measure responses to treatment has not yet been demonstrated.

In the article titled: "*A Rasch Analysis of the Charcot-Marie-Tooth Neuropathy Score (CMTNS) in a Cohort of Charcot-Marie-Tooth Type 1A Patients*" authors Wenjia Wang *et al*. report that CMTNS remains particularly useful for measuring disease severity in CMT1A. However, CMTNS appears less sensitive in assessing mild forms of the disease. Therefore, the authors recommend further refinement of the scale to better assess therapeutic efficacy in all forms of CMT1A: mild, moderate and severe.

Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext, said "*One of the major challenges of CMT1A trial design is selecting a clinically meaningful endpoint. The findings confirm the limitations of the CMTNS. They support Pharnext's decision, endorsed by the European and American regulatory agencies, not to use CMTNS as the primary efficacy endpoint in our pivotal PLEO-CMT trial for patients suffering from mild to moderate forms of CMT1A, and to use the Overall Neuropathy Limitations Scale (ONLS) instead. The ONLS has been used as the primary endpoint to assess treatment efficacy in other peripheral neuropathies pivotal trials.*"

The publication can be found here: http://journals.plos.org/plosone/article?id=10.1371/journal.pone.0169878

About 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 at least 125,000 people in Europe and the U.S. 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) responsible for nerve dysfunction, followed by loss of nerve conduction. As a result of peripheral nerve degradation, patients suffer from progressive muscle atrophy of legs and arms causing walking, running, balance problems and abnormal hand functioning. CMT1A patients end up in wheelchairs in at least 5% of cases. They might also suffer from mild to moderate sensitive disorders. First symptoms usually appear during adolescence and will progressively evolve through patients' life.

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 PLEO-CMT Trial

PLEO-CMT is a pivotal, multi-center, randomized, double blind, placebo-controlled, three-arm Phase 3 study that was initiated in December 2015 and has enrolled 323 patients with mild to moderate CMT1A in 30 sites across Europe, the U.S. and Canada. Diagnosis of CMT1A has been confirmed genetically through detection of PMP22 gene duplication. Over 15 months, Pharnext will compare in parallel groups the efficacy and safety of two orally administered doses of PXT3003 to placebo. Efficacy will be assessed through one primary endpoint: change in the ONLS score at 12 and 15 months of treatment to measure improvement of patients' disability with PXT3003. Additional secondary outcome measures will be assessed including functional and electrophysiological endpoints. A nine month follow-up study is planned thereafter, where all patients who will have completed the first 15 months, will receive the active PXT3003 dose.

For more information about the PLEO-CMT clinical trial, please visit the following website:
U.S. NIH ClinicalTrials.gov website at: https://clinicaltrials.gov/ct2/show/study/NCT02579759

About Pharnext

Pharnext is an advanced clinical stage biopharmaceutical company founded by renowned scientists and entrepreneurs including Professor Daniel Cohen, a pioneer in modern genomics. Pharnext focuses on neurodegenerative diseases and has two lead products in clinical development: PXT3003 is currently in an international Phase 3 trial 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 positive Phase 2 results in Alzheimer's disease. Pharnext is the pioneer of a new drug discovery paradigm: PLEOTHERAPY©. The company identifies and develops synergic combinations of repositioned drugs at low dose. These PLEODRUG© offer several key advantages: efficacy, safety, and intellectual property including several composition of matter patents already granted. The Company is supported by a world-class scientific team.

The company Pharnext is listed on Euronext Alternext Stock Exchange in Paris (ISIN code: FR00111911287).

For more information, visit www.pharnext.com

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