Press Release

Findings From 'Real-World' Digital Lifestyle Study, CMT&Me, on Symptom Burden of Charcot-Marie-Tooth Disease Type 1A Published in the Journal of Clinical Neuromuscular Disease

PARIS, France, September 14, 2022, 8:30am CET – Pharnext SA (FR0011191287 – ALPHA) (the "**Company**"), an advanced late-clinical stage biopharmaceutical company developing novel therapeutics for neurodegenerative diseases with high unmet medical need, today announces the publication of symptom burden findings of Charcot-Marie-Tooth Disease Type1A ("CMT1A") from the 'Real-World' Digital Lifestyle Study, CMT&Me, in the Journal of Clinical Neuromuscular Disease ("JCNMD").

CMT&Me is a real-world, observational, non-interventional, digital lifestyle study launched in October 2018 for a duration of 5 years, both in Europe and the US, where self-reported data from patients with CMT, all subtypes including CMT1A, are collected on a regular basis. The objective of the study is to better understand the impact of the disease on patients' daily lives and help them manage their condition and treatment, as well as raise awareness and assess the value of potential new treatments. This study is managed by the company Vitaccess in collaboration with patient advocacy groups and key opinion leaders in the field, with the support of Pharnext.

The findings on patients with the 1A subtype of CMT (CMT1A) showed that patient-reported symptoms burden is high, with study participants' registering difficulties using limbs, fatigue, pain, and impaired quality of life. Burden severity appears to differ across the population, possibly driven by differences in rehabilitative and prescription-based interventions, and country-specific health care variability.

Symptoms ranked with highest importance were weakness in the extremities, difficulty in walking, and fatigue. Almost half of study participants experienced a worsening of symptom severity following diagnosis. Anxiety and depression were each reported by over one-third of participants in the study and use of rehabilitative interventions, medications, and orthotics or walking aids was high.

Florian P. Thomas, MD, PhD, Founding Chair & Professor, Department of Neurology, Hackensack University Medical Center & Hackensack Meridian School of Medicine (NJ, USA) and U.S. lead investigator of the PLEO-CMT trial, said: *"These first findings from the CMT&Me digital lifestyle help to better understand the consequences of this debilitating and progressive disease, for which there are currently no approved specific therapies, on patients' life. These data demonstrate the high unmet medical need in CMT1A caused by the symptoms' burden, are in line with previous natural history studies and provide more precise information for those following their disease progression, enabling more insightful conversations when meeting with their neurologists and caregivers. They also illustrate the need for education of patients and providers with the goal of empowering patients to advocate for their healthcare needs and for providers to be cognizant of the need for comprehensive interprofessional care over the entire disease course that connects patients to other professionals including rehab specialists, foot and ankle surgeons and health psychologists."*

The article, titled, "**Patient-Reported Symptom Burden of Charcot–Marie–Tooth Disease Type 1A: Findings From an Observational Digital Lifestyle Study**" can be accessed online here.

About the Digital Lifestyle Survey CMT&Me

Started in 2018 and conducted over a five-year period in the US and Europe, the CMT&Me digital lifestyle study enabled patients with Charcot-Marie-Tooth diseases to report via an app how their condition affects their quality of life, including

their day-to-day pain, mobility and ability to work. The study was managed by the company Vitaccess in collaboration with patient advocacy groups and key opinion leaders in the field, with the support of Pharnext.
More information about the CMT&Me study on https://clinicaltrials.gov/ct2/show/NCT03782883

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 Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for 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. Both of Pharnext's lead assets originated from the Pleotherapy R&D approach. More information can be found at www.pharnext.com.
Pharnext is listed on the Euronext Growth Stock Exchange in Paris (ISIN code: FR0011191287).

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