Press Release

Findings on Symptom Burden of Charcot-Marie-Tooth Disease Type 1A From 'Real-World' Digital Lifestyle Study, CMT&Me, to be Presented at the American Association of Neuromuscular & Electrodiagnostic Medicine Annual Meeting

PARIS, France, September 15th, 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 that findings on the symptom burden of Charcot-Marie-Tooth Disease Type 1A (CMT1A) from the 'Real-World' Digital Lifestyle Study, CMT&Me, will be presented in three poster presentations at the upcoming American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) 2022. The conference is taking place from September 21-24, 2022, in-person and with an option to participate virtually, at Gaylord Opryland Resort & Conference Center in Nashville, Tennessee.

Dr Youcef Boutalbi, Medical Affairs Director at Pharnext, will present the three posters from CMT&Me, in sessions I, II and III on Thursday 22nd and Friday 23rd September. The Pharnext team will also be present throughout AANEM at booth #426.

Details on the CMT&Me poster presentations at AANEM 2022 (https://www.aanem.org/Meetings/Annual-Meeting) are as follows:

Title: Work impacts in patients with Charcot-Marie-Tooth disease type 1A (CMT1A): results from an international digital real-world evidence study
Poster number: 89
Poster sessions I & II: Thursday, September 22, 6:00 – 6:30pm & Friday, September 23, 9:30 – 10:00am

Title: Depression in patients with Charcot-Marie-Tooth disease type 1A (CMT1A): results from an international digital real-world evidence study
Poster number: 102
Poster sessions I & III: Thursday, September 22, 6:00 – 6:30pm & Friday, September 23, 3:30 – 4:00pm

Title: Patient-reported symptom burden of Charcot-Marie-Tooth disease Type 1A: results from an international digital real-world evidence study
Poster number: 128
Poster sessions I & III: Thursday, September 22, 6:00 – 6:30pm & Friday, September 23, 3:30 – 4:00pm

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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