Press Release

Findings on the impact of fatigue on patients living with Charcot-Marie-Tooth diseases from 'Real-World' digital lifestyle study, CMT&Me, to be presented in a webinar organized with CMT UK

PARIS, France, March 23, 2023, 08:30 am CET – Pharnext SCA (FR001400BV89 - 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 from the digital study CMT&Me on the impact of fatigue on patients with Charcot-Marie-Tooth Diseases (CMT) will be presented in a 45-minute webinar on Wednesday 29th March, 2023 at noon (UK time).

The international CMT&Me digital study helps better understand the impact of CMT on patients' daily lives. As of today, more than 3,133 patients worldwide are participating in this important study.

The UK CMT community contributing hugely to this important patient-led study, Vitaccess, in partnership with the patient advocacy group in the UK, CMT-UK, and supported by Pharnext, is hosting a 45-minute webinar on March 29th at noon (UK time) to share new findings in the UK from the CMT&Me study app.

Prof Mario Saporta, Associate Professor of Neurology & Human Genetics at the University of Miami, Miller School of Medicine, and Chair of the CMT&Me study scientific advisory board, will present these most recent findings specifically related to fatigue and discuss the impact of CMT on patients' daily lives. This presentation will be followed by a live survey which will open a Q&As session.

To attend this free webinar on the latest results from CMT&Me findings in the UK, you must register before the event with this LINK.

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 therapies for neurodegenerative diseases currently without satisfactory therapeutic solutions. Pharnext has a first-in-class drug candidate, PXT3003, in development for Charcot-Marie-Tooth disease type 1A (CMT1A), a rare, debilitating, inherited peripheral neuropathy. PXT3003 benefits from orphan drug status in Europe and the United States. In 2018, PXT3003 completed a Phase III clinical trial, the PLEO-CMT trial, with encouraging topline results. This trial was followed by an open-label extension study, the PLEO-CMT-FU trial, with 120 patients continuing treatment with PXT3003. Long-term data suggest a sustained benefit, safety, and efficacy, after 5 years of total trial time. An international pivotal Phase III study of PXT3003, the PREMIER trial, is currently ongoing with 387 CMT1A patients enrolled. PREMIER topline results are expected in Q4 2023. PXT3003 originated from the Pleotherapy™ R&D approach. Pharnext draws the attention of investors to the financial and other risk factors detailed in its financial reports. More information can be found at www.pharnext.com.
Pharnext is listed on the Euronext Growth market in Paris (ISIN code: FR001400BV89).

Contacts

Financial Press Relations
ACTUS finance & communication
Déborah Schwartz
dschwartz@actus.fr
+33 (0)1 53 67 36 35

Investor Relations
ACTUS finance & communication
Jérôme Fabreguettes Leib
pharnext@actus.fr
+33 (0)1 53 67 36 78