

Press release

Pharnext Confirms its Continuous Support of the Hereditary Neuropathy Foundation to Increase Global Awareness of Charcot-Marie-Tooth Disease

Paris, September 15, 2016 — Pharnext (ISIN FR00111911287), a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, today confirmed its continuous support of the Hereditary Neuropathy Foundation (HNF) at the occasion of the Charcot-Marie-Tooth (CMT) disease awareness month.

CMT, as do many orphan diseases, suffers from a clear lack of awareness amongst the general public, the medical and research communities and health authorities. Pharnext's support of HNF – initiated two years ago – is aimed at increasing awareness of this disease and strengthening the patient community to ensure: better and faster diagnosis of patients with CMT, optimal management of these patients and strong support of researchers and clinicians to ultimately find a cure for this debilitating group of inherited peripheral neuropathies.

Throughout the month of September, to celebrate CMT Awareness Month, HNF is promoting key events and projects (http://www.hnf-cure.org/september-awareness-2016) including:

- The Global Registry for Inherited Neuropathies (GRIN) co-founded by HNF and Hannah's Hope Fund (HHF) for Giant Axonal Neuropathy (GAN) (www.neuropathyreg.org).
- The online CMT patient community on the INSPIRE platform (https://www.inspire.com/groups/charcot-marie-tooth-cmt).
- The neurotoxic fact card to alert CMT patients about medications that may worsen their neuropathy to be shared with Health Care Providers (http://weblink.donorperfect.com/neurotoxicmeds).
- The CMT Moon Run Fundraiser (http://hnf.donorpages.com/MoonRun/).
- The CMT-Connect program: an initiative providing educational workshops to local communities across the USA (http://www.hnf-cure.org/cmt-connect).

"We are grateful to Pharnext for its continued support over the past two years. During CMT Awareness Month in September, we have an opportunity to draw public attention to this disease. We hope that our joint effort with Pharnext will ultimately provide people with CMT and other rare neuropathies with resources that will prove useful, such as the Global Registry for Inherited Neuropathies (GRIN) or the recently launched CMT Connect program," said Allison Moore, Founder and Chief Executive Officer of the Hereditary Neuropathy Foundation (HNF).

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Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext added, "Pharnext is continuing to support HNF's programs because we are dedicated to improving the lives of people with CMT. HNF has been doing excellent work supporting patients as well as raising awareness about this debilitating disease. Supporting patient groups like HNF is critical to Pharnext's mission as we continue to advance the development of PXT3003, currently in an international Phase 3 trial, for the treatment of patients with CMT type 1A."

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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 PMP 22 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 sensory 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 Hereditary Neuropathy Foundation

Hereditary Neuropathy Foundation (HNF) is a US non-profit 501(c)3 organization whose mission is to increase awareness and accurate diagnosis of Charcot-Marie-Tooth disease (CMT) and related inherited neuropathies, support patients and families with critical information to improve quality of life, and support research. HNF has notably developed the Therapeutic Research in Accelerated Discovery (TRIAD) program, a collaborative effort with academia, government, and industry to support and develop treatments for CMT.

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For further information, visit www.hnf-cure.org

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 and 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 "pleodrugs" 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.

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

For more information, please visit <u>www.pharnext.com</u>