Press Release

Vitaccess and Pharnext
Launch International CMT&Me Observational Study

Oxford (UK), Paris (France), 7:30 am, October 09, 2018 – Digital health company **Vitaccess** and **Pharnext SA, (FR0011191287 – ALPHA)** a biopharmaceutical company pioneering a new approach to the development of innovative drug combinations based on big genomic data and artificial intelligence, today announced the launch of an international study on Charcot Marie Tooth disease (CMT) using a bespoke digital app named CMT&Me.

CMT&Me is an observational, non-interventional study, sponsored by Pharnext, to collect Real-World Evidence (RWE) from people living with CMT. The objective of the study is to better understand the impact of the disease on patients' daily lives: the burden of CMT, its natural history and treatment, and medical, social and pharmacoeconomic effects.

The app launches this week in the U.S. and will expand in a matter of weeks to Germany, France, Italy, Spain and the U.K. It has been developed in collaboration with international CMT experts and Patient Advocacy Organisations (PAOs): HNF (Hereditary Neuropathy Foundation) and CMTA (Charcot Marie Tooth Association) in the U.S.; Federación ASEM (Federación Española de Enfermedades Neuromusculares) in Spain; ACMT-Rete in Italy; CMT U.K.; CMT France; and the CMT European Federation. The data collected will be valuable to researchers, scientific societies, PAOs and Pharnext. Access to the data is subject to approval by the study's Scientific Advisory Board (SAB), composed of international CMT experts and representatives of the PAOs mentioned above.

Using the MyRealWorld™ RWE digital platform developed by Vitaccess, the study will provide an extensive overview of what it is like to live with CMT. Using "Bring Your Own Device" (BYOD) technology, participants will be able to use the CMT&Me app on their smartphones (Android or iOS) in the comfort of their own home or on-the-go, and data will be collected in real-time. They can fill out background and quality of life questionnaires, record their symptoms in a diary and access a knowledge feature that contains medical and practical information about the condition. Data collected are sent to a central database and aggregated. Researchers and PAOs can explore the anonymised, aggregated data via dashboards in order to further knowledge of CMT and improve patient care. Tailoring of the digital platform and supporting materials has been informed by close collaboration with PAOs and patients.

From October 10 to 13, the Pharnext and Vitaccess teams will attend the **2018 American Association of Neuromuscular & Electrodiagnostic Medicine Annual Meeting** in Washington, D.C. to showcase the project. Join the chat session organised by HNF on Friday, October 12 from 08:00 to 09:30 Eastern Time, where Mark Larkin (Vitaccess Founder and CEO) will present the CMT&Me app.

For more information about the study and the CMT&Me app, please visit https://vitaccess.com/cmt-and-me/ or contact us at cmt@vitaccess.com.

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 US. 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 and 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 sensitivity disorders. First symptoms usually appear during adolescence and will progressively evolve through patients' lives. 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 Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for orphan and common neurodegenerative diseases that currently lack curative and/or disease-modifying treatments. Pharnext 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 US. The results of this trial are expected in the second half of 2018. PXT864 has generated positive Phase 2 results in Alzheimer's disease. Pharnext has developed a new drug discovery paradigm based on big genomic data and artificial intelligence: PLEOTHERAPY™. The Company identifies and develops synergic combinations of drugs called PLEODRUG™, offering several key advantages: efficacy, safety and robust intellectual property. The Company was founded by renowned scientists and entrepreneurs including Professor Daniel Cohen, a pioneer in modern genomics and is supported by a world-class scientific team.

Pharnext is listed on Euronext Growth Stock Exchange in Paris (ISIN code: FR0011191287).
For more information, visit www.pharnext.com

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About Vitaccess

Vitaccess is a digital health company based in Oxford, UK. Our experience in Health Economics and Outcomes Research (HEOR) and Market Access consultancy makes us a trusted partner to the pharmaceutical industry. We understand the complexities of governance – including GDPR and HIPAA - and these standards have been applied to the creation of our digital platform, MyRealWorld™ where we capture real-world evidence in real-time.

MyRealWorld™: the patients' voice, digitally

Our powerful real-world evidence platform quantifies patients' experience of illnesses and treatments in everyday life, which can be very different from the impact measured in clinical trials. We are producing smartphone apps for which we can adapt data collection.

HEOR and Market Access, optimised
We develop effective and pragmatic recommendations that optimize patient access and health technology assessment (HTAs). Our approach is grounded in detailed and up-to-the-minute understanding of the global P&R landscape, supported by analytical rigour and insight.

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