

CMT Research Foundation Invests in Vanderbilt University School of Medicine to Advance Research for CMT1A

Third Major Investment by CMTRF in First Quarter

ATLANTA, USA, March 13, 2023 /EINPresswire.com/ -- The [CMT Research Foundation](#) (CMTRF), a non-profit focused solely on delivering treatments and cures for [Charcot-Marie-Tooth disease](#)^{*}, has invested in a project at Vanderbilt University School of Medicine Basic Sciences that seeks to treat the problem of overproduction of PMP22, the primary genetic cause of CMT in patients with CMT1A. This project, to be run by Charles Sanders, Ph.D., Vice Dean and Professor of Biochemistry and Medicine, aims to restore PMP22 to healthy functioning levels.



“Vanderbilt has world-class expertise and capabilities to make breakthrough discoveries toward the development of novel therapeutics,” says Cleary Simpson, CEO of the CMTRF. “Recently, Dr. Sanders and his team developed new cell-based approaches to target the PMP22 protein and are now ready to direct these approaches to enable drug discovery. If this effort is successful, the project will be well positioned for further preclinical evaluation in mouse models.”

Dr. Sanders will be joined by Bruce Carter Ph.D., Professor of Biochemistry at Vanderbilt and member of the CMTRF Scientific Advisory Board, as co-principal investigator. Aspects of their research are supported by Ancora Innovation, a company wholly owned by affiliates of Deerfield Management that supports Vanderbilt’s life science research and leverages Deerfield’s expertise in accelerating drug development.

“Currently, there are no effective treatments or cures for people with CMT1A. Fortunately, the primary target in CMT1A is known – excess PMP22,” says Dr. Sanders “This is a multi-step project using multiple approaches to discover agents that restore PMP22 to healthy functioning levels. This raises the possibility that CMT1A could be treated by improving PMP22 trafficking within Schwann cells without changing the amount of gene or protein expression.”

[The Vanderbilt University School of Medicine Basic Sciences](#) is a leader in biomedical research and in training the next generation of scientists to improve health and cure disease. The School bridges the gap between foundational scientific discovery and the patient bedside using cutting-

edge technology to enable breakthroughs at the molecular level that pave the way for innovations bringing critical therapies to those who need them.

CMT Research Foundation (CMTRF) is a patient-led, non-profit focused on delivering treatments and cures for CMT. The foundation identifies significant obstacles or deficiencies impeding progress towards a cure and seeks out collaborators to address these issues. It's their mission to raise funds to invest in promising science with high potential of leading to treatments and cures. Founded by two patients who are driven to expedite drug delivery to people who live with CMT, the 501(c)(3) federal tax-exempt organization is supported by personal and corporate financial gifts.

*Charcot-Marie-Tooth encompasses a group of inherited, chronic peripheral neuropathies that result in nerve degradation. CMT patients suffer from progressive muscle atrophy of legs and arms, causing walking, running and balance problems as well as abnormal functioning of hands and feet. CMT affects one in 2,500 people (about the same prevalence as cystic fibrosis), including 150,000 Americans and nearly 3 million people worldwide. At the moment, there is no treatment or cure for CMT.

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