

CMT Research Foundation Launches Research Advisory Committee of Renowned Scientific Experts

ATLANTA, GA, USA, October 18, 2023 /EINPresswire.com/ -- The CMT Research Foundation (CMTRF), a patient-led, non-profit focused on delivering treatments and cures for Charcot-Marie-Tooth (CMT) disease*, today announced the formation of a Research Advisory Committee (RAC) consisting of: Dr. William Motley (Rapport Therapeutics), Dr. Paul August (ReviR Therapeutics), Dr. Arthur Suckow, former Co-founder and Chief Executive Officer of DTx



Pharma, and Dr. Charlotte Sumner (Johns Hopkins). Collectively, the RAC members represent decades of insights, experience, and accomplishments within the therapeutic and drug development space for CMT and other neurological diseases.



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Edritz Javelosa, CSO, CMTRF

Working alongside CMTRF Chief Scientific Officer Edritz Javelosa, the RAC will be tasked with maintaining and enhancing scientific strategies that support the Foundation's singular mission to find treatments and cures for CMT. They will work closely with the Scientific Advisory Board to fine-tune project selection and prioritization in order to fund the types of research that have the greatest potential for impact. Moreover, they will provide scientific guidance to the Board of Directors.

"The RAC's profound expertise will offer invaluable insights into the existing gaps and challenges within the field. Furthermore, their guidance will play a pivotal role in developing targeted strategies that align with our Foundation's mission." says Dr. Javelosa. "This partnership marks a significant leap forward in advancing our mission and driving meaningful progress in CMT research."

"The acquisition of DTx by Novartis has accelerated not only interest in CMT drug development but the number of research projects coming to the Foundation. This, together with remarkable advancements in gene therapy, drug delivery, novel technologies and new modalities has created a complex landscape of funding possibilities," says Cleary Simpson, CEO of the CMTRF.

"To stay true to our mission, we will look to the RAC to ensure that CMTRF is focused on the right projects, the right metrics and the right priorities that will result in cures for all types of CMT."

Dr. Paul August Chief Scientific Officer ReviR Therapeutics

Dr. August, who serves on the CMTRF Scientific Advisory Board, is a scientific and business leader with extensive drug discovery experience within multiple therapeutic areas. He played a pivotal role in the advancement of the DTX CMT1A program through his advocacy efforts at the CMTRF. With an 18-year tenure at Sanofi, he demonstrated exceptional leadership of the Early to Candidate Unit and pioneered innovative rare disease research programs. He is a Co-founder of AcuraStem, a leader in the development of therapeutics for neurodegenerative diseases such as ALS, FTD and CMT. In his prior role at Agios Pharmaceuticals, he led the Genetically Defined Disease preclinical research efforts. Currently, Dr. August's expertise is focused on Neuroscience and Oncology at ReviR Therapeutics. Primarily he is focused on the development of therapeutics to treat and prevent Huntington's Disease.

Dr. William Motley Senior Director of Clinical Development and Program Lead Rapport Therapeutics

Dr. Will Motley has worked as a consultant to the company's founding team at Boston-based Third Rock Ventures. Previously he served as Director of Translational Medicine for Flare Therapeutics and was a Senior Associate at Third Rock Ventures where he was a member of Flare's founding team and worked on diverse aspects of company creation. Dr. Motley also serves as a member of the Medical and Scientific Advisory Board of Palvella Therapeutics, a company focused on bringing therapies to patients with rare dermatologic diseases with no approved therapies. He is a member of CMTRF's Board of Directors.

Arthur Suckow former Co-founder and Chief Executive Officer of DTx Pharma

DTx Pharma was co-founded in 2017 by Dr. Suckow, John Grundy and Adam Pavlicek, and developed a platform for RNA therapeutics called Fatty Acid Ligand Conjugated Oligonucleotide. DTx was recently acquired by Novartis for \$1 billion. Dr. Suckow now plans to work independently with investors to build new companies tackling diseases with significant unmet need. Prior to DTx, Dr. Suckow held positions at Janssen (J&J), MedImmune/AstraZeneca and Regulus Therapeutics, leading projects across multiple therapeutic areas and modalities (small molecules, peptides, antibody conjugates and oligonucleotide therapeutics). He received his Honors B.Sc. in Biological Sciences from University of Delaware and his Ph.D. in Biomedical

Sciences from the University of California, San Diego. During graduate school, he founded Synervate–a cell therapy company focused on a treatment for Alzheimer's Disease.

Dr. Charlotte Sumner
Professor of Neurology and Neuroscience
Johns Hopkins University School of Medicine

Dr. Sumner co-directs the Johns Hopkins Muscular Dystrophy Association Care Center, the Spinal Muscular Atrophy, and the Charcot-Marie-Tooth clinics, which deliver multi-disciplinary clinical care, engage in international natural history studies, and provide cutting-edge therapeutics. Her practice is notable for its focus on individuals with inherited neuromuscular disorders of peripheral nerves and motor neurons, including spinal muscular atrophy and CMT. Her research focuses on developing treatments for degenerative disorders of motor neurons and peripheral nerves. Notably, Dr. Sumner was a previous member of the SAB, and her recommendations played a pivotal role in steering the foundation toward addressing delivery challenges.

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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