

CMTRF 2024 Global CMT Research Convention to Gather Scientific Experts and Patients to Discuss Treatments/Cures for CMT

Renowned Scientific Experts to Discuss the Status of Treatments and Cures for Charcot-Marie-Tooth (CMT) Disease

ATLANTA, GA, USA, July 18, 2024 /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 most of the speakers at its upcoming 2024 [Global CMT Research Convention](#) to be held September 26-28, in Cambridge, MASS.



Thursday, September 26th CMTRF will host a [Young Researcher Innovation Forum](#) for CMT Research. Designed to inspire young scientists to explore innovative ideas in Charcot-Marie-Tooth disease drug development, the grant program will provide project-based funding to young researchers with novel ideas that advance CMT research. For more information please visit: [Young Researcher Innovation Forum](#).

Friday, September 27th is for scientists, industry and investors to convene and discuss pathways and challenges in drug development for CMT. The list of speakers is headlined by:

- Carsten Bönnemann, MD, Senior Investigator at the National Institute of Neurological Disorders and Stroke, whose Keynote address will discuss on the development of a gene therapy for giant axonal neuropathy and the learnings that can be applied to CMT drug development.
- Peter Marks, MD, PhD, Director of the Center for Biologics Evaluation and Research at the Federal Drug Administration (FDA) will discuss the role of the FDA in the future of rare disease drug approvals.
- Scott Harper, PhD, Principal Investigator at Nationwide Children's Hospital and Chief Scientific Advisor at Armatus Bio will discuss the results of intrathecal administration of a gene therapy for CMT1A.
- Rob Burgess, PhD, Professor at the Jackson Laboratory will provide an overview of CMT's biology to identify therapeutic targets.
- Kleopas Kleopa, MD, PhD, FAAN, FEAN, Head of the Neuroscience Department and Acting Head of the Neuropathology Department at The Cyprus Institute of Neurology and Genetics will provide an overview of the progress and challenges of gene therapies for CMT.

□ David Goldstein, PhD, CEO at Actio Biosciences will discuss TRPV4 inhibitors as a therapeutic for CMT2C.

□ Frederik Rombouts, PhD, Vice President, Drug Discovery at Augustine Therapeutics will speak about developing best-in-class HDAC6 inhibitors for the treatment of CMT,

Saturday, September 28th is a day for patients and their families- both in-person and virtually – to connect with one another and with clinicians and researchers to learn about recent progress in drug development and receive critical information on upcoming clinical trials. It is a great opportunity to talk with other patients about the power of the community's participation in driving CMT research forward at the fastest pace in history.

You can find more information here: [Global CMT Research Convention](#)

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 toward a cure and seeks out collaborators to address these issues. To date, CMTRF has funded 24 projects, of which 8 are completed. Of those 8 completed projects, 5 have clinical candidates. CMTRF's mission to invest in promising science with high potential of leading to treatments and cures was proven effective and ground-breaking when DTx Pharma with a CMTRF- backed program as its lead candidate was acquired by Novartis for \$1 billion. 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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