

CMT Research Foundation To Host First Global Research Convention

Several Hundred Charcot-Marie-Tooth* World Leaders Gather to Accelerate Collaboration and Advancements in Research; Pharma Partner DTx to Review Significant Progress in Drug Delivery



Georgia, Atlanta, Jun 15, 2021 ([Issuewire.com](https://www.issuewire.com)) - The CMT Research Foundation ([CMTRF](https://www.cmtrf.org)), a non-profit focused solely on delivering treatments and cures for Charcot-Marie-Tooth disease, will host the first Global CMT Research Convention on September 24-25, 2021 in Alexandria, VA (and online), it was announced today by Susan Ruediger, CMTRF's co-Founder, and CEO.

"The CMT scientific community is growing rapidly. We know that real progress happens in environments that foster open collaboration and sharing of science," says Ms. Ruediger. "The CMT Research Convention is designed to facilitate greater sharing of ideas, identify areas of need and opportunity for high-impact collaborative research projects, and to update patients on our remarkable progress in just three years."

"The 2021 Global CMT Research Convention unites patients, researchers, pharmaceutical companies, and government agencies to streamline efforts, collaborate more effectively, and to accelerate strategies for CMT treatments," says Keith N. Fargo, PhD and CMTRF's Chief Scientific Officer. "We expect hundreds of researchers and pharma industry leaders both in-person and joining virtually from around the world, to work together to identify and solve key barriers to drug development for CMT."

Among the highlights will be keynote addresses by Arthur T. Suckow, PhD, Chief Executive Officer of DTx Pharma, Inc., and Dr. James M. Wilson, gene therapy innovator, and pioneer.

DTx is a privately-held biotechnology company creating novel RNA-based therapeutics to treat the genetic drivers of disease and has been a research partner to CMTRF since 2019. Significant CMT drug delivery progress has been made by DTx.

“The 2021 Global CMT Research Convention is yet another example of CMTRF’s leadership and innovation in the search for a cure for CMT,” says Arthur T. Suckow, PhD, CEO of DTx Pharma.

“The CMT Research Foundation’s early financial support of DTx, and the access it gives us to thought leaders and patients, make CMTRF an integral contributor to our program for CMT1A. I look forward to sharing our combined journey to date at the 2021 Global CMT Research Convention.”

James M. Wilson is a biomedical researcher and global leader in gene therapy. His research involves the development of virus-based gene therapies for genetic diseases including work on gene therapy for CMT2A. His laboratory’s translational research portfolio includes more than 30 orphan disease programs. In a career spanning four decades, Dr. Wilson has identified, isolated, and developed virus-based vectors for human gene therapy. His laboratory is responsible for discovering a new family of endogenous adeno-associated viruses that have become best-in-class for in vivo gene therapy vectors. Dr. Wilson is the Director of the Gene Therapy Program; Rose H. Weiss Professor and Director of the Orphan Disease Center; and Professor of Medicine and Pediatrics at the Perelman School of Medicine at the University of Pennsylvania.

Other notable speakers will include Zarife Sahenk and Kleopas Kleopa - renowned gene therapy researchers; Jeff Milbrandt and Ludo Van Den Bosch - experts in nerve health and Rob Burgess and Cat Lutz - masters in disease models. The speakers will represent a broad set of leading research institutions including the University of Miami, the Jackson Laboratory, and Institut National de la Sante et de la Recherche Medicale; senior leaders from the NIH; and global patient advocacy groups including the Muscular Dystrophy Association (MDA).

The Convention will also be streamed live online to enable global participation.

“Never before have academic researchers, industry partners, regulatory agencies, international patient groups, and patients united to attack key problems in drug development for CMT in this way,” says Dr. Fargo. “This is truly an extraordinary opportunity to network with the people most critical to finding a cure for the 3 million people around the world with CMT.”

The two-day convention starts with a full-day meeting for the research community on September 24 and on September 25th, patients will convene with researchers to understand the research landscape, its challenges, and how they can get involved to help move progress forward.

“The CMT Research Foundation has one single focus: deliver treatments and cures for CMT during our lifetime,” says Patrick Livney, Co-founder and Chairman of the CMT Research Foundation. “We fund translational research that leads to the development of treatments and cures for CMT. We are a patient-centered foundation - united in one cause - ENDING CMT. It is critical that patients be involved in the drug development process – from lending their voices to discovering important outcomes to participating in clinical trials. Patients are why we exist and are at the heart of everything we do.”

Among the sponsors helping to underwrite the event are [DTx](#), [Cellatoz](#), [IGT](#), and the [MDA](#).

A complete calendar of events at the first Global CMT Research Convention can be found [here](#).

[The CMT Research Foundation](#) (CMTRF) is focused solely on delivering treatments and cures for CMT. Founded by two patients who are driven to expedite drug delivery to people who live with CMT globally, the organization funds research for drug development. 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 and abnormal hand and foot functioning. At the moment, there is no treatment or cure for CMT.

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