

# CMT Research Foundation (USA) collaborates with Augustine on new therapy against Charcot-Marie-Tooth disease

LEUVEN, BELGIUM, March 2, 2023

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Therapeutics announces a collaboration with the Charcot-Marie-Tooth Research Foundation (CMTRF) in the USA. CMTRF is a US-based, patient-led, non-profit focused on delivering treatments for Charcot-Marie-Tooth

disease (CMT). With the financial support of CMTRF, Augustine Tx will investigate the efficacy and safety of their newly discovered class of selective HDAC6 inhibitors.



### Promising effect of novel HDAC6 inhibitors against CMT

Augustine Tx creates innovative medications that target the fundamental disease mechanisms of Charcot-Marie-Tooth - a category of rare, inherited chronic peripheral neuropathies causing progressive nerve and muscle deterioration. With almost three million individuals affected globally, there is currently no treatment approved for CMT. Augustine Tx's pipeline is based on small molecule inhibitors of HDAC6, a class II histone deacetylase. This is a well-known molecular target in neurodegenerative disorders and neuropathies. Augustine's third generation HDAC6 inhibitors do not exert known liabilities of earlier generations and therefore represent a very promising novel class of therapeutic molecules. Augustine Tx lead drug candidate AGT-216 has demonstrated significant potential in preclinical trials. In CMT1A C3 mice, modeling subtypes of the disease with the PMP22 underlying genetic mutation, the candidate has demonstrated HDAC6 target engagement and reversal of disease phenotypes. Effect in axonal damage biomarkers has been shown too, as well as halting disease progression after once-daily treatment for 4 weeks.

### CMTRF supports Augustine Tx in advancing lead drug candidate

Helped by the funding of CMTRF, Augustine Tx now aims to continue development of AGT-216. The objective is to gain an in-depth understanding of the compound by conducting pharmacological dose range finding, non-GLP toxicology and safety studies, as well as long-term efficacy tests in rodents. Conducting these studies is essential to advance the development of AGT-216 towards clinical studies in humans. Apart from its lead candidate, Augustine Tx will also continue assessments on other compound candidates identified using its drug discovery

platform.

Prof. dr. Ludo Van Den Bosch (VIB-KU Leuven), founding scientist at Augustine Tx and chairman of the Scientific Advisory Board says: “At Augustine Tx, we aim to deliver the first truly curative option for CMT patients. Following promising preclinical studies, we are on the right track to advance towards future clinical tests”.

Cleary Simpson, CEO of CMTRF says: “HDAC6 inhibitors are promising as potential therapeutics for several forms of CMT. With funding from the CMT Research Foundation, Augustine Tx will be able to continue research on their promising lead candidate tackling CMT1A disease and to take the necessary steps towards first-in-human trials. We are hopeful that this collaboration will accelerate the progress to finding a cure for CMT1A patients who currently have no treatment options available.”

Sylvain Celanire, CEO of Augustine Tx adds: “Our development progress will be greatly expedited thanks to the support provided by the CMT Research Foundation along with support by our historical investors as we move towards IND enabling studies”.

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#### About Augustine Therapeutics

Augustine Therapeutics is a Belgian biotech company, spin-off company of VIB/KU Leuven backed by seed investors V-Bio Ventures, PMV, AdBio Partners, VIB and Gemma Frisius Fund. The company focuses on the development of novel, innovative medicines for rare neurodegenerative disorders, tackling severe debilitating diseases with high unmet medical needs such as Charcot-Marie-Tooth disease, a serious and rare disease, as well as other peripheral neuropathies. The company is building a portfolio of first-in-class therapeutic modalities from the ground-breaking research of Prof. Ludo Van Den Bosch from the VIB/KU Leuven, Center for Brain and Disease Research. <https://augustinetx.com/>

#### About the 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.

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