Using gene therapy to treat chronic traumatic encephalopathy

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A new study shows the feasibility of using gene therapy to treat the progressive neurodegenerative disorder chronic traumatic encephalopathy (CTE). The study, which demonstrated the effectiveness of direct delivery of gene therapy into the brain of a mouse model of CTE, is published in *Human Gene Therapy*.

Ronald Crystal and colleagues from Weill Cornell Medical College, New York, NY, coauthored the article entitled "Anti-Phospho-Tau Gene Therapy for Chronic Traumatic Encephalopathy."

There is currently no treatment for CTE, which is caused by repeated trauma to the central nervous system (CNS), such as that suffered by soldiers, athletes in contact sports, and in accident-related trauma. Inflammation results in the accumulation of hyperphosphorylated forms of Tau protein (pTau). Crystal et al. developed an adeno-associated virus (AAV) vector to deliver an anti-pTau antibody to the (CNS). They showed that direct delivery of the AAVrh.10anti-pTau directly into the hippocampus of brain-injured mice was associated with a significant reduction in pTau levels across the CNS. They propose that doses could be scaled up and this strategy could be effective in humans as well.

"CTE is much more prevalent than was initially realized, and there is currently no therapy available," says Editor-in-Chief Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School, Worcester, MA. "This new work from the Crystal laboratory is potentially ground-breaking as a means to remove the offending Tau phoshoprotein."

More information: Chester Bittencourt Sacramento et al, Anti-Phospho-Tau Gene Therapy for Chronic Traumatic Encephalopathy, *Human Gene Therapy* (2019). DOI: 10.1089/hum.2019.174