

## Gene therapy offers hope for untreatable CTE brain disorder

[Researchers from Weill Cornell Medical College] published a new study ("[Anti-Phospho-Tau Gene Therapy for Chronic Traumatic Encephalopathy](#)") in Human Gene Therapy that shows the feasibility of using gene therapy to treat the progressive neurodegenerative disorder chronic traumatic encephalopathy (CTE). The study demonstrated the effectiveness of direct delivery of gene therapy into the brain of a mouse model of CTE.

"CTE is a progressive neurodegenerative disorder caused by repetitive trauma to the central nervous system (CNS) suffered by soldiers, contact sport athletes, and civilians following accident-related trauma. CTE is a CNS tauopathy, with trauma-induced inflammation leading to accumulation of hyperphosphorylated forms of the microtubule-binding protein Tau (pTau), resulting in neurofibrillary tangles and progressive loss of neurons. At present, there are no therapies to treat CTE. We hypothesized that direct CNS administration of an adeno-associated virus (AAV) vector coding for an anti-pTau antibody would generate sufficient levels of anti-pTau in the CNS to suppress pTau accumulation thus interrupting the pathogenic process," the investigators wrote.

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"Using doses that can be safely scaled to humans, the data demonstrate that CNS administration of AAVrh.10anti-pTau is effective, providing a new strategy to interrupt the CTE consequences of TBI."

**Read full, original post:** [Gene Therapy Shows Promise in the Treatment of Chronic Traumatic Encephalopathy](#)