# Prednisone may improve effectiveness of AAV-based gene therapy by reducing immune response

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A new study of gene transfer using adeno-associated virus (AAV)-based gene delivery into skeletal muscle of rhesus macaques showed that oral prednisone reduced immune responses to AAV that can weaken expression of the therapeutic transgene over time. Animals given prednisone before the gene therapy had a 60% decrease in immune cell infiltrates, mainly comprised of cytotoxic T cells, according to the study published in *Human Gene Therapy*, a peer-reviewed journal from Mary

Ann Liebert, Inc., publishers. The article is available free on the *Human Gene Therapy* website until July 9, 2017.

Megan Cramer, The Ohio State University, Paul Martin, The Research Institute at Nationwide Children's Hospital and The Ohio State University College of Medicine, Columbus, and coauthors also reported that AAV-treated muscles had higher levels of a biomarker called PD-L2, which can induce programmed T-cell death. The researchers report their findings in the article entitled "Induction of T-Cell Infiltration and Programmed Death Ligand 2 Expression by Adeno-Associated Virus in Rhesus Macaque Skeletal Muscle and Modulation by Prednisone."

"Prednisone is frequently used in conjunction with AAV gene therapy in the hope of blunting harmful immune responses to the AAV capsid. However, very little is known about the precise immune mechanisms involved in its use, or even if it is beneficial with various different routes of AAV administration," 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.

**More information:** Megan L. Cramer et al, Induction of T-Cell Infiltration and Programmed Death Ligand 2 Expression by Adeno-Associated Virus in Rhesus Macaque Skeletal Muscle and Modulation by Prednisone, *Human Gene Therapy* (2017). DOI: 10.1089/hum.2016.113

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