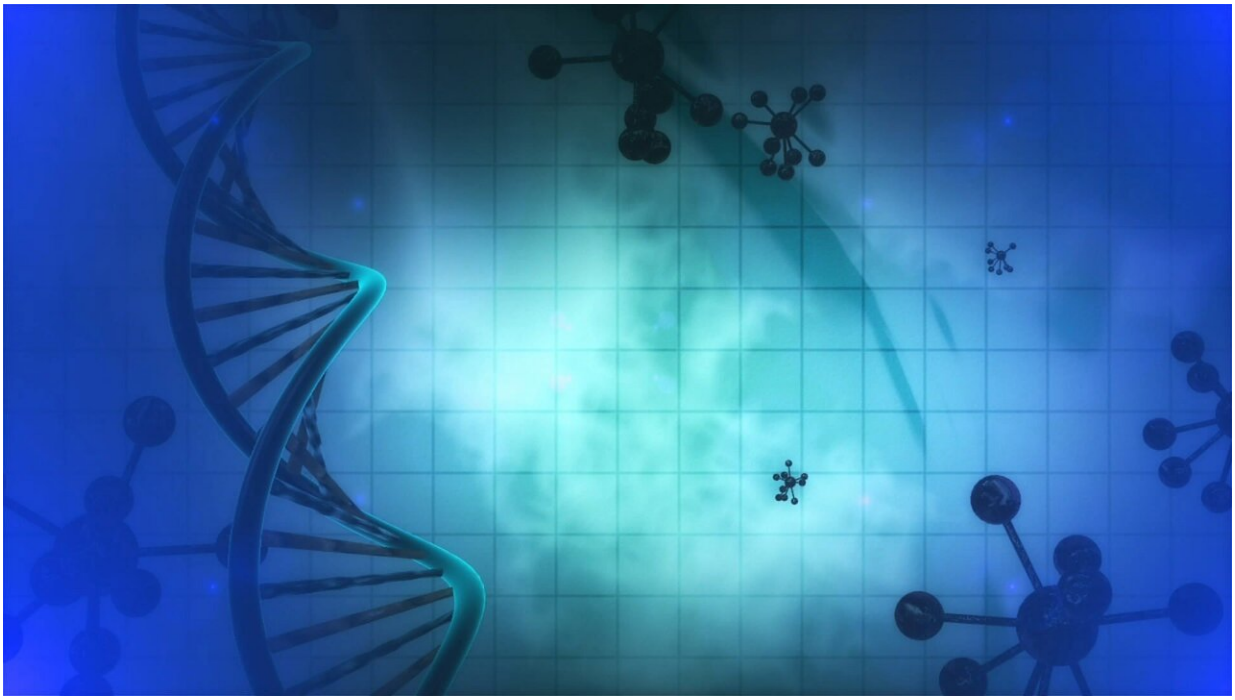
Engineered capsids for efficient gene delivery to the eye

August 14 2020



Credit: CC0 Public Domain

A rational design approach created novel variants of adeno-associated viral (AAV) capsids. These have improved transduction properties in the mouse retina and cornea. as reported in the peer-reviewed journal *Human Gene Therapy*.

The efficient gene delivery of these variants was confirmed in non-

human primate tissue. "The capsid modified AAV2 and AAV5 variants described here have novel attributes that will add to the efficacy and specificity of their potential use in gene therapy for a range of human ocular diseases," said Catherine O'Riordan and coauthors from Sanofi.

"The structural domains of the AAV capsid have become fundamental building blocks of many gene therapy vectors. The work by Dr. O'Riordan and her colleagues takes advantage of the new age of structural biology to intelligently redesign these building blocks rather than relying on empiric screening of variants. This rational design approach holds great promise for the field," according to Editor-in-Chief of Human Gene Therapy Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School.

More information: Amy Frederick et al. Engineered Capsids for Efficient Gene Delivery to the Retina and Cornea, *Human Gene Therapy* (2020). DOI: 10.1089/hum.2020.070

Provided by Mary Ann Liebert, Inc