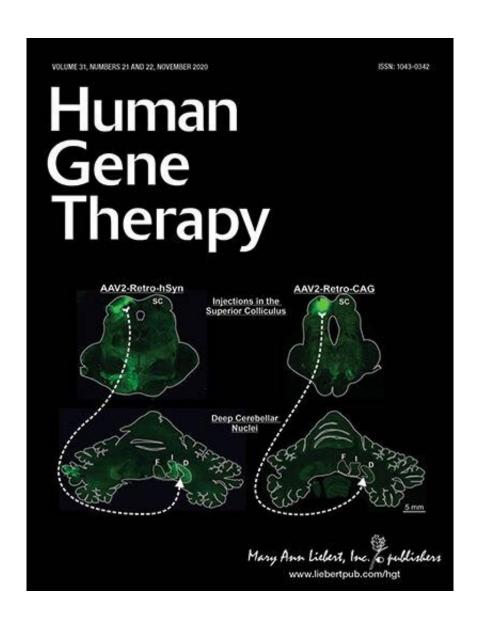


AAV capsid-promoter interactions in the nonhuman primate brain

December 11 2020



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The phenomenon of AAV capsid-promoter interaction recently seen in the rat central nervous system has now been shown to occur in the nonhuman primate brain. This interaction can directly determine cellspecific transgene expression, as described in *Human Gene Therapy*.

An adeno-associated virus (AAV) contains a single-stranded DNA genome encapsulated in a capsid comprised of three <u>structural proteins</u>.

"We document a unique attribute of AAV vectors in both rodent and primate models that until recently remained undescribed: namely capsid/promoter interactions, that dictated cell type transduction profiles regardless of virus permissivity," state R. Jude Samulski, University of North Carolina School of Medicine, and coauthors.

"Up until now, we have thought of the AAV capsid as a 'delivery truck,' and once it dropped off its payload at the right cellular address, the vector promoter would do the rest. This work refutes that concept, showing that the <u>capsid</u> continues to have an effect on <u>gene expression</u> within specific cells of the brain and spinal cord. This has <u>profound implications</u> for vector design in the future," 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.

Research reported in this publication was supported by the National Institutes of Health under Award Numbers NEI R21, EY030278, EY013692, NS082289. The content is solely the responsibility of the authors and does not necessarily represent the official views of the National Institutes of Health.



More information: Martin O. Bohlen et al, Adeno-Associated Virus Capsid-Promoter Interactions in the Brain Translate from Rat to the Nonhuman Primate, *Human Gene Therapy* (2020). DOI: 10.1089/hum.2020.196

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Citation: AAV capsid-promoter interactions in the non-human primate brain (2020, December 11) retrieved 23 April 2024 from https://medicalxpress.com/news/2020-12-aav-capsid-promoter-interactions-non-human-primate.html

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