



Nov 21,
2019

Systemic AAV vectors for widespread and targeted gene delivery in rodents [↗](#)

Rosemary C. Challis¹, Sripriya Ravindra Kumar¹, Ken Y. Chan¹, Collin Challis¹, Keith Beadle¹, Min J. Jang¹, Hyun Min Kim¹, Pradeep S. Rajendran², John D. Tompkins², Kalyanam Shivkumar², Benjamin E. Deverman¹, [Viviana Gradinaru](#)¹

¹Division of Biology and Biological Engineering, California Institute of Technology, Pasadena, CA, USA,

²Cardiac Arrhythmia Center and Neurocardiology Research Center of Excellence, University of California, Los Angeles, Los Angeles, CA, USA

1 Works for me dx.doi.org/10.17504/protocols.io.84ahyse

 [Viviana Gradinaru](#) 

ABSTRACT

We recently developed adeno-associated virus (AAV) capsids to facilitate efficient and noninvasive gene transfer to the central and peripheral nervous systems. However, a detailed protocol for generating and systemically delivering novel AAV variants was not previously available. In this protocol, we describe how to produce and intravenously administer AAVs to adult mice to specifically label and/or genetically manipulate cells in the nervous system and organs, including the heart. The procedure comprises three separate stages: AAV production, intravenous delivery, and evaluation of transgene expression. The protocol spans 8 d, excluding the time required to assess gene expression, and can be readily adopted by researchers with basic molecular biology, cell culture, and animal work experience. We provide guidelines for experimental design and choice of the capsid, cargo, and viral dose appropriate for the experimental aims. The procedures outlined here are adaptable to diverse biomedical applications, from anatomical and functional mapping to gene expression, silencing, and editing.

EXTERNAL LINK

<https://www.nature.com/articles/s41596-018-0097-3>


s41596-018-0097-3.pdf



This is an open access document distributed under the terms of the [Creative Commons Attribution License](#), which permits unrestricted use, distribution, and reproduction in any medium, provided the original author and source are credited