PHENOVISTA

READY-2-GO AAV GENE DELIVERY ASSAY SERVICE

BACKGROUND

Adeno-associated viruses (AAVs) are a promising vector for treating genetic diseases, with rapidly increasing numbers of FDA-approved therapies already on the market. The general goal of AAV therapy is to deliver genes to specific cell types to treat genetic diseases at their source. However, administration of AAVs has faced numerous challenges such as accumulating in untargeted organs (*e.g.*, liver and kidney) and difficulty traversing the blood-brain barrier. Assessing the tropism and preferential uptake of AAVs is key to overcoming these hurdles and developing safe, targeted, AAV-based, gene therapies.

KEY FEATURES

- Uses human, iPSC-derived cell types
- Comparative assessment of AAV transduction in various cell types
- Only 6-8 weeks from assay to report
- Ability to bundle R2G assay services or transition to more complex, bespoke assay services with the same service provider

THE CHALLENGE

Currently, AAV-based therapies require extremely high doses of AAV, as only a fraction of the vectors will be taken up by the cell type of interest. Optimizing the tropism of new AAVs can lower the effective dose and increase efficiency of treatment. Typically, assessments of AAV tropism are conducted in animal models or immortalized cell lines, which are not scalable and/or clinically translatable.

THE SOLUTION

Our Ready-2-Go AAV Gene Delivery Assay Service provides a panel of AAV serotypes packaged with a fluorescent reporter for you to assess your AAVs. This service will provide you with information about the cell-type specificity of your AAVs in human, iPSC-derived glutamatergic neurons, GABAnergic neurons, astrocytes, cardiomyocytes, hepatocytes, and/or retinal pigment epithelial cells (FUJIFILM CDI). The use of iPSC-derived cells offers the most scalable and clinically relevant model available. If your AAVs are not pre-labeled/packaged with a fluorescent reporter, we can easily transition your project to our bespoke assay services.

	Ready-2-Go AAV Gene Delivery
Cells	Your choice of human, iPSC-derived, glutamatergic neurons, GABAnergic neurons, astrocytes, cardiomyocytes, hepatocytes, and/or retinal pigment epithelial cells
Markers	Nuclei, GFP
Dosing	3 MOIs of each of your AAVs
Positive Controls	AAV2, AAV6, AAV9 packaged with GFP gene
Negative Control	Vehicle(s)

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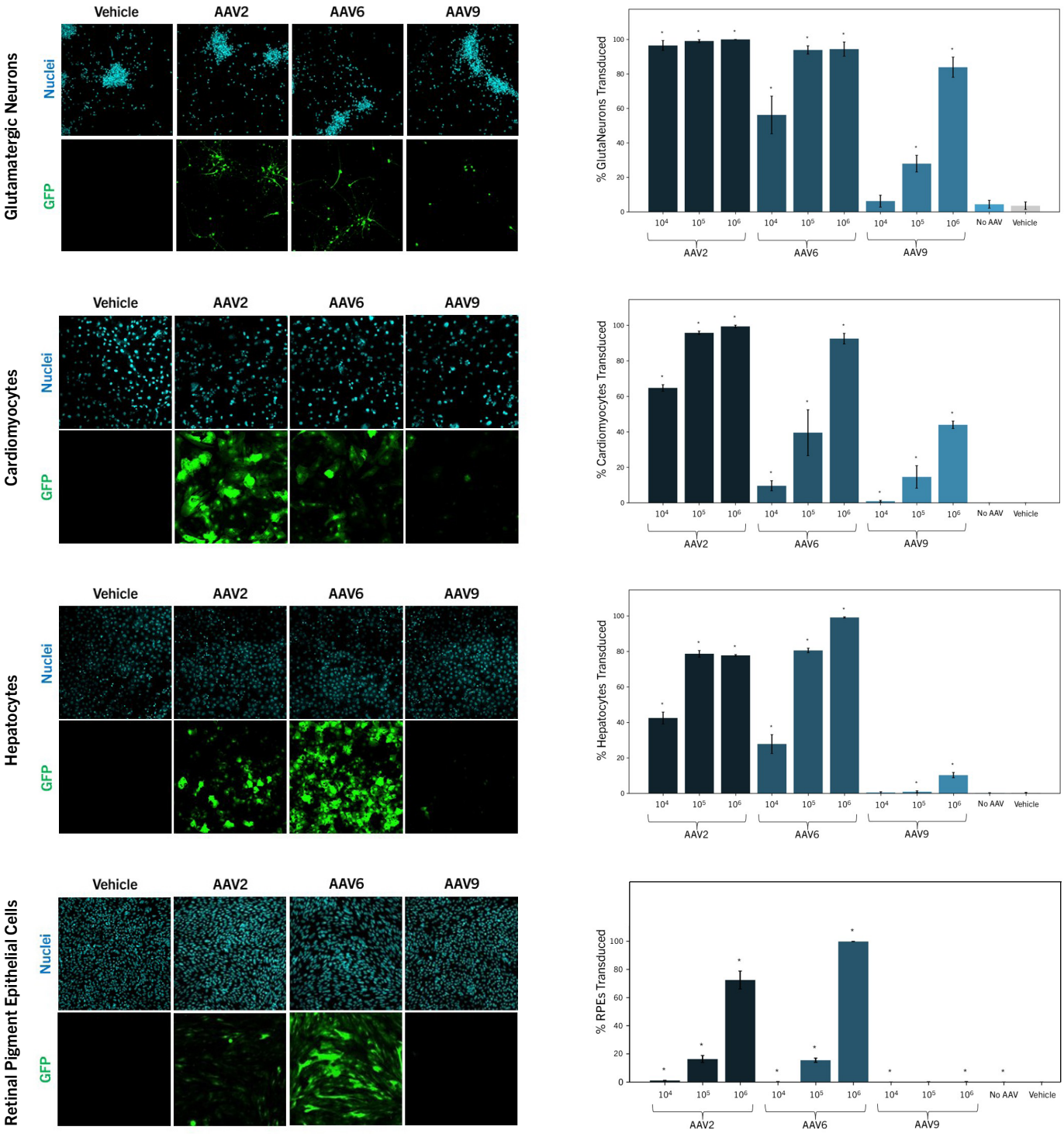
Representative Images: Glutamatergic neurons, cardiomyocytes, hepatocytes, and retinal pigment epithelial cells on day 7 after treatment with vehicle, AAV2, AAV6, or AAV9 packaged with a GFP reporter.

Quantitative analysis shows varying levels of transduction of each AAV serotype in each cell type. Statistical significance was calculated against vehicle.



Data from other cell-type offerings available upon request.