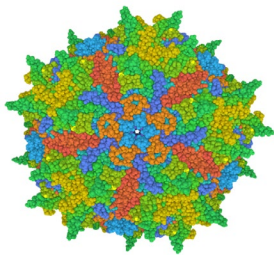
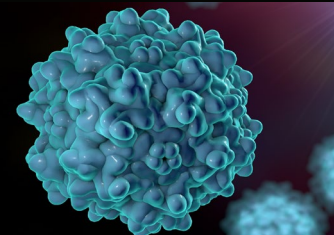


AAV Particles for Gene Delivery



Preclinical and clinical successes of Adeno-Associated Virus (AAV)-mediated gene therapy have established AAV as a near-ideal therapeutic vector. AAVs are non-enveloped viral particles and consist of a capsid containing a short, single-stranded DNA genome. Recombinant AAVs are integration-deficient and deliver a small gene of interest in place of the viral genome. Inside the cell, the recombinant AAV vector exists as an episome and results

in sustained expression of the gene of interest in non-dividing cells. Due to its low immunogenicity and lack of insertion, AAVs are safe for clinical use. They are the best available gene delivery system for animal studies.

Advantages

- Long-term transgene expression (up to 6 months in non-dividing cells)
- Low immunogenicity and low toxicity
- Tissue-specific transduction (tropism)
- Non pathogenic, biosafety level 1
- Best gene delivery system for *in vivo* animal studies

[View our AAV products](#)

AAV Serotypes

Eleven AAV serotypes have been characterized to date, each showing preferential binding to specific cell types. Scientists use this tropism to efficiently target a tissue of interest. In addition, genetically engineered AAV serotypes such as AAV-DJ have been developed to further increase tissue range.

AAV-DJ is a synthetic serotype made from eight wild-type AAV serotypes (AAV2, 4, 5, 8, 9, avian, bovine, and goat AAV) using DNA shuffling. BPS Bioscience's [AAV-DJ](#) particles infect a broad range of cell types and have better transduction efficiency *in vitro* and *in vivo* compared to wild-type serotypes.

BPS Bioscience has developed a portfolio of AAV products, including AAV-DJ products, to support your research needs.

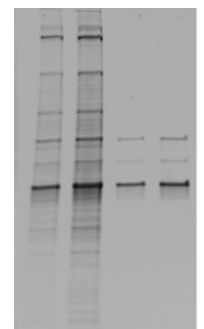
- High titer $\geq 1 \times 10^{12}$ vector genomes (vg)/ml, determined by qPCR.
- Lot-specific quality control, including purity and titer quantification.
- Custom AAV services.

Target tissue	Adeno-associated virus (AAV) serotype										
	AAV1	AAV2	AAV3	AAV4	AAV5	AAV6	AAV7	AAV8	AAV9	AAV-DJ	
CNS/Retina	Yes	Yes		Yes	Yes			Yes	Yes	Yes	Yes
Liver			Yes				Yes	Yes	Yes	Yes	Yes
Lung				Yes	Yes	Yes			Yes	Yes	Yes
Skeletal muscle	Yes					Yes	Yes	Yes	Yes	Yes	Yes
Heart	Yes							Yes	Yes	Yes	Yes
Kidney		Yes									Yes
Pancreas								Yes			Yes

AAV Purification

Animal experimentation requires the use of highly purified particles with minimal amounts of contaminating host cell proteins. The AAV [ONE-Extract™](#) Solution provides a simple and efficient method for particle isolation and is suitable for all AAV serotypes, enabling improved performance over conventional freeze-thaw or sonication methods.

SDS-PAGE analysis of purified AAV2 particles from a crude preparation. Crude particles were purified by iodixanol discontinuous gradient ultracentrifugation and subjected to Freeze-Thaw method (left: 2×10^9 vg and 5×10^9 vg) or ONE-Extract™ (right: 2×10^9 vg and 5×10^9 vg).

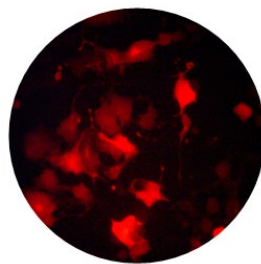
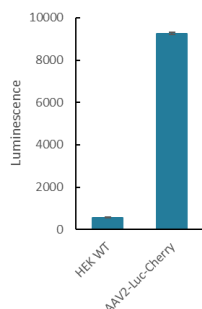
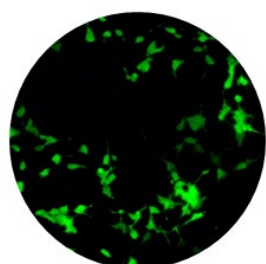


Trust our Quality: we are ISO9001:2015 certified

AAV Particles for Gene Delivery

AAV Reporter Particles

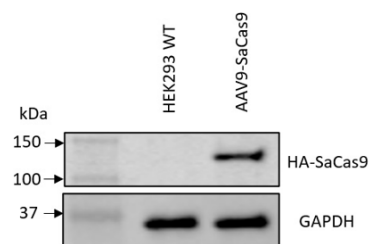
Reporter proteins such as luciferase or fluorescent proteins are ideal to visualize and/or quantify protein expression following AAV transduction. Luciferase, eGFP (green fluorescent protein), ZsGreen, and mCherry-containing AAVs are used to optimize transduction and experimental conditions, track transgene expression over time, or monitor AAV behavior and transduction in vivo.



Fluorescence microscopy and luciferase assay performed in HEK293 cells 72 hours after transduction with AAV1 ZsGreen (left), or AAV2 mCherry-Luciferase (middle and right).

CRISPR/Cas9-based genetic engineering

Endonuclease Cas9 is recruited to a specific DNA sequence by a single-guide RNA and introduces a double stranded break in the DNA. SaCas9 (*Staphylococcus aureus* CRISPR associated protein 9) has high cutting efficiency in mammalian cells, and its small size makes it ideal for packaging into AAV. Our AAV-SaCas9 viruses transduce an HA-tagged SaCas9 to enable detection of the expressed protein. These [AAV-SaCas9](#) particles are used to express SaCas9 in a cell or tissue of interest for knockout or knockin engineering.



Western Blot detection of SaCas9 expression 72 hours after transduction of HEK293 cells with AAV-SaCas9.

shRNA scramble controls

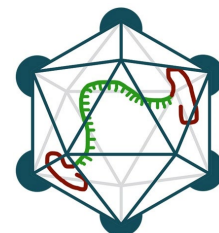
Short-hairpin RNA (shRNA) is one of the most widely used RNA interference methods to block protein translation. AAV-based expression of shRNA drives prolonged reduction of target expression in live animals. AAV-scramble shRNA vectors transduce a 29-mer scramble shRNA sequence to use as control in AAV-mediated shRNA silencing experiments, along with a turbo GFP reporter gene to control for efficiency of transduction.



Fluorescence microscopy detection of GFP in NCI-H358 cells 72 hours after transduction with AAV-DJ scramble shRNA Control.

Custom AAV design and production

BPS Bioscience offers both custom AAV design services and custom packaging service. Our scientific team will generate custom AAV constructs with the reporters and selection markers of your choice, and will manufacture ready-to-use viral particles to transduce your gene(s) of interest. Applications include CRISPR-mediated genetic engineering, shRNA-mediated silencing, protein expression, and the design and optimization of AAV-mediated gene transfer using reporter AAVs.



What information is needed when ordering a custom AAV design or custom AAV packaging service?

Visit our website [FAQs](#) for more information.