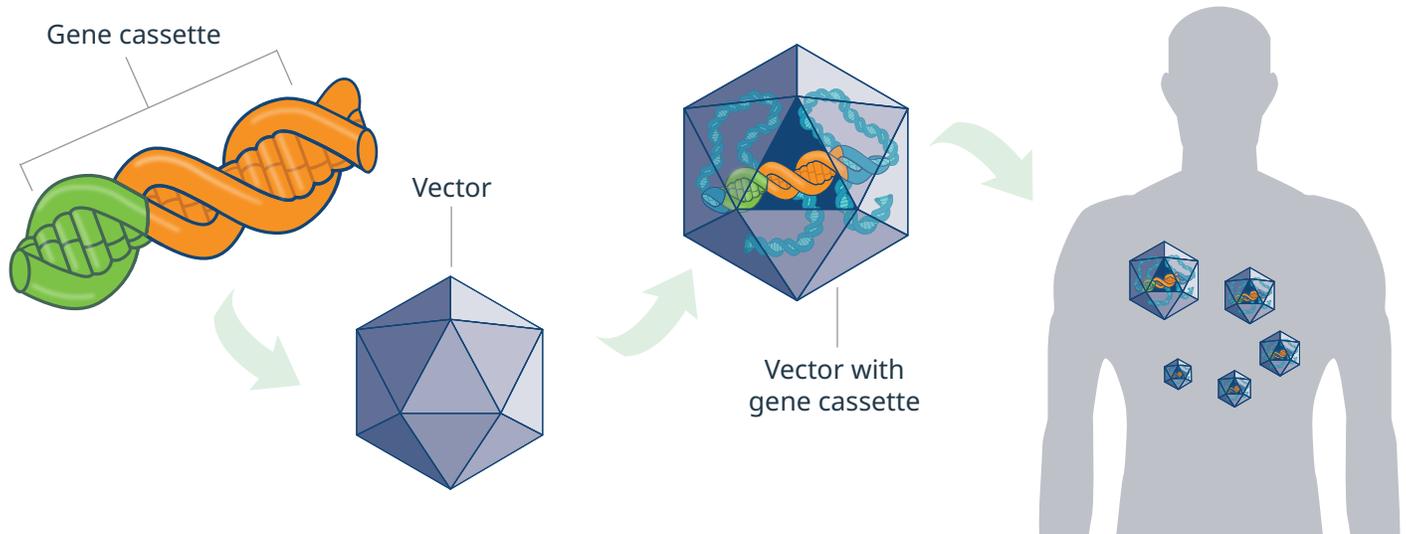


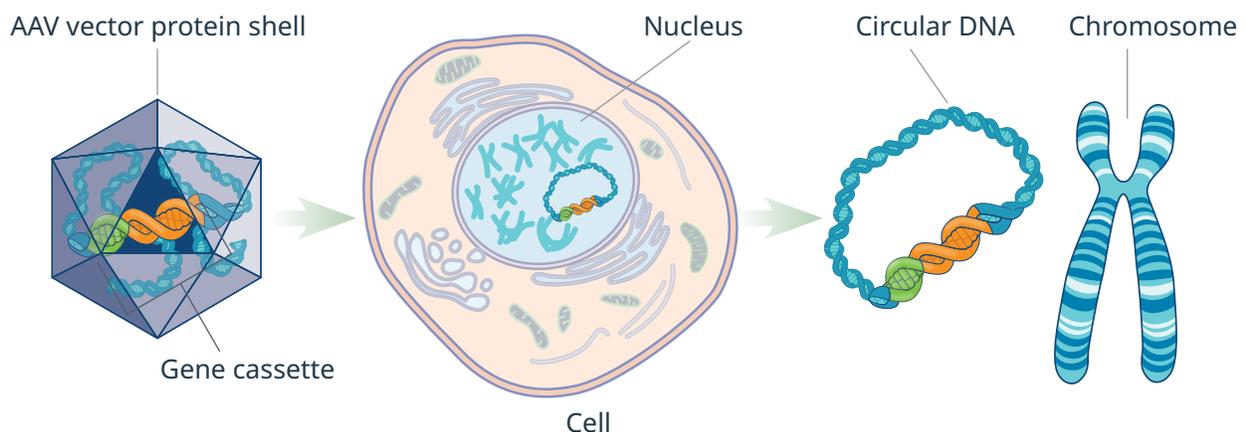
# What are the properties of adeno-associated virus (AAV)-based gene therapy and how does it work?

For 30 years, viral vectors have been investigated for use to deliver gene therapy.<sup>1</sup> Each vector has unique properties.



## What is AAV-based gene therapy?

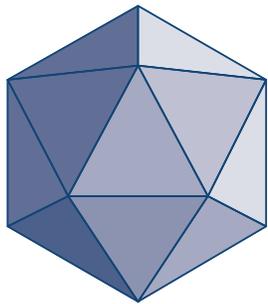
Gene therapy is an experimental method that uses genes to treat or prevent disease.<sup>2</sup> AAV vectors are just one type of delivery vehicle among many.



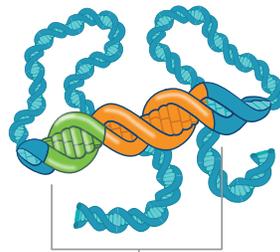
- AAV-based gene therapy vectors are designed to enter the cell and deliver genetic material to the nucleus, which then forms a circle of DNA that exists separately from a person's chromosomes<sup>3</sup>
- This circle of DNA serves as a template for the long-term production of the therapeutic molecule

## What is a vector and a gene cassette?

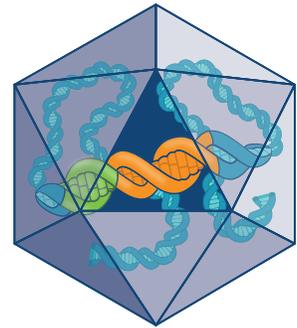
AAVs are commonly used in investigational gene therapies because their virus protein shell can be repurposed as a vector protein shell to deliver a gene cassette to target cells.<sup>4</sup>



Empty AAV vector protein shell



Gene cassette



AAV vector with gene cassette

### Vector protein shell<sup>4</sup>

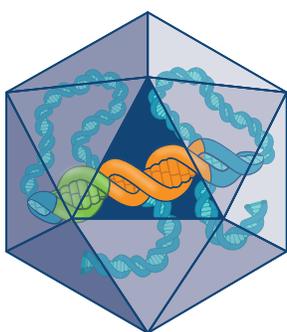
The outer packaging that helps deliver the gene cassette to the cells where the therapeutic molecule will be made

### Gene cassette<sup>5</sup>

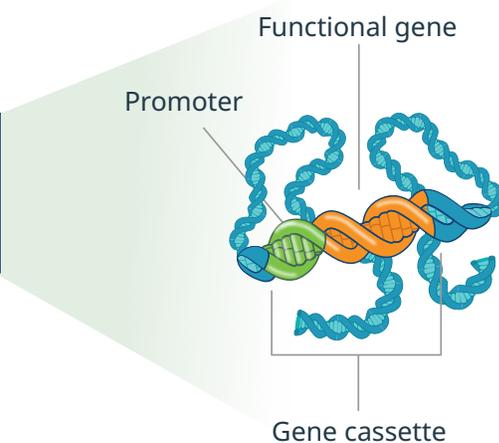
The genetic material the gene therapy vector delivers to the target cells. It typically contains the functional gene and the promoter to help direct the production of a therapeutic molecule

## What is a promoter and a functional gene?

AAV-based gene therapy uses a promoter and a functional gene. Some AAV-based gene therapies use tissue-specific promoters to target expression of the therapeutic molecule in certain cells, restricting expression in other cells.<sup>5</sup>



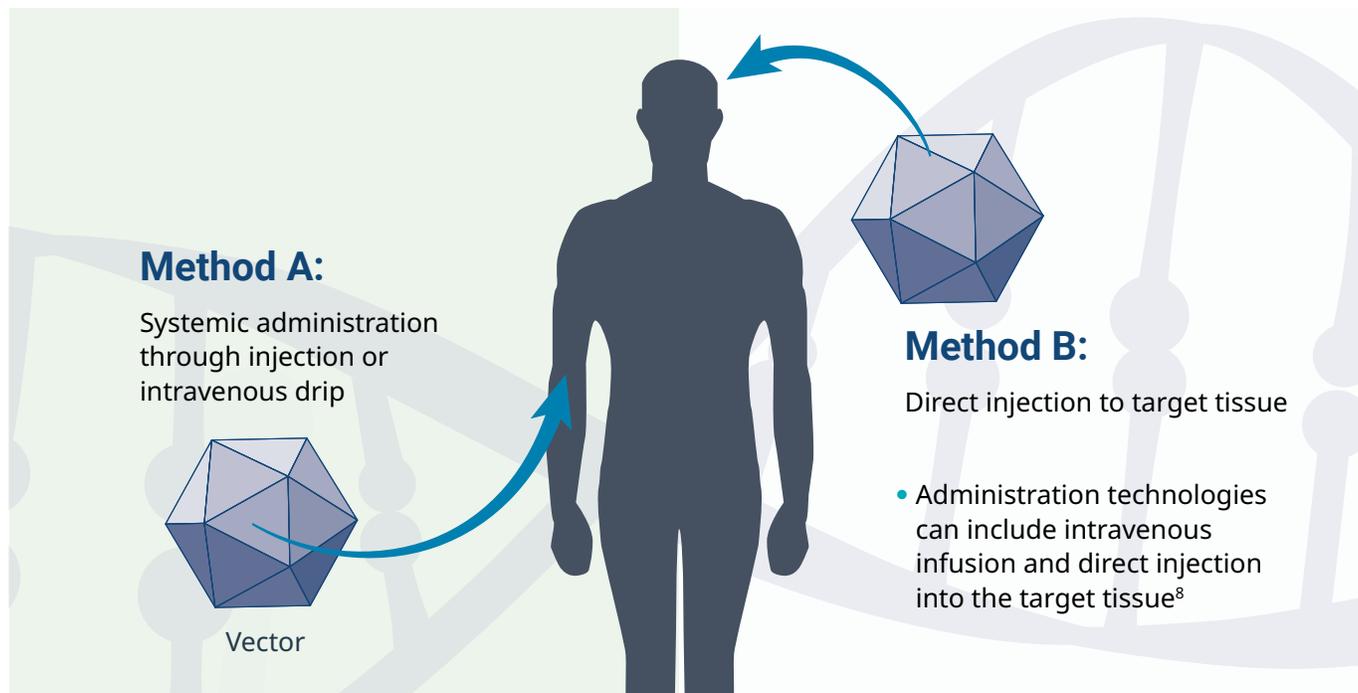
AAV vector with gene cassette



- The gene cassette contains a promoter and a functional gene<sup>5</sup>
- A promoter instructs the cell to produce a therapeutic molecule
- Tissue-specific promoters are advantageous because of their ability to direct expression of protein in target cells, while having little to no effect in other cell types<sup>6,7</sup>
- The functional gene serves as the template (or recipe) for the therapeutic molecule that is inserted into the adeno-associated virus (AAV) vector

## How is adeno-associated virus (AAV)-based gene therapy administered?

AAV vectors may be administered in different ways, depending on the tissue that will be producing the therapeutic molecule. Systemic administration via intravenous infusion and direct injection are two examples.<sup>8</sup>



## What does a profile for AAV-based gene therapy look like?

Gene therapy presents an opportunity to transform lives. The use of AAV vectors has the potential to advance the treatment of genetic diseases.



### AAV-based gene therapy

- Delivers a gene cassette to tissue for the production of a therapeutic molecule<sup>4</sup>
- Can be used to produce therapeutic molecules in a variety of tissue types<sup>6,7</sup>
- Can persist as circular DNA separate from a person's chromosomes<sup>3</sup>
- Delivery strategies can be systemic, such as intravenous infusion, or direct injection, such as into the brain or the eye<sup>8</sup>

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