uniQure

develops medicines that use viral vector gene therapy to treat genetic diseases

How VIRUSES and VECTORS are similar and how they differ

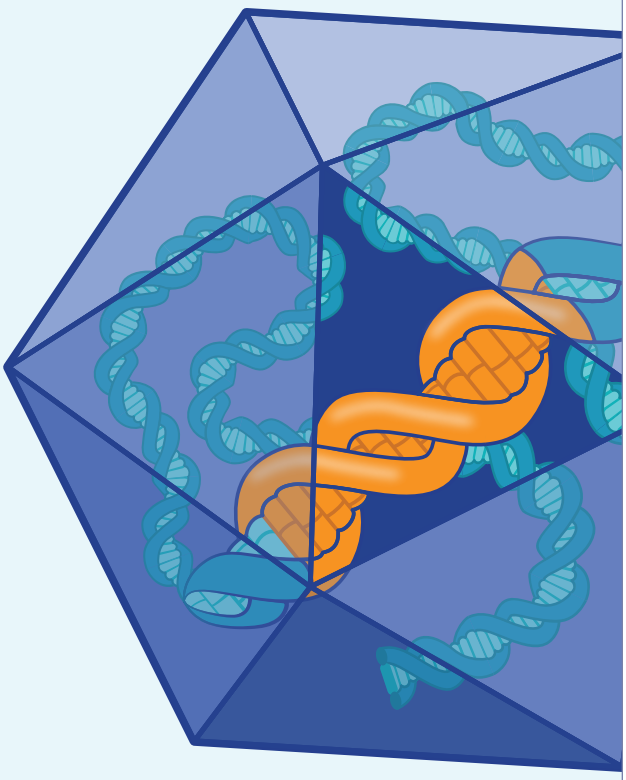


Illustration by Virginia Ferrante-Iqbal

Many human diseases are caused by errors in genes and improper protein functioning.[1] Gene therapy is a technique that uses genetic material to treat diseases.[2] Viral vectors are commonly used to deliver the genetic material.[3]

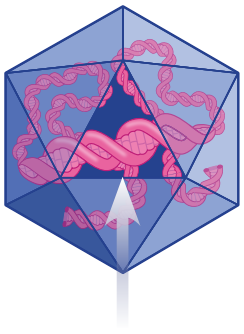
Our mission at uniQure is to deliver single treatment gene therapies that transform the lives of patients. We manufacture non-replicating viral vectors to deliver our investigational gene therapies.

What do viruses and vectors look like?

Viruses are made up of a genome and protein shell.[4] When making a vector, the viral-derived protein shell is repurposed to deliver therapeutic genetic material.[5]

Virus

Protein Shell

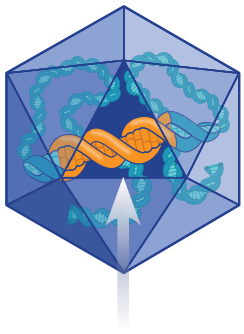


Genome

- Simple viruses have 2 parts: a genome (DNA or RNA) and a protein shell that protects and delivers the genome to the cells it is targeting[4]
- The genome instructs the cell machinery to make more copies of the virus[4]

Vector

Protein Shell



Therapeutic Gene

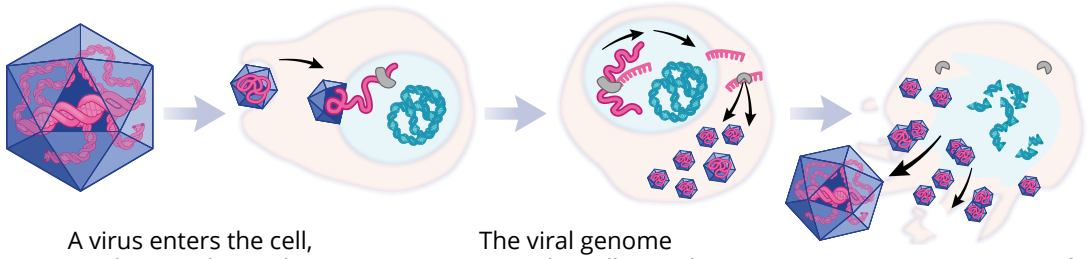
- A vector is the carrier construct containing the therapeutic gene to be delivered to a cell[4]
- Vectors are made up of a therapeutic gene and a viral-derived protein shell that protects and delivers the therapeutic gene to the cells it is targeting[4]
- The therapeutic gene instructs cell machinery to make a therapeutic molecule[4]

Illustrations by Virginia Ferrante-Iqbal

How do viruses and vectors work?

Viruses use their genetic material to replicate.[4]

Virus[4]



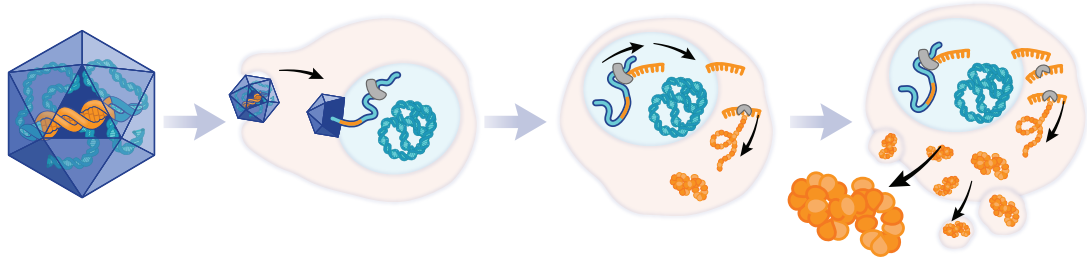
A virus enters the cell, releasing the viral genetic material

The viral genome instructs the cell to make more copies of the virus

Copies of the virus are released

Vectors don't replicate; they carry the therapeutic genetic material to cells.[6]

Vector[6]



A vector enters the cell, releasing the therapeutic genetic material

Therapeutic genetic material instructs the host's cells to create a therapeutic molecule

The therapeutic molecule travels to a site of action to produce a desired effect

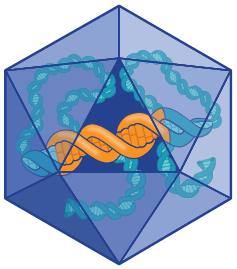
Illustrations by Virginia Ferrante-Iqbal

How do we harness the power of nature to treat disease?

uniQure is leveraging our more than 20 years of gene therapy leadership to advance exciting new treatments for severe genetic diseases.

Healthy Gene

Vector



Vector With Therapeutic Genetic Material

- The protein shell of a virus is repurposed as a package to protect and deliver therapeutic genetic material to treat genetic diseases[4]
- uniQure leverages adeno-associated virus (AAV) vector technology to deliver gene therapy

This is an EXCITING time for science because we can harness the power of nature to deliver transformative therapies

Illustrations by Virginia Ferrante-Iqbal

REFERENCES: 1. National Institutes of Health. How can gene mutations affect health and development? Genetics Home Reference. Accessed April 28, 2020. https://ghr.nlm.nih.gov/primer/mutationsanddisorders/mutationscausedisease **2.** National Institutes of Health. Do all gene mutations affect health and development? Genetics Home Reference. Accessed April 13, 2020. https://ghr.nlm.nih.gov/primer/mutationsanddisorders/neutralmutations **3.** Naso MF, Tomkowicz B, Perry WL III, Strohl WR. Adeno-associated virus (AAV) as a vector for gene therapy. *BioDrugs*. 2017;31:317-334. **4.** Lodish H, Berk A, Zipursky SL, Matsudaira P, Baltimore D, Darnell J. Viruses: structure, function, and uses. In: *Molecular Cell Biology*. 4th ed. W. H. Freeman; 2000:sect 6.3. Accessed April 16, 2020. https://www.ncbi.nlm.nih.gov/books/NBK21523/ **5.** Samulski JR, Muzyczka *N. Annu Rev Virol*. 2014;1:427-551. **6.** Mingozzi F, High KA. Immune responses to AAV vectors: overcoming barriers to successful gene therapy. *Blood*. 2013;122(1):23-36. doi:10.1182/blood-2013-01-306647