# Working Together to Improve the Lives of Hemophilia B Patients

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#AccessToHealthCare

# Gene Therapy overview



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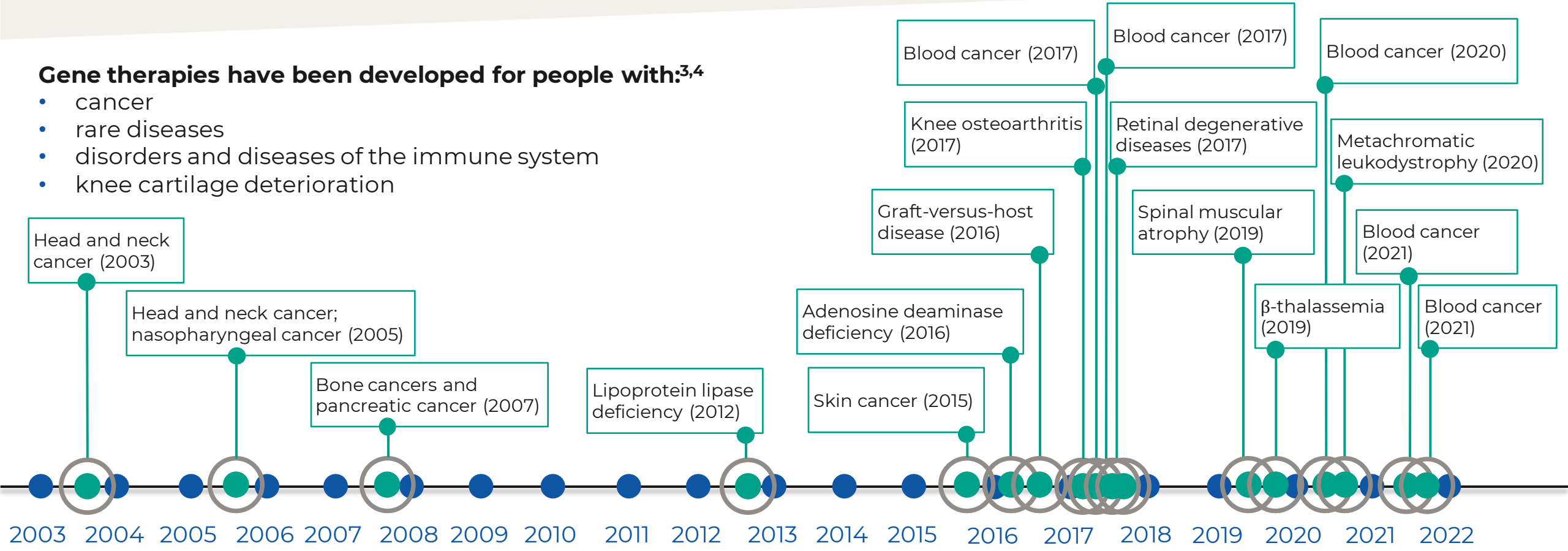
# Many gene therapy products have already been approved for several conditions[1–3]

**Gene therapies have been developed for people with:[3,4]**

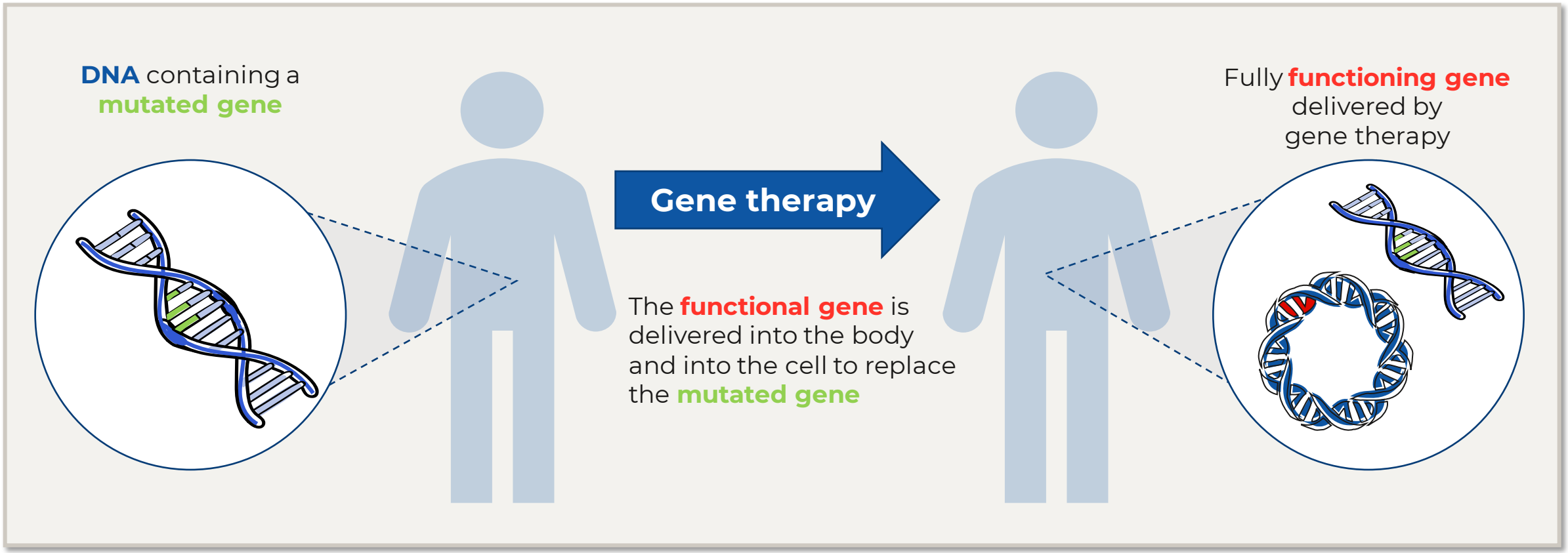
- cancer
- rare diseases
- disorders and diseases of the immune system
- knee cartilage deterioration





1. Shahryari A, et al. *Front Genet* 2019; 10: 10 3389; 2. Cui-Cui Ma, et al. *Biotech Adv* 2020; 40: 107502; 3. Exothera. Timeline of viral vector-based Cell & Gene therapy approvals. Available at: https://exothera.world/wp-content/uploads/2021/05/Timeline-of-viral-vector-based-gene-therapy-approvals.pdf?message=exoscan#contact-form-section. Accessed May 2022.; 4. Exothera. Worldwide approved gene therapies based on viral vectors. Available at: https://exothera.world/worldwide-approved-gene-therapies-based-on-viral-vectors/. Accessed September 2022.

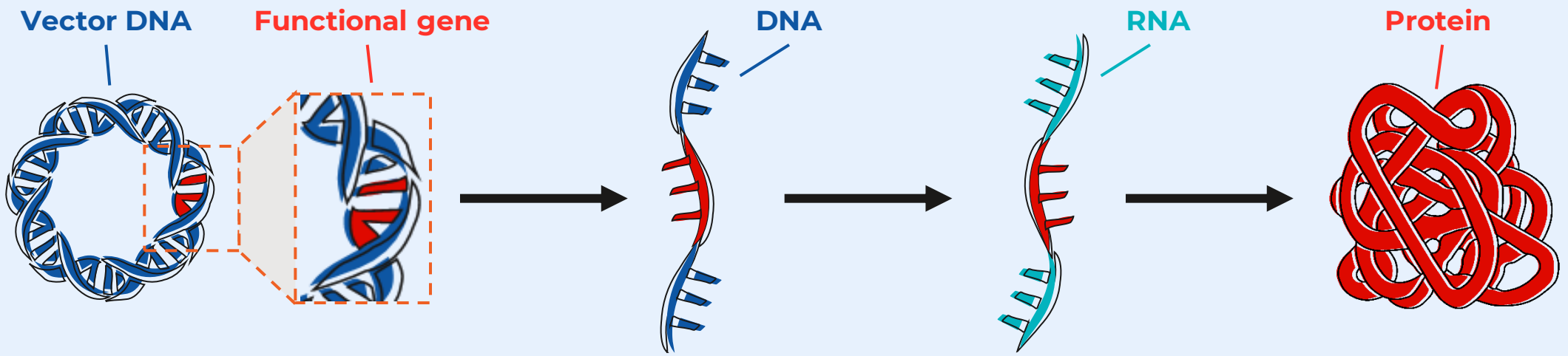
# Gene therapy is an innovative treatment that aims to overcome conditions caused by a gene mutation[1–3]




1. Yourgenome. What is gene therapy? Available at: https://www.yourgenome.org/facts/what-is-gene-therapy. Accessed May 2022; 2. US Food and Drug Administration. What is Gene Therapy? 2018. Available at: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/what-gene-therapy. Accessed May 2022; 3. Miesbach W, et al. *Haemophilia* 2019; 25: 545–557.

# From gene to protein

**The production of RNA and protein from our own DNA and genes is a routine process occurring in all the cells of our body everyday[1]**



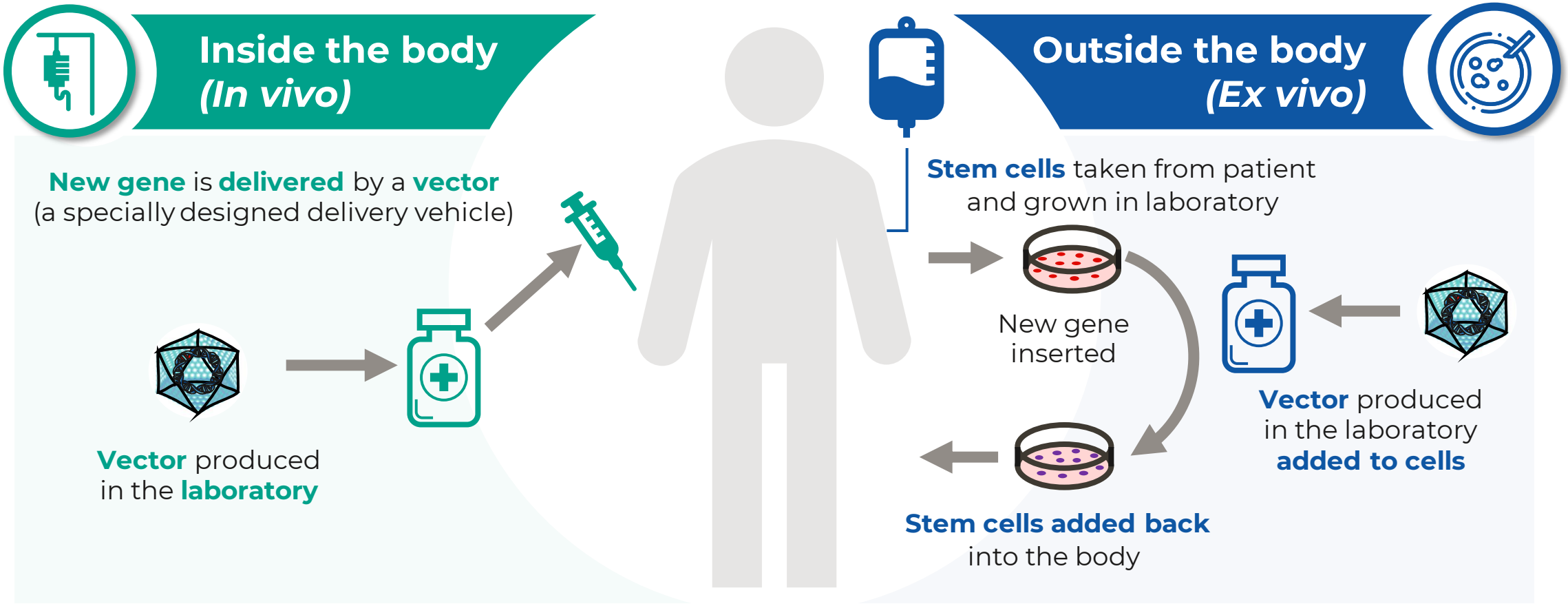

DNA: The blueprint – read by our cells machinery by RNA

RNA: The instructions for building proteins

In AAV-based gene therapy, the DNA is provided by the vector; the cells own machinery carry out the rest of the process[2]

1. Yourgenome. From DNA to protein. Available at: https://www.yourgenome.org/video/from-dna-to-protein/. Accessed September 2022; 2. Naso MF, et al. *BioDrugs* 2017; 31: 317–334.

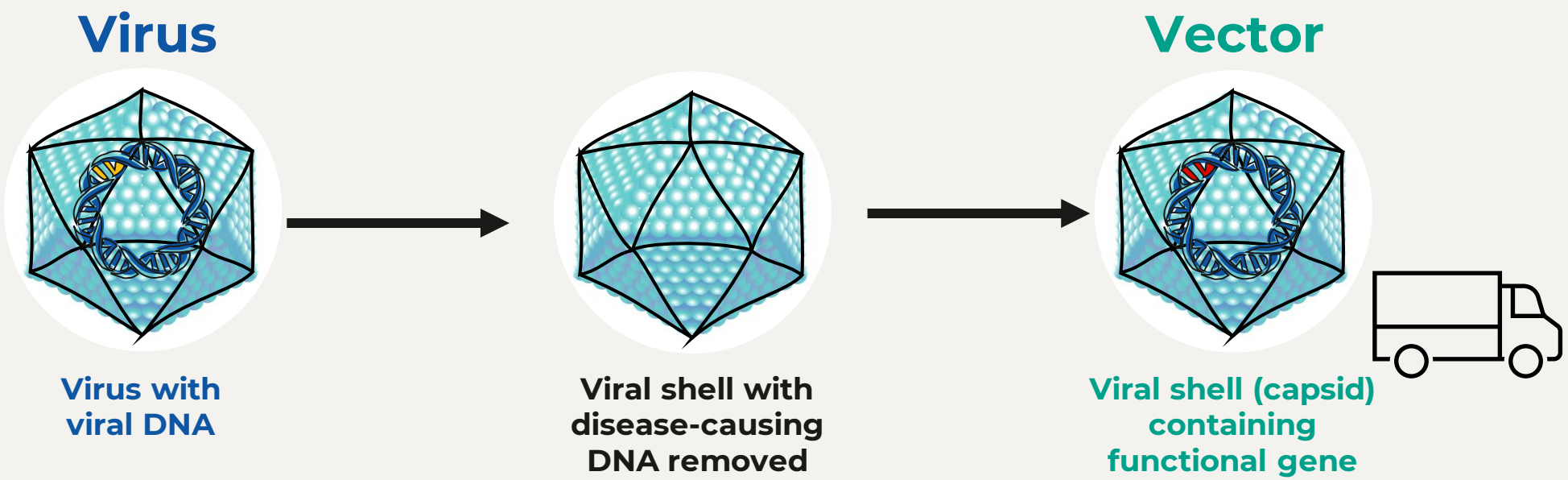
# Gene therapy can be delivered inside (*in vivo*) or outside (*ex vivo*) the body[1–3]




1. Adapted from: Bulcha JT, et al. *Signal Transduct Target Ther* 2021; 6: 53; 2. High KA, Roncarolo MG. *N Engl J Med* 2019; 381: 455–464; 3. High KA, Roncarolo MG. *N Engl J Med* 2019; 381: Supplementary graphic.

# To insert new genes directly into cells, scientists use a delivery vehicle called a “vector”

## Viruses are currently the most commonly used vectors in gene therapies[1–4]




DNA, deoxyribonucleic acid. 1. Polyplus. Viral vectors for gene therapy in a nutshell: AAVs, lentivirus, adenovirus and retrovirus. Available at: https://www.polyplus-transfection.com/blog/article/viral-vectors-for-gene-therapies/. Accessed August 2022; 2. US Food and Drug Administration. What is Gene Therapy? 2018. Available at: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/what-gene-therapy. Accessed August 2022;
3. Kay MA, et al. *Nat Med* 2001; 7: 33–40; 4. agtc. Science & Technology. Available at: https://agtc.com/science/. Accessed August 2022.

# Why are viruses used in gene therapies?

## Vectors (vehicles) are required to deliver the gene and genetic material into cells[1]

- **Naturally occurring viruses** are very **effective at entering cells in the body**[1,2]

### Virus

**Viral shell (capsid) containing virus DNA**

### Vector

**Viral shell (capsid) containing functional gene**

- **In gene therapy, virus DNA is removed from the viral shell (or capsid)** so that they cannot cause disease[1,2]
- The **functional gene** is **inserted into the empty viral shell (or capsid), creating the gene therapy vector**, which is then given to patients[4]

DNA, deoxyribonucleic acid. 1. US Food and Drug Administration. How Gene Therapy Can Cure or Treat Diseases 2022. Available at: https://www.fda.gov/consumers/consumer-updates/how-gene-therapy-can-cure-or-treat-diseases. Accessed August 2022; 2. US Food and Drug Administration. What is Gene Therapy? 2018. Available at: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/what-gene-therapy. Accessed August 2022; 3. Kay MA, et al. *Nat Med* 2001; 7: 33–40; 4. AGTC. Science & Technology. Available at: https://agtc.com/science/. Accessed August 2022.

# Gene Therapy in Hemophilia



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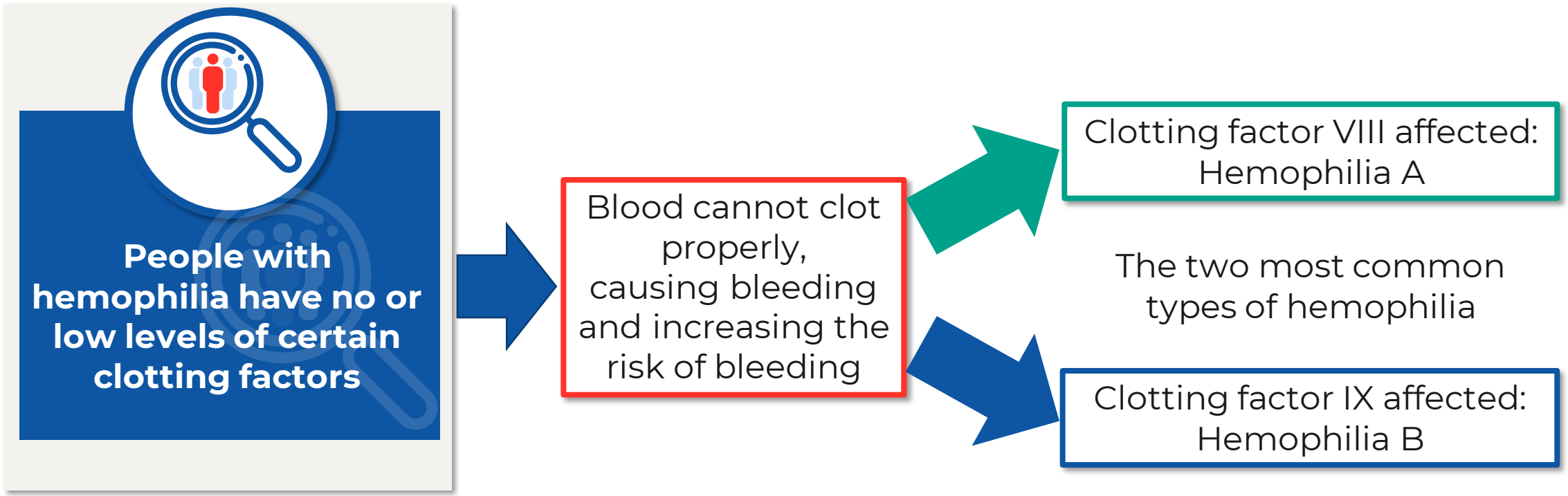
# Hemophilia is a rare bleeding condition where blood does not clot properly, causing excessive bleeding

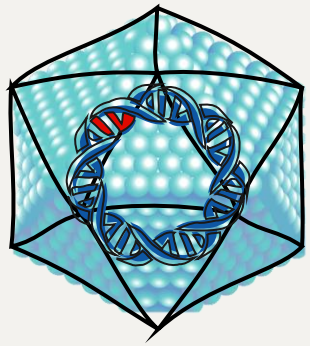



CDC. Hemophilia facts. https://www.cdc.gov/ncbddd/hemophilia/facts.html. Accessed July 2022.

# AAV-based gene therapy is well suited for hemophilia B

**The gene that makes factor IX is relatively small**

Easier to “pack” it into the AAV vector and deliver it to the cells



AAV, adeno-associated viruses.
Hemevoluiton. AAV-based gene therapies are well suited for hemophilia B. Available at: https://www.hemevolution.com/hcp/aav-gene-therapy-hemophilia-b-fit. Accessed May 2022.

Thank you