

A (very) brief introduction to Gene Therapy

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Some basics of gene therapy in advance of hearing about current treatments

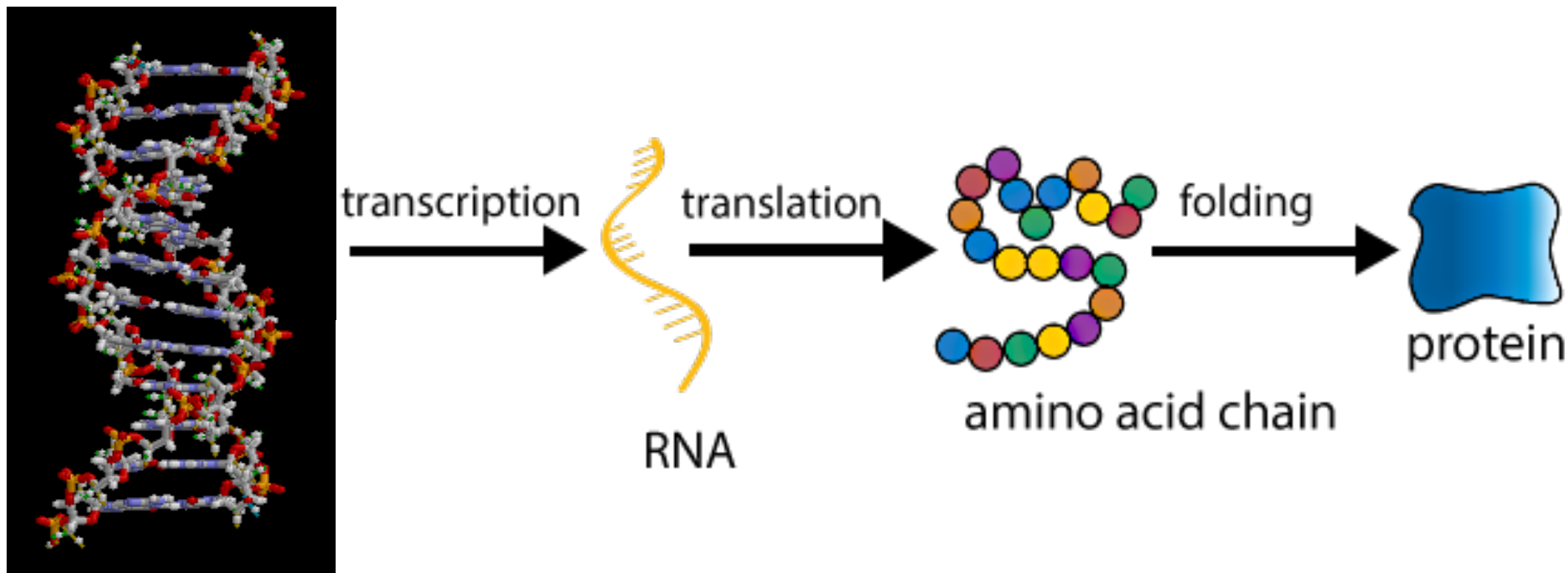
Covering.....

Genes and DMD

Gene Delivery

Microdystrophin and future prospects

From Genes to Proteins



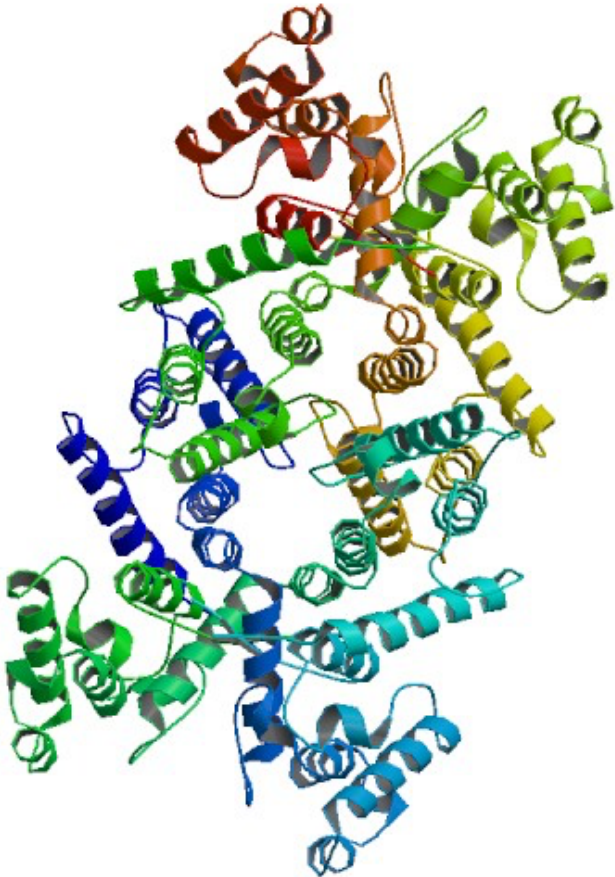
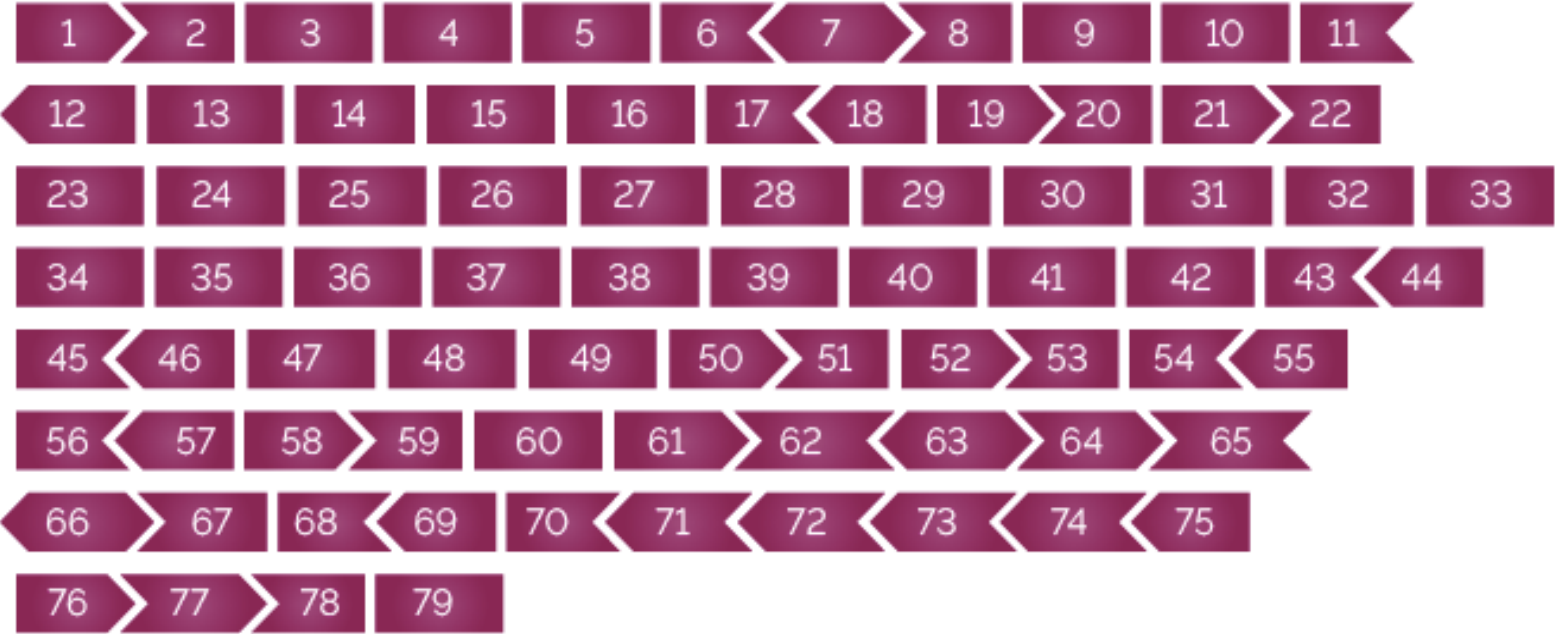
In DMD, the instructions for making Dystrophin are wrong, missing or duplicated and so the protein is not made

Gene Therapy

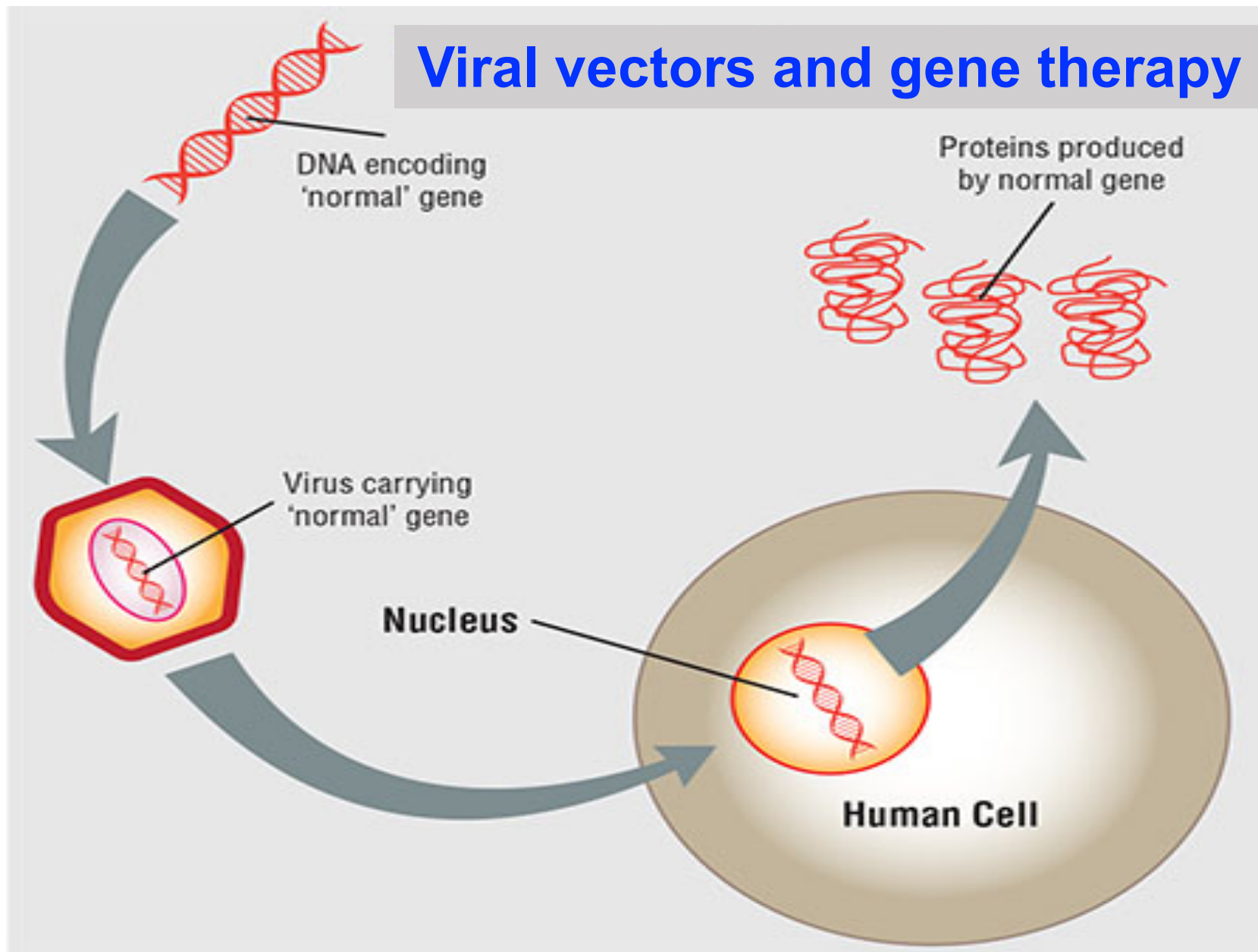
Gene therapy is the ‘modification’ of the genetic material in the cells of a person in order to:

- replace a faulty gene
- introduce a new gene to modify the symptoms of a disease
- modify the way genes are expressed within a cell

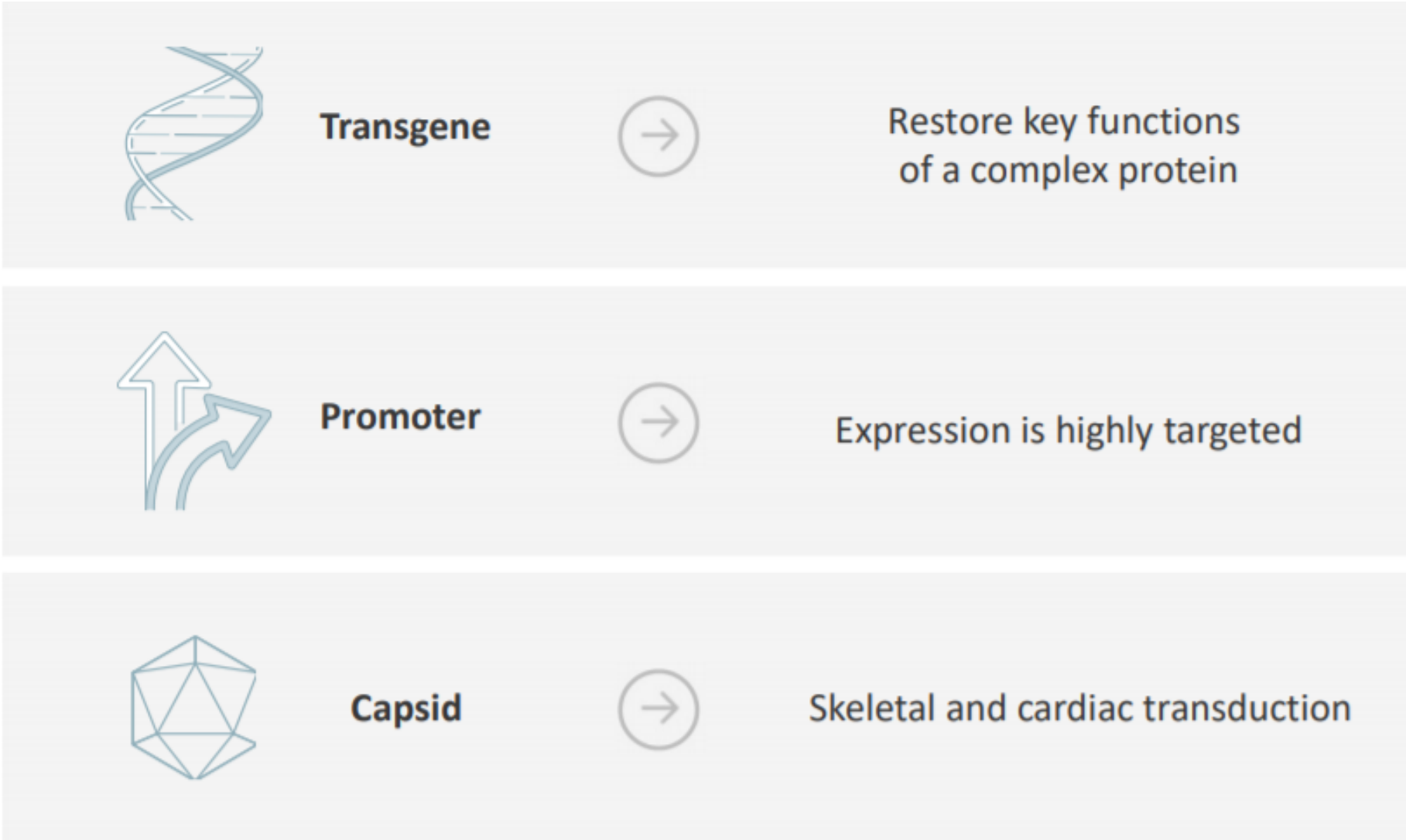
What are we trying to replace?



Dystrophin gene is large – made up of 79 exons
 We need to deliver this to all the muscle cells
 How can we do this?



Three important elements make up a viral vector



There are some challenges to viral vector delivery of gene therapy

Size restrictions

Microdystrophin

Exosomes in the future?

Immunology/Safety

Innate immune response – already present

Adaptive immune response – as a result of the gene therapy

Physical Barriers within the body

Manufacture of vectors

Now let's hear how this is progressing in clinical trials.