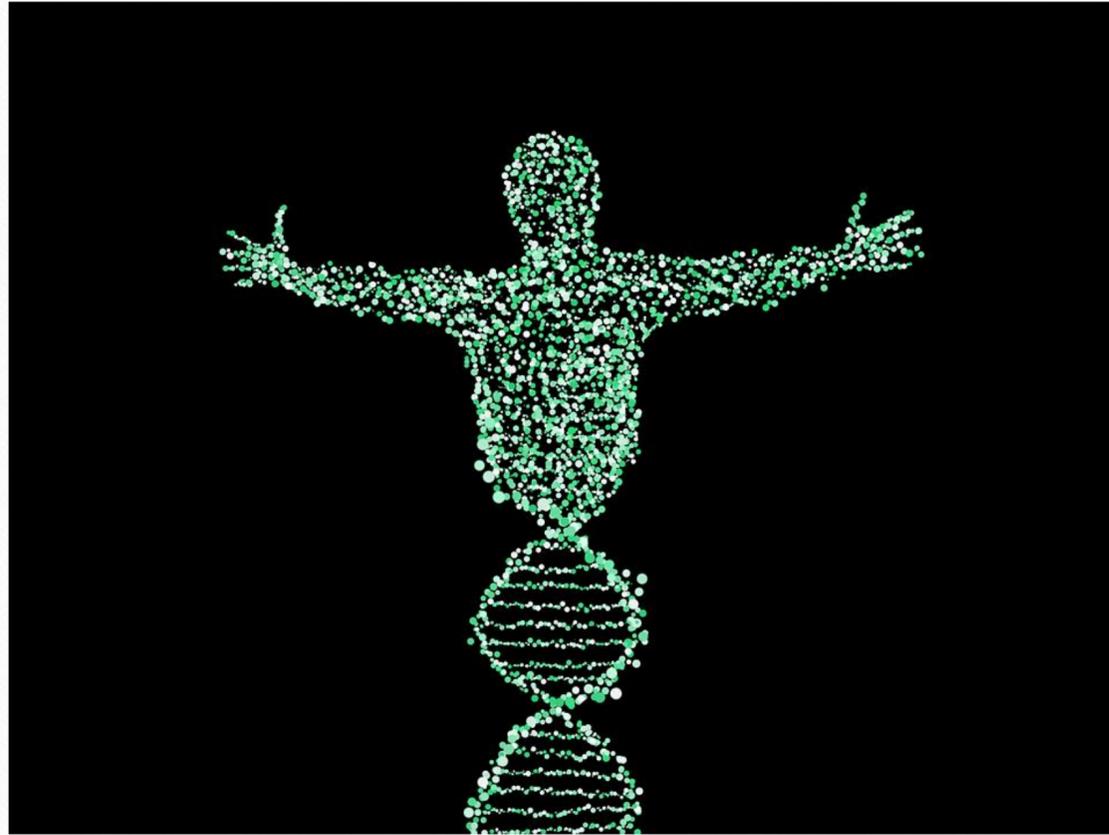


Genome editing and CRISPR

What is a gene?

What are germ cells, somatic cells, and
stem cells?

How are CRISPR therapies delivered?

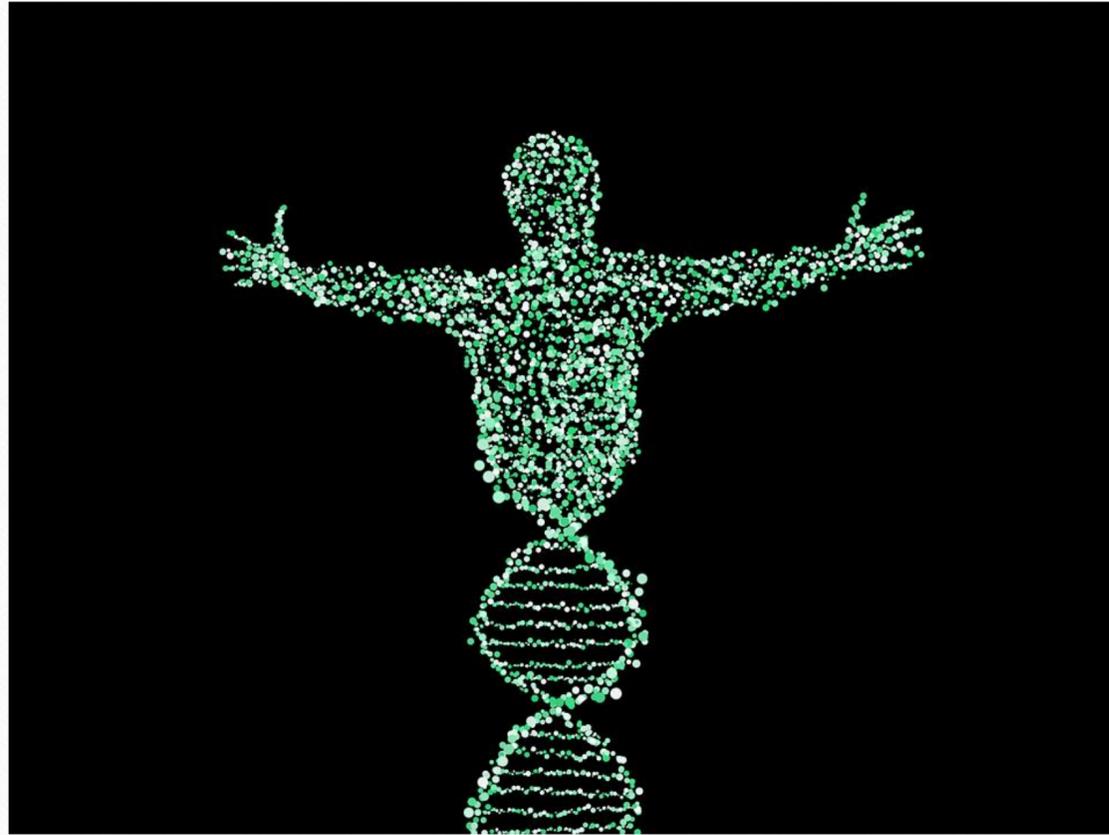


**ACTGTAGCCATAGAATAGTCT
GTAATAGCTCGATGCTCGGTA
GATCTTAGACAGACAGTATCG
GCTTTAGACAGATAGTCTCGA
CGCTGACGCTTCTGATACGCT
GATAGACAGTCTCGTGACAG
ACGACAATAGACGCTCGTCG
CAATCGGC**

DNA is the 'Recipe book of Life'

and

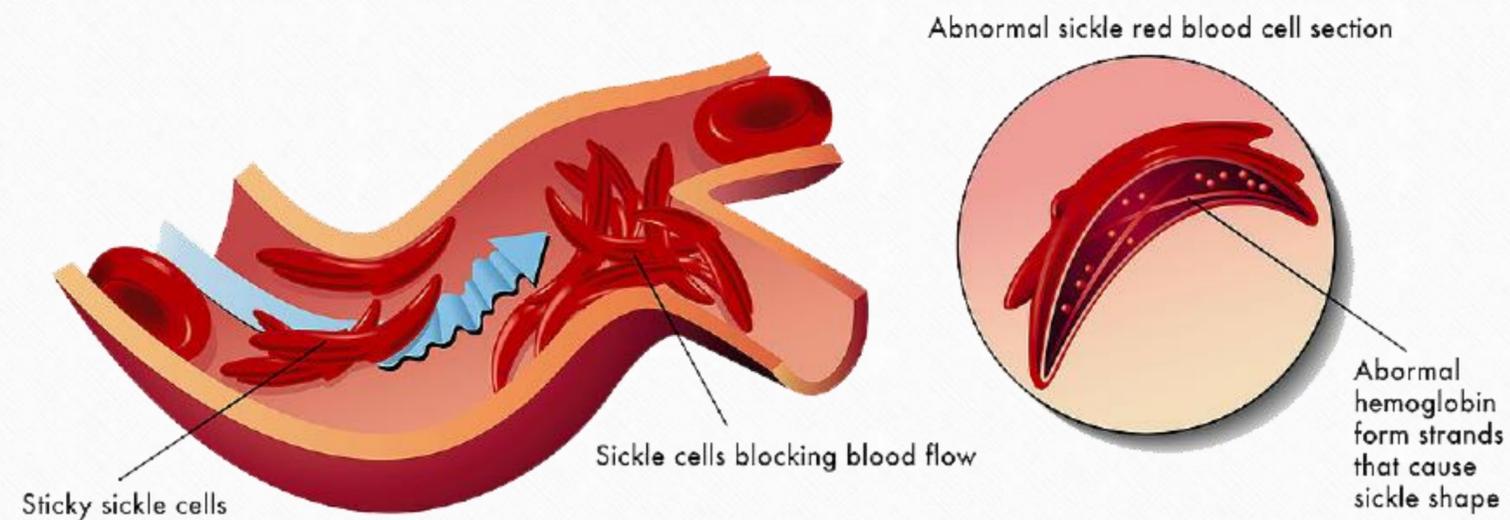
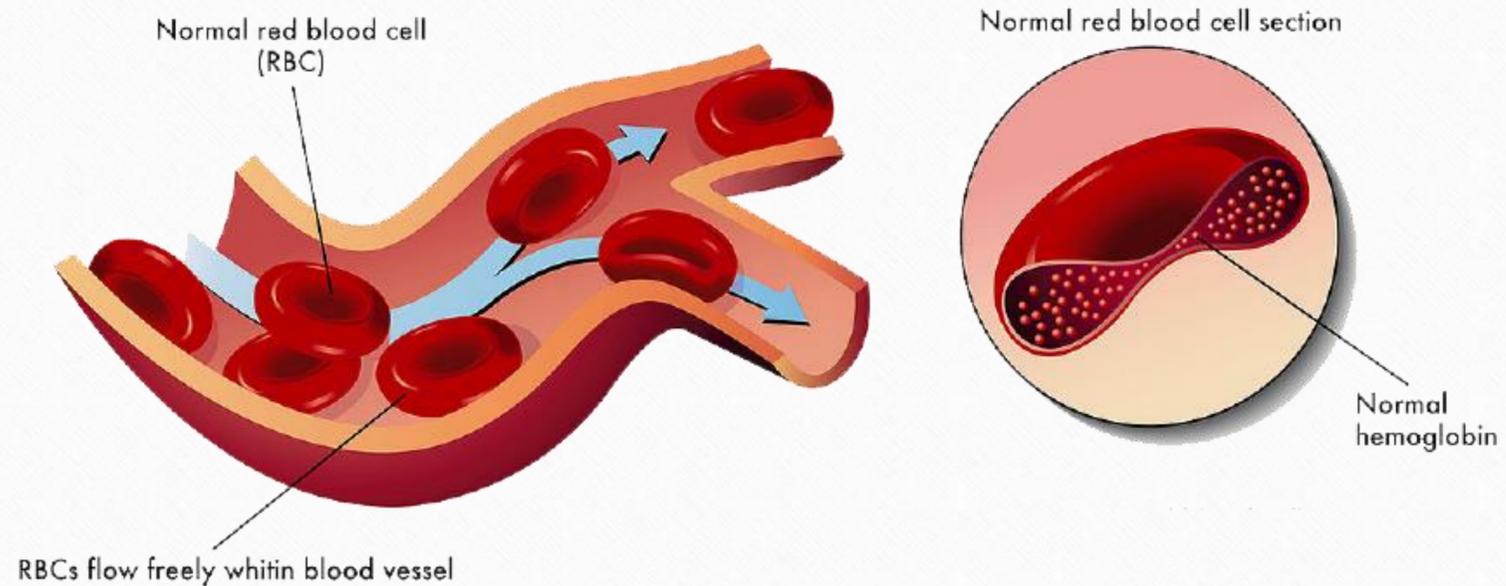
DNA can be found in virtually every cell of the body



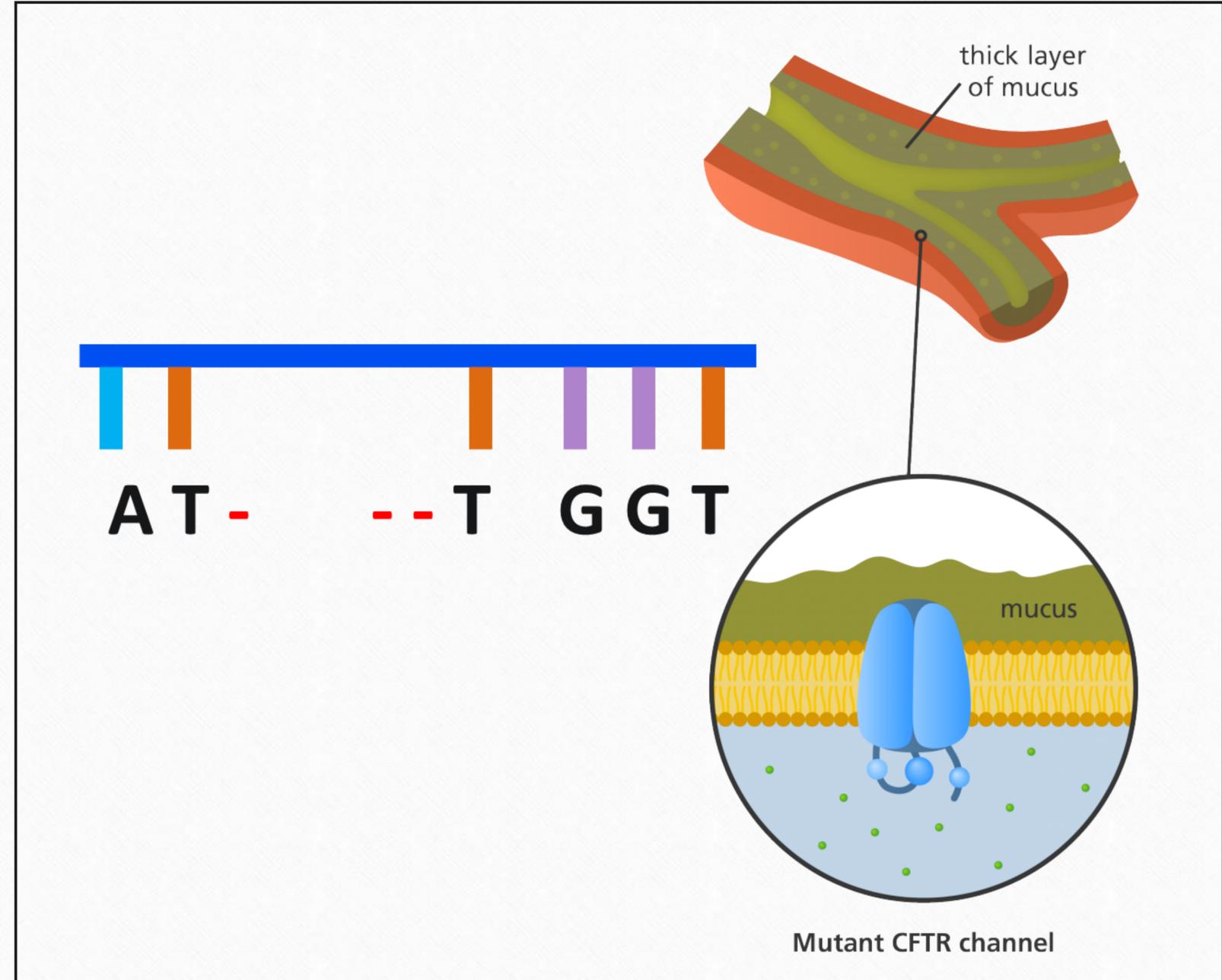
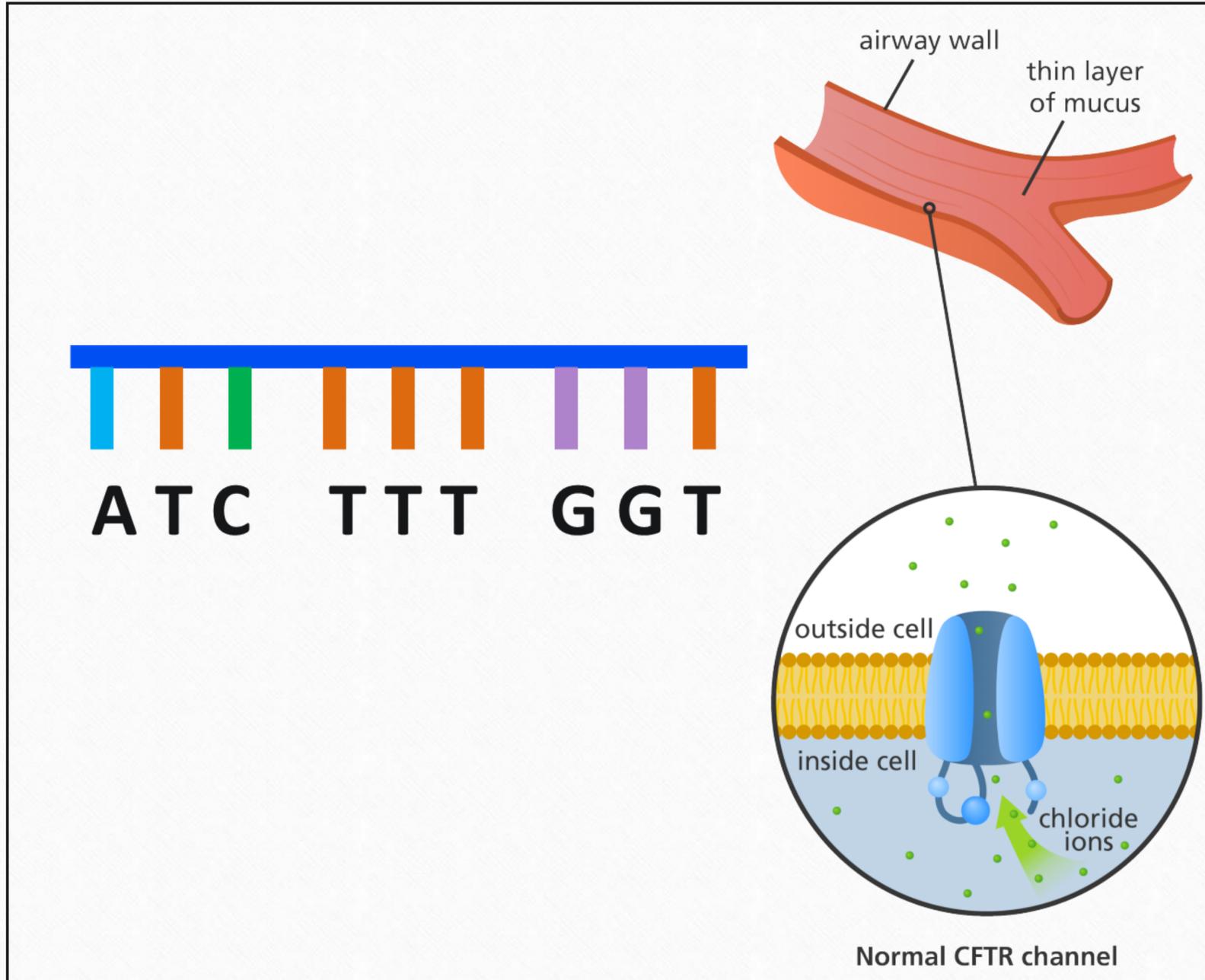
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CAATCGGC
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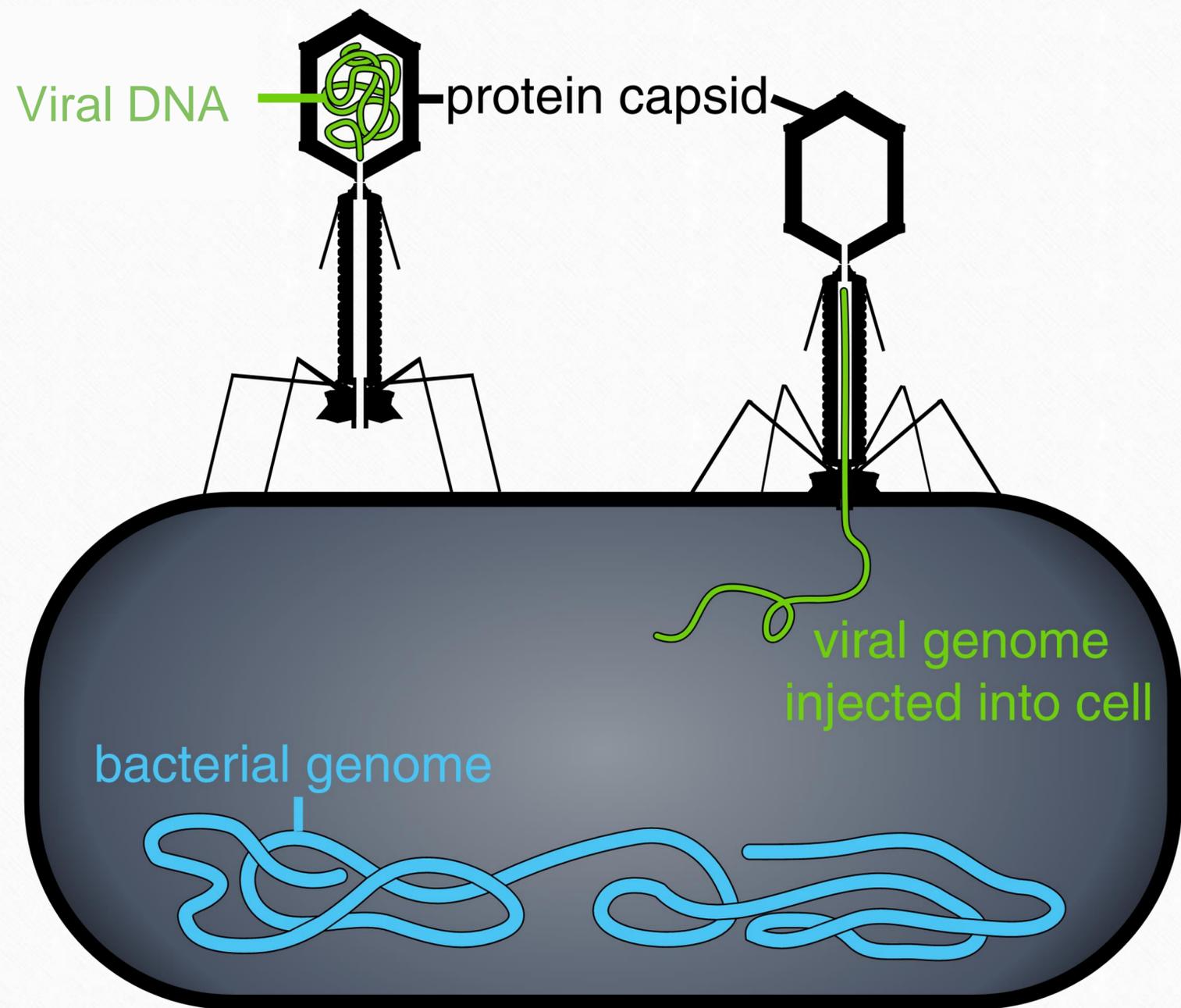
Changes in the DNA code create variation
some of which can be detrimental to human health

Sickle Cell



Cystic Fibrosis





CRISPR

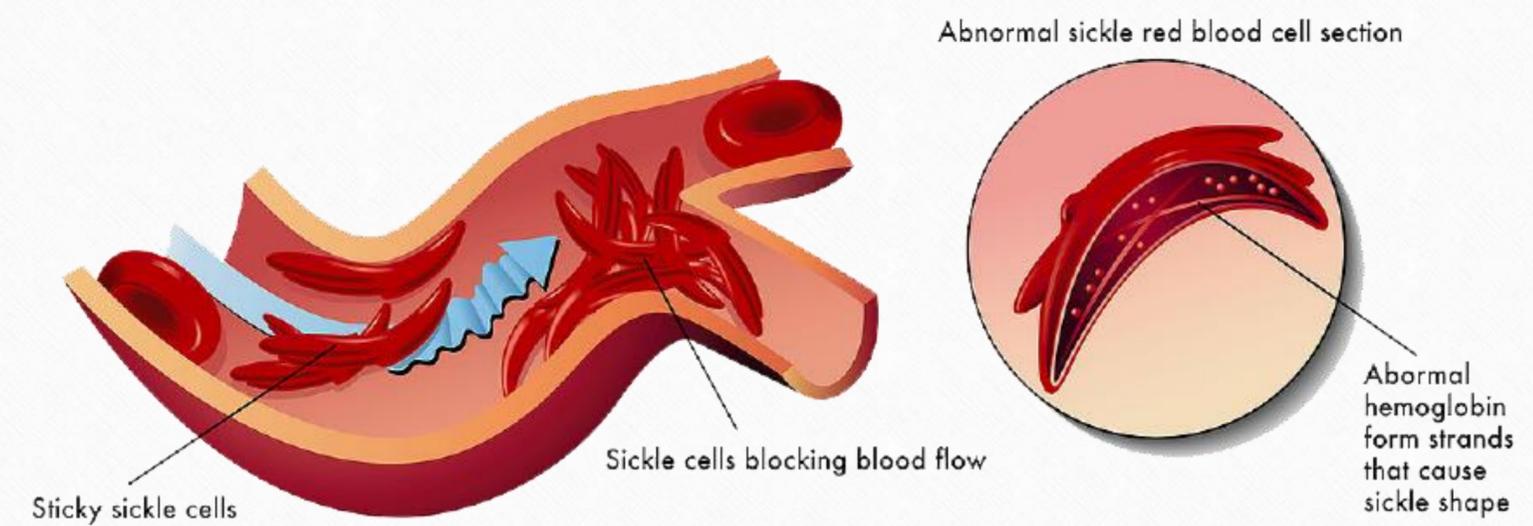
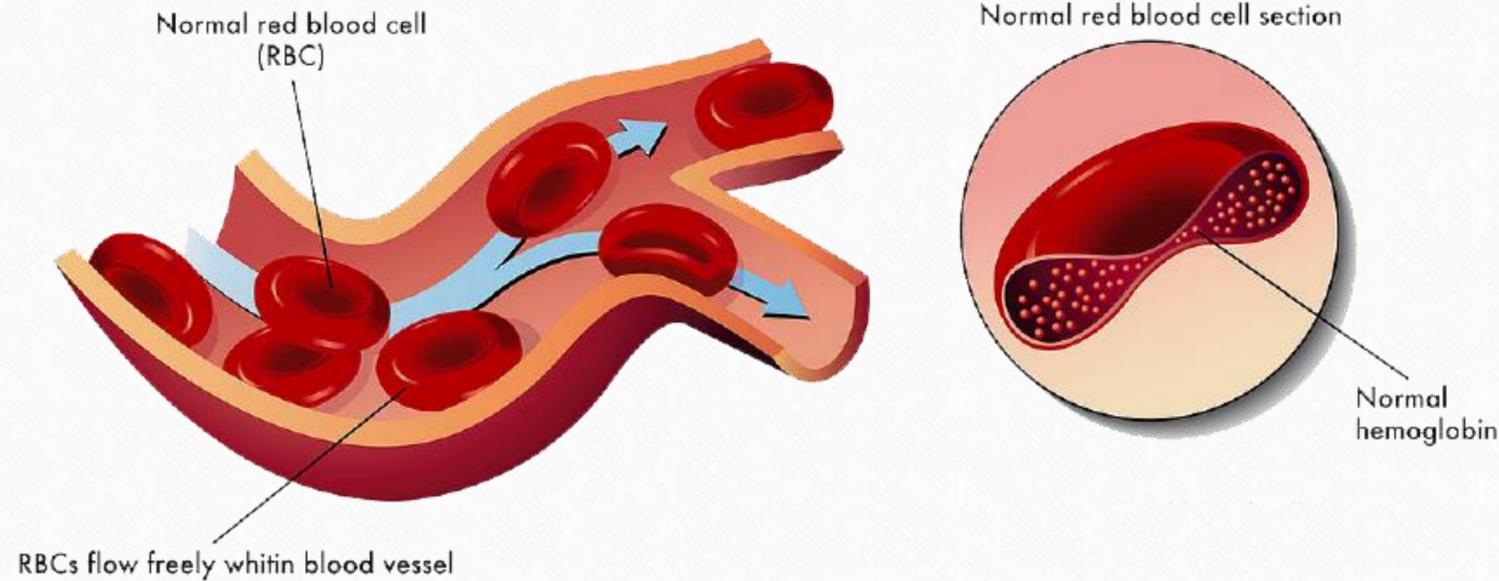
- Bacterial 'immune system'
- Recognizes specific DNA sequence
- Changes DNA



CRISPR

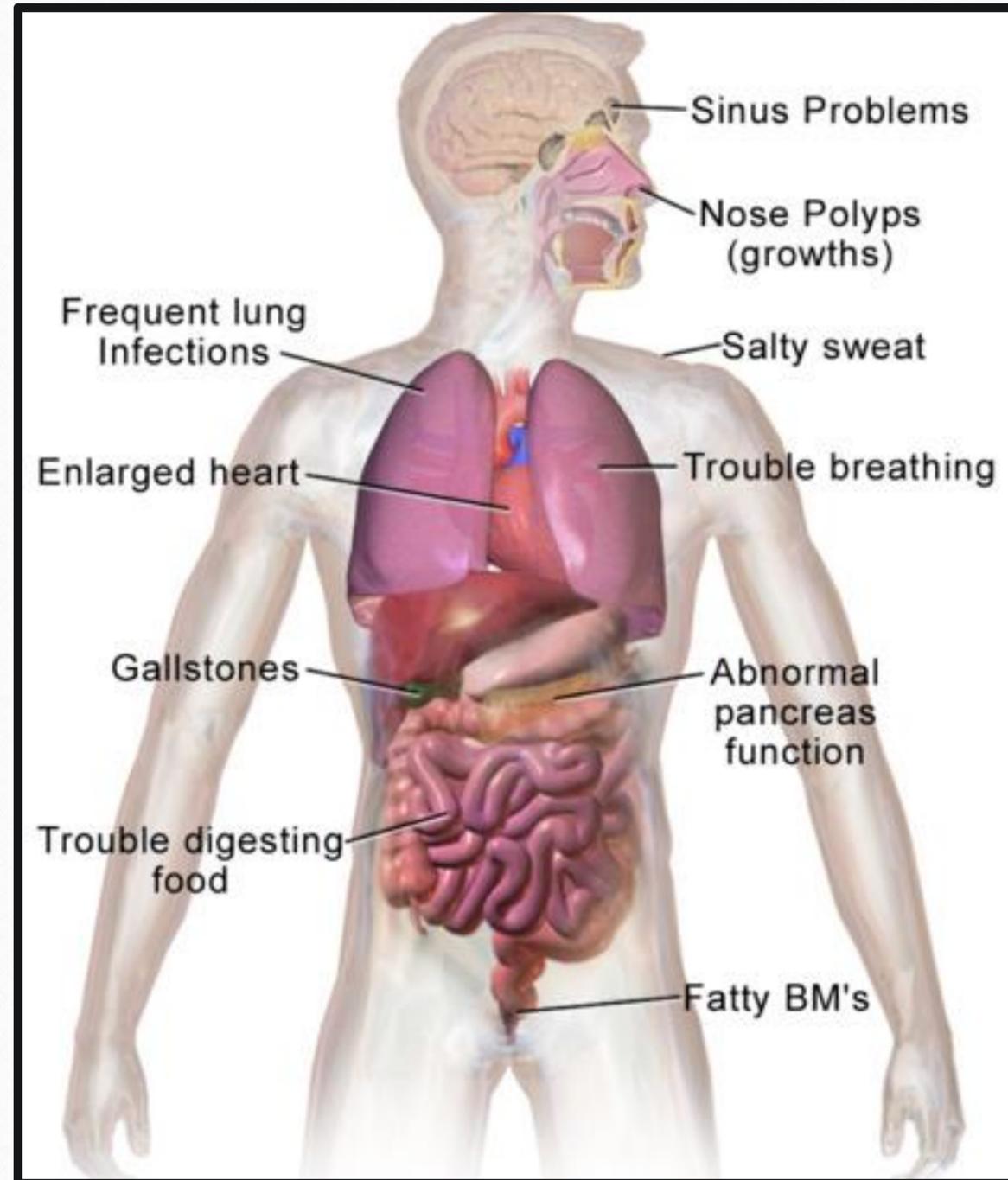
- Bacterial 'immune system'
- Recognizes specific DNA sequence
- Changes DNA
- Adapted as genetic tool in human cell culture (2013)

Sickle Cell



Blood cells can be treated outside the body

Cystic Fibrosis



Need to target CRISPR to specific organ systems



CRISPR

- Genetic tool used in scientific research
- Under development
- Some early clinical trials to use CRISPR as a medical treatment

Layla Richards (2015) the first success of genome editing-based gene therapy



Great Ormond Street Hospital

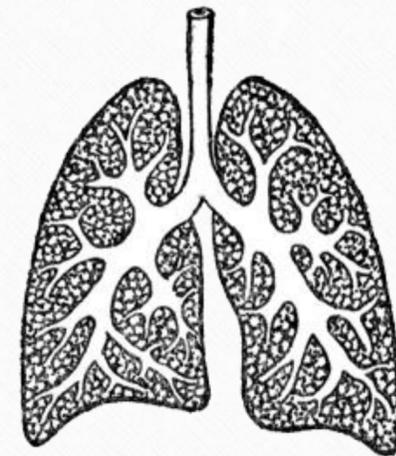


Image: Maidiel1, CC BY-SA 4.0

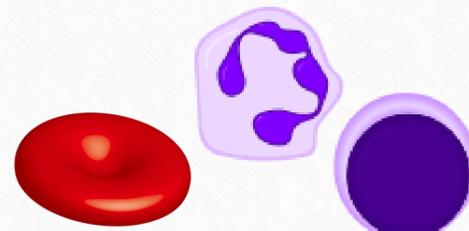
Might genome editing
one day lead to a
solution to the global
shortage of organs?

Somatic cells

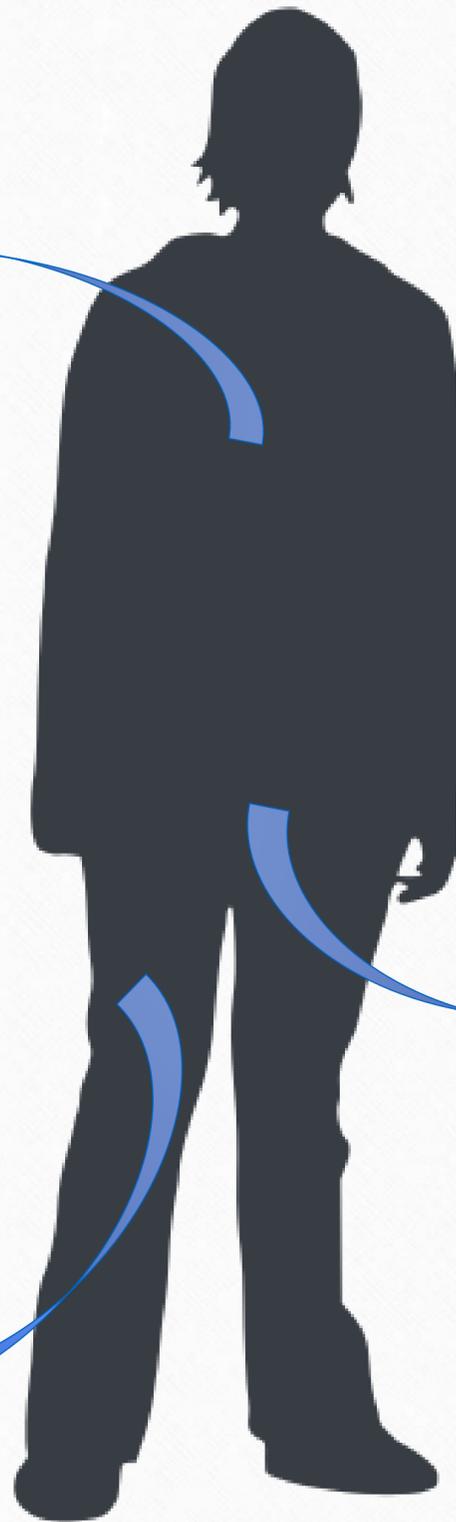
"Body" cells,
Not passed on to future
generations



Lungs



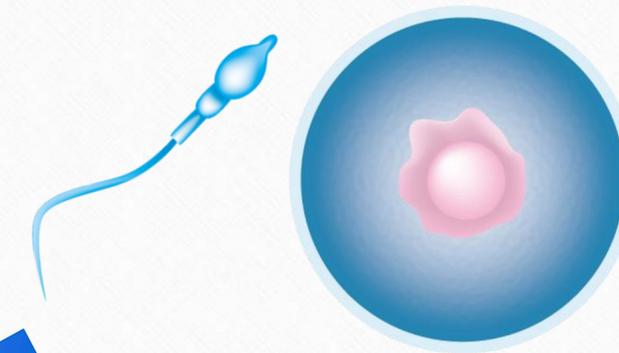
Blood cells



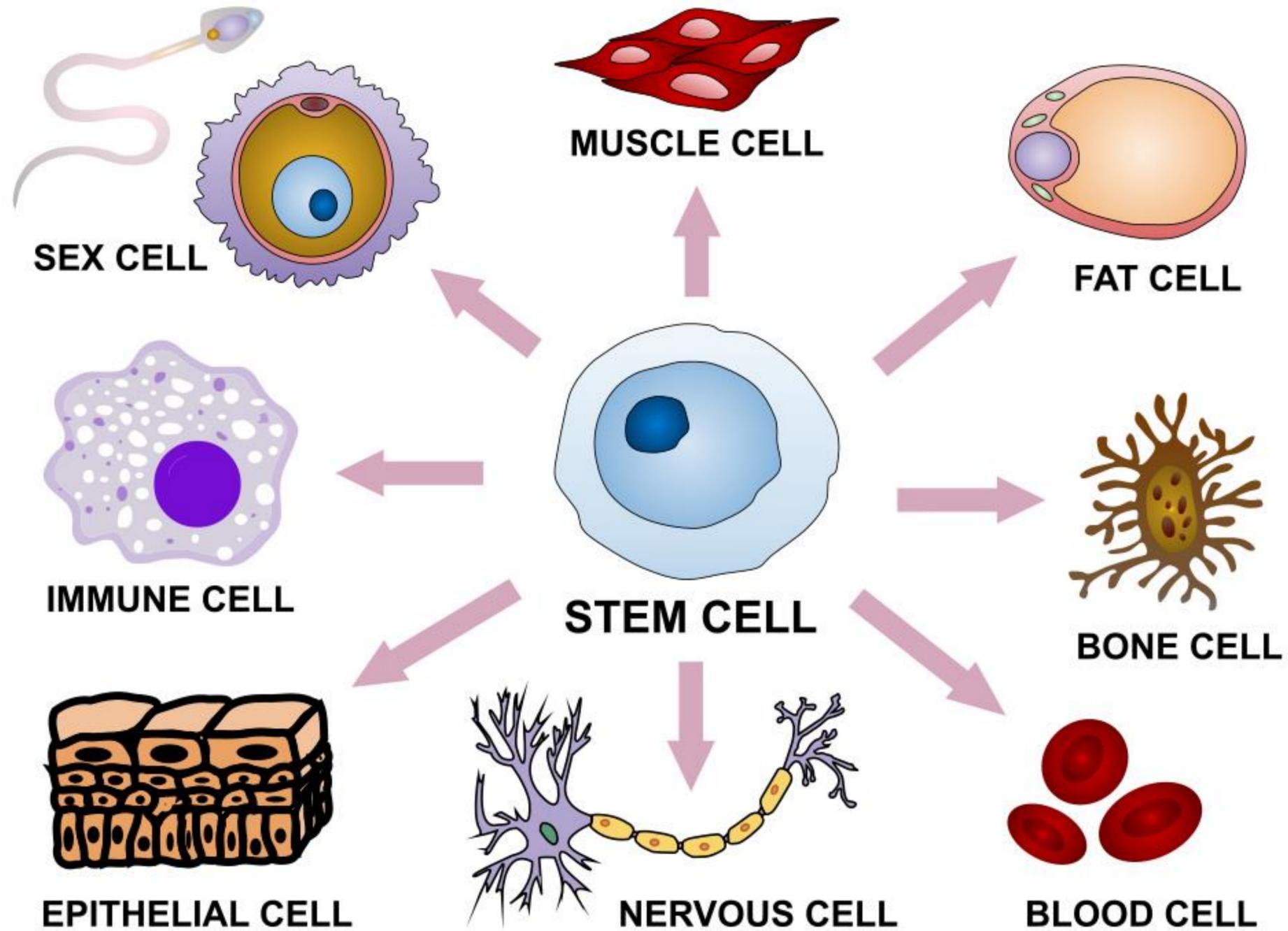
Germline cells

Reproductive cells,
Passed on to future generations

Sperm / Egg



Stem cells



CRISPR is moving fast, but are we?

2015: A research group used CRISPR to make genetic changes in non-viable human embryos

2018: The birth of CRISPR edited twins



Image: ekem (courtesy: RWJMS IVF Program), public domain