

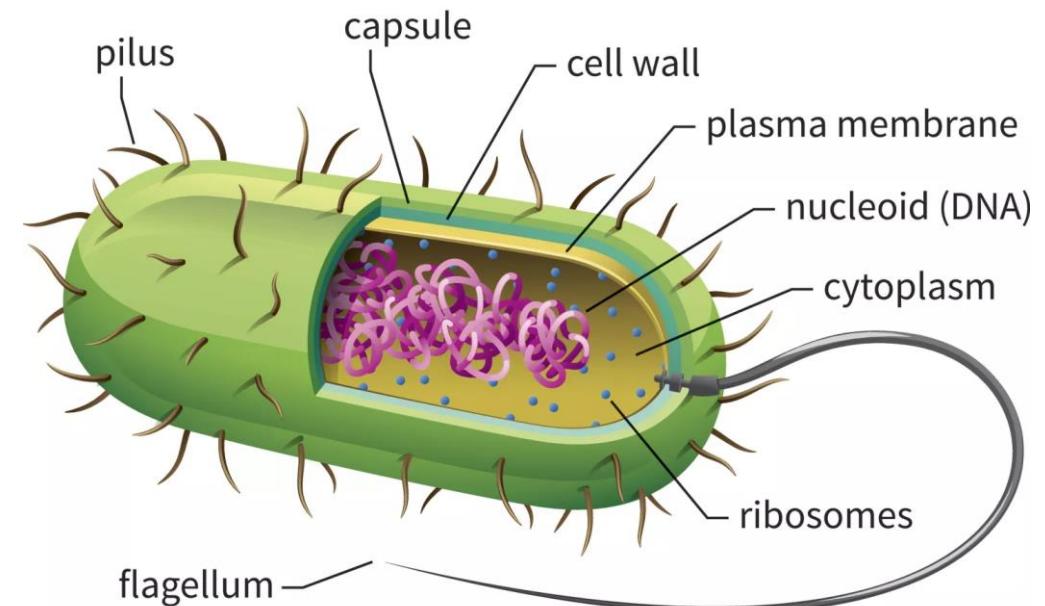
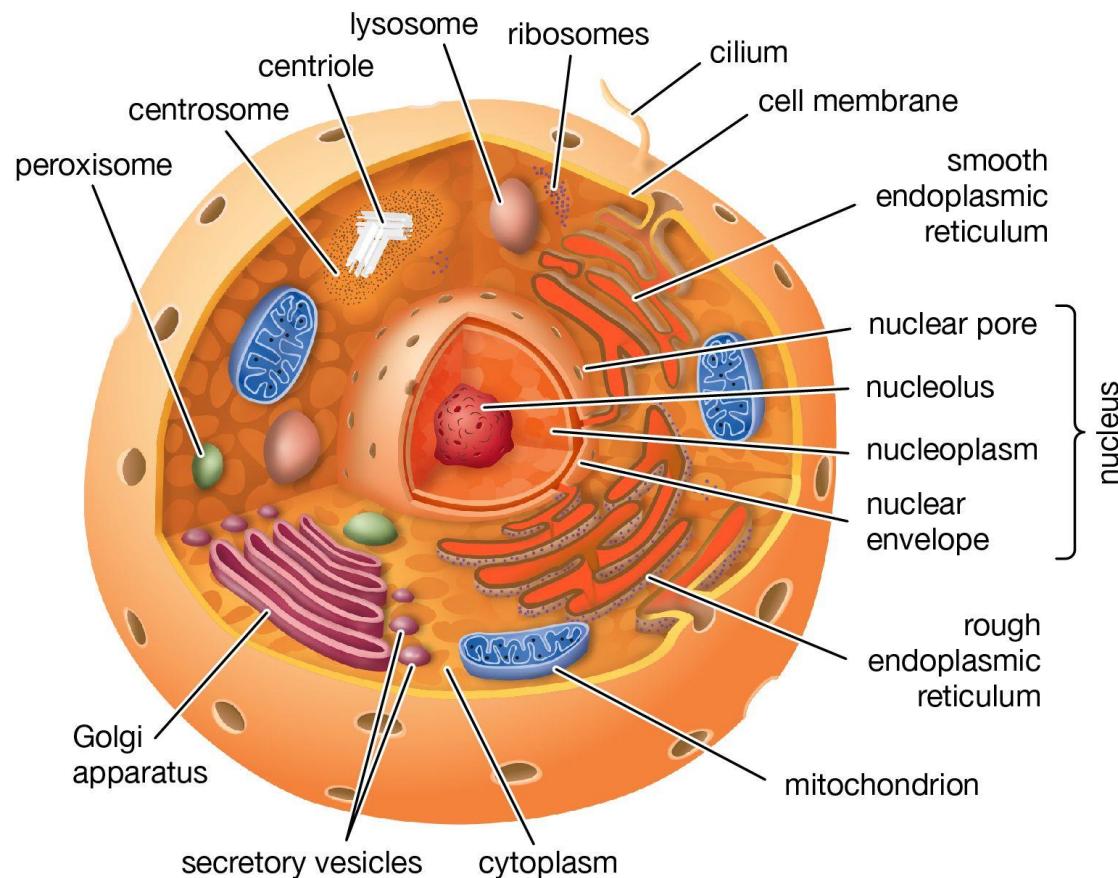
# CRISPR-Cas Genome Editing

Chen Gang Goh  
Graduate Student  
Steve Jackson Lab  
Gurdon Institute, University of Cambridge

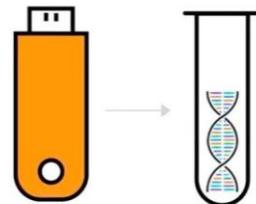
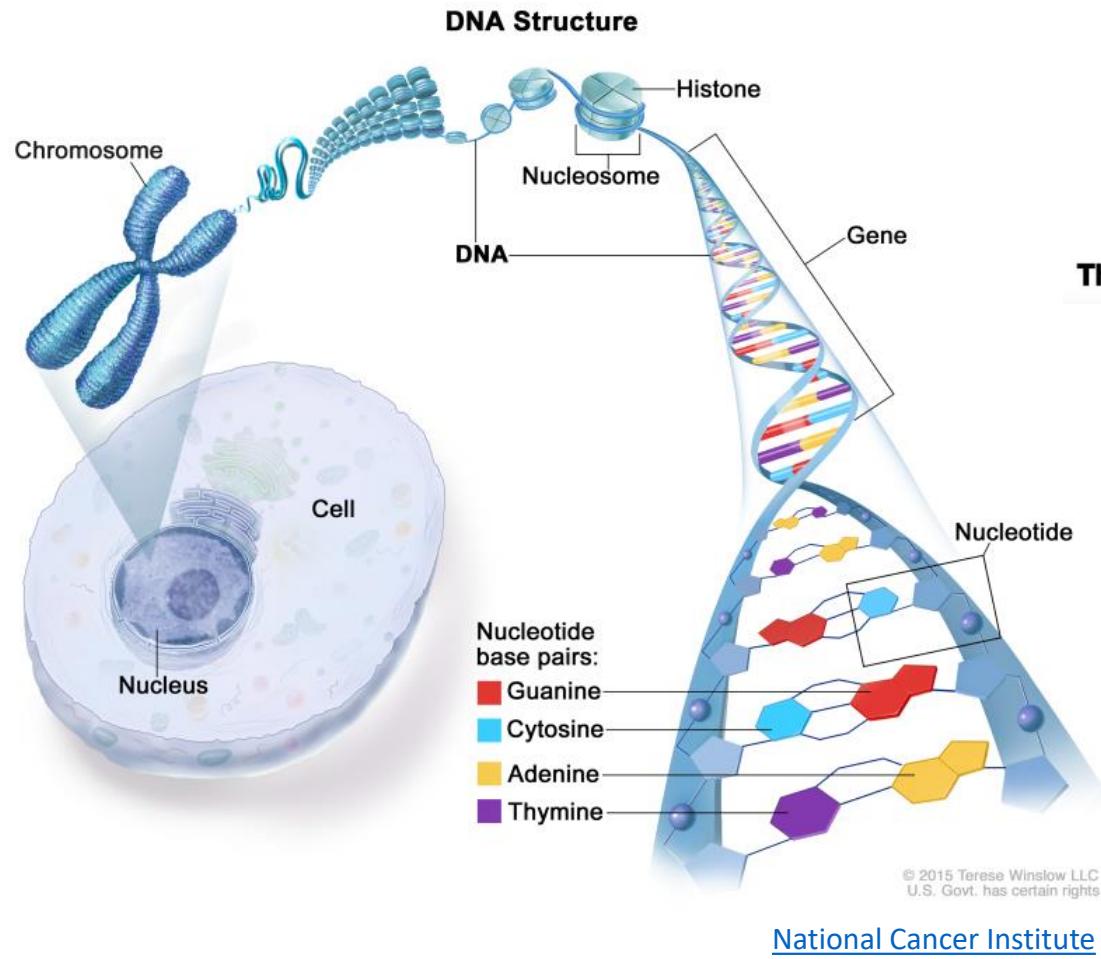
# Part 1: The ‘Central Dogma’

# Eukaryotic cell

Animal cell



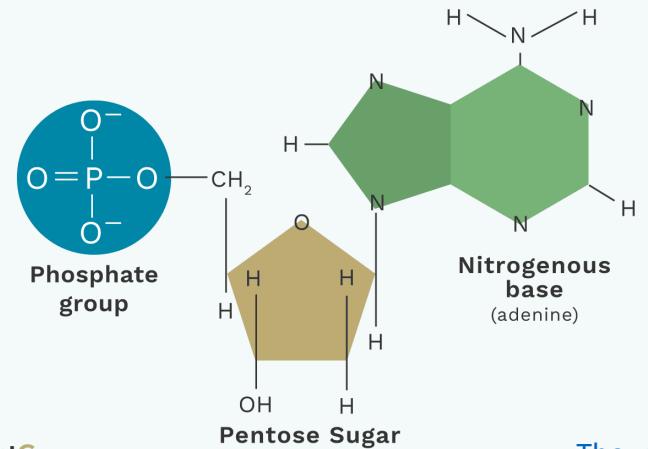
# Nucleus - DNA and chromosome



The Future of Digital Data Storage

[Twist Bioscience](#)

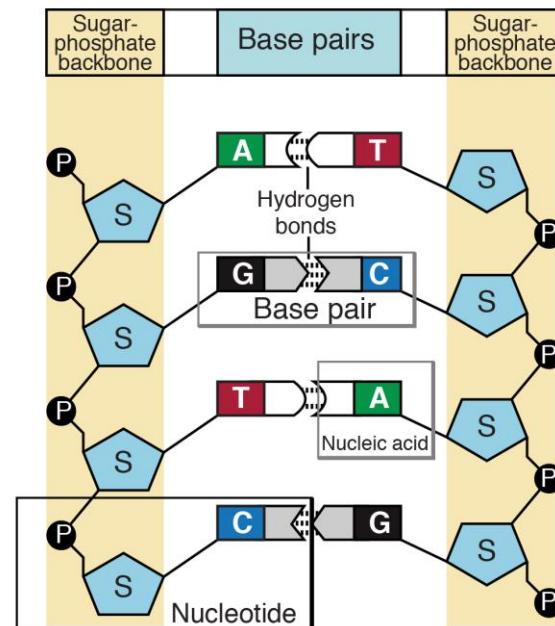
## 3 Parts of a Nucleotide



[ThoughtCo.](#)

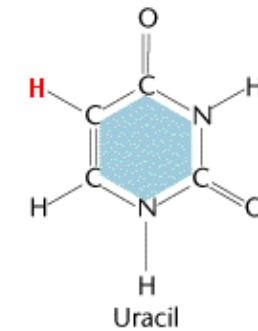
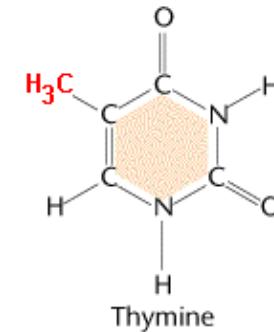
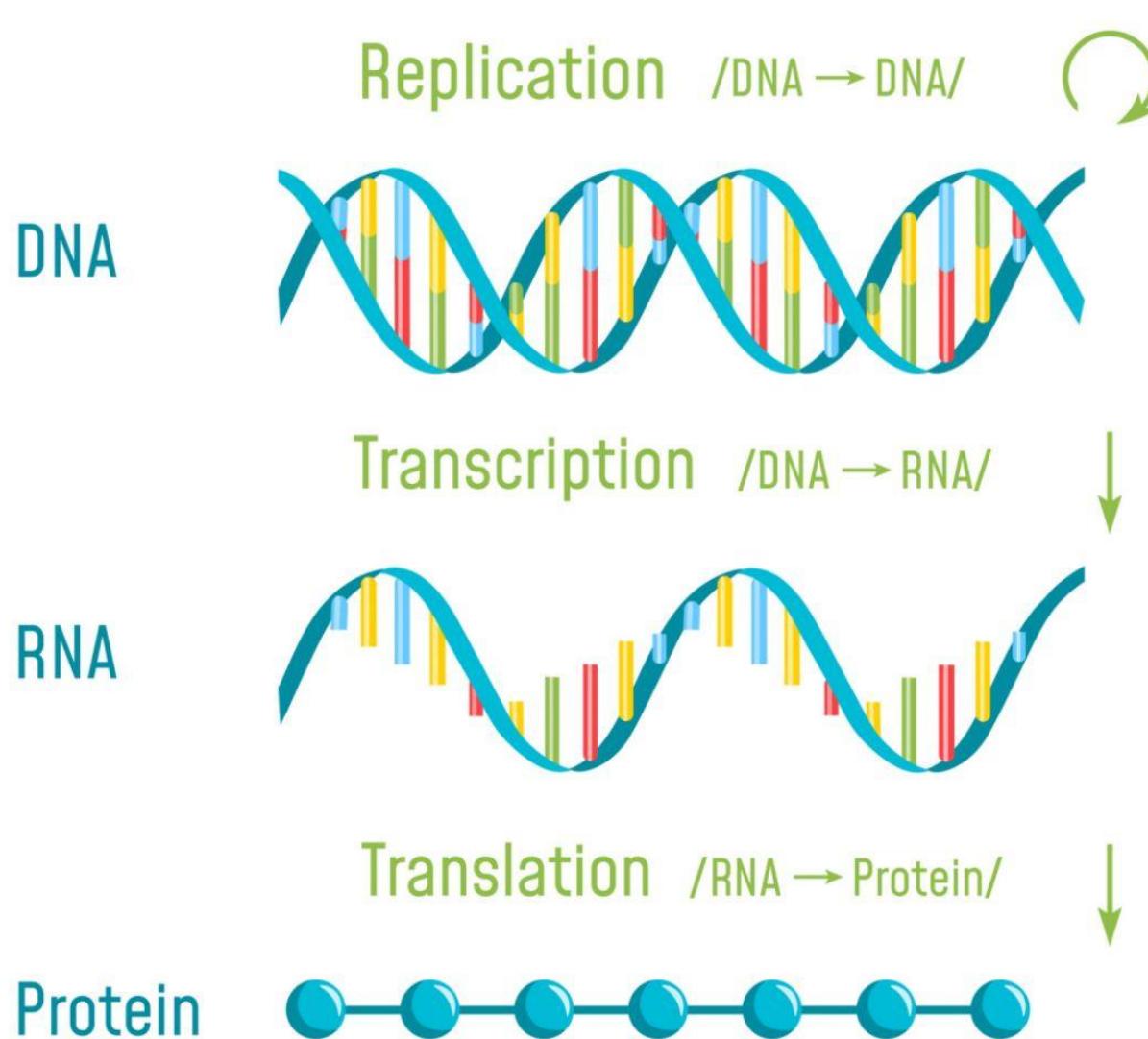
[ThoughtCo.](#)

Deoxyribonucleic Acid (DNA)



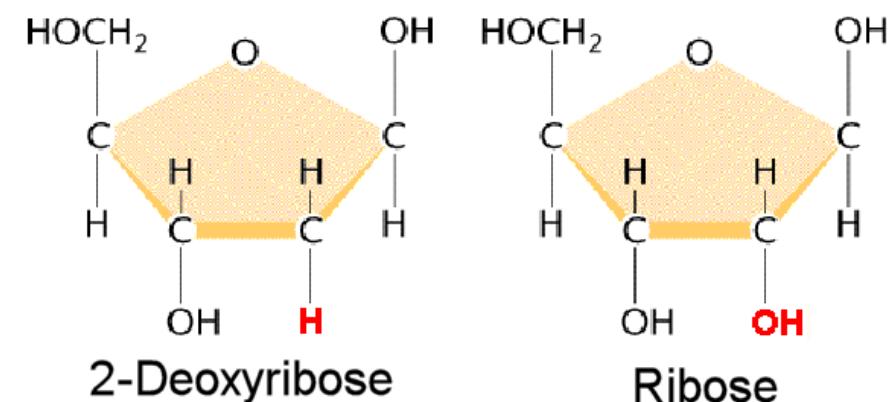
[National Human Genome Research Institute](#)

# The “Central Dogma”



(Klug & Cummings 1997)

[Mun.ca](#)

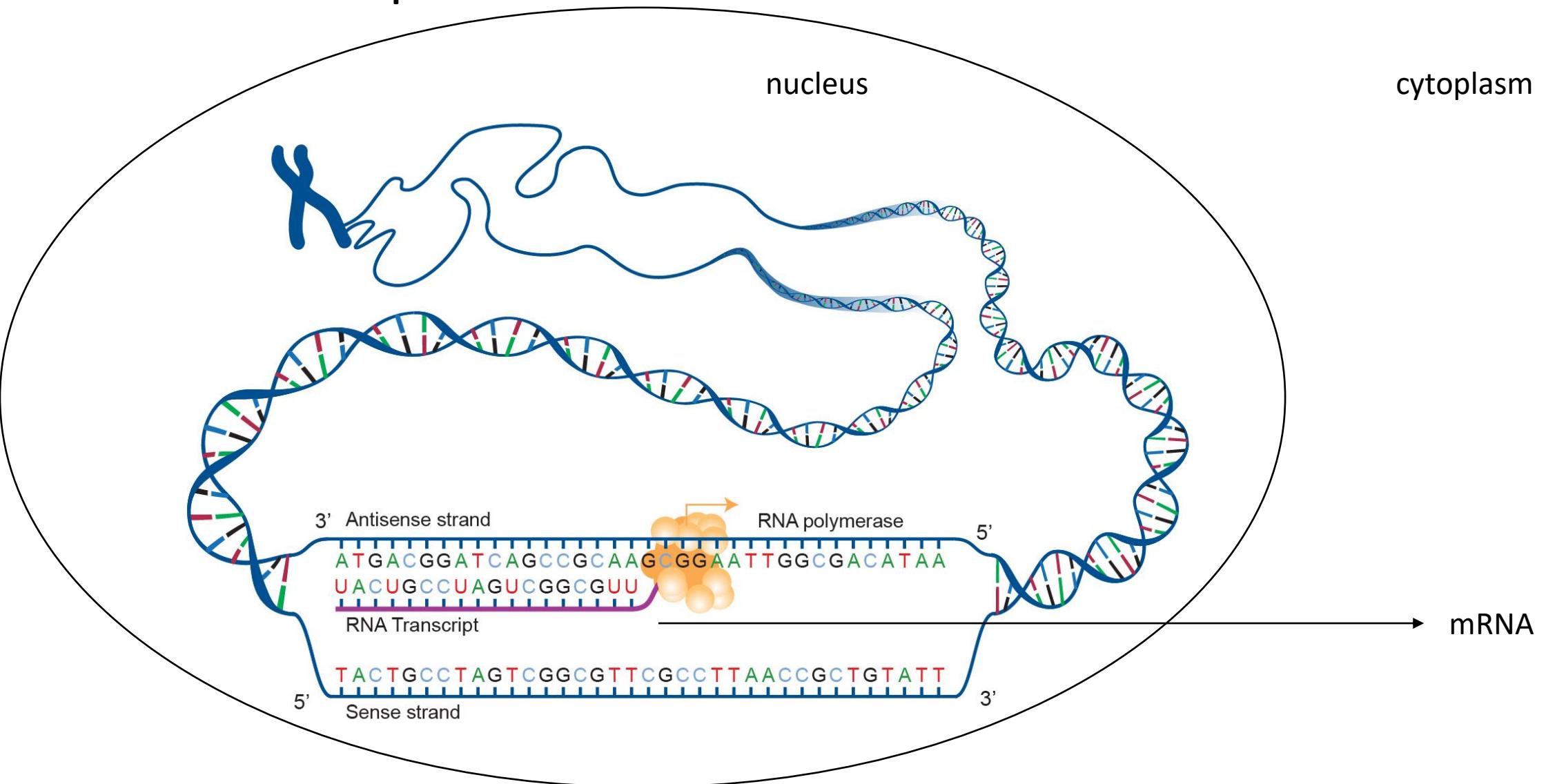


2-Deoxyribose

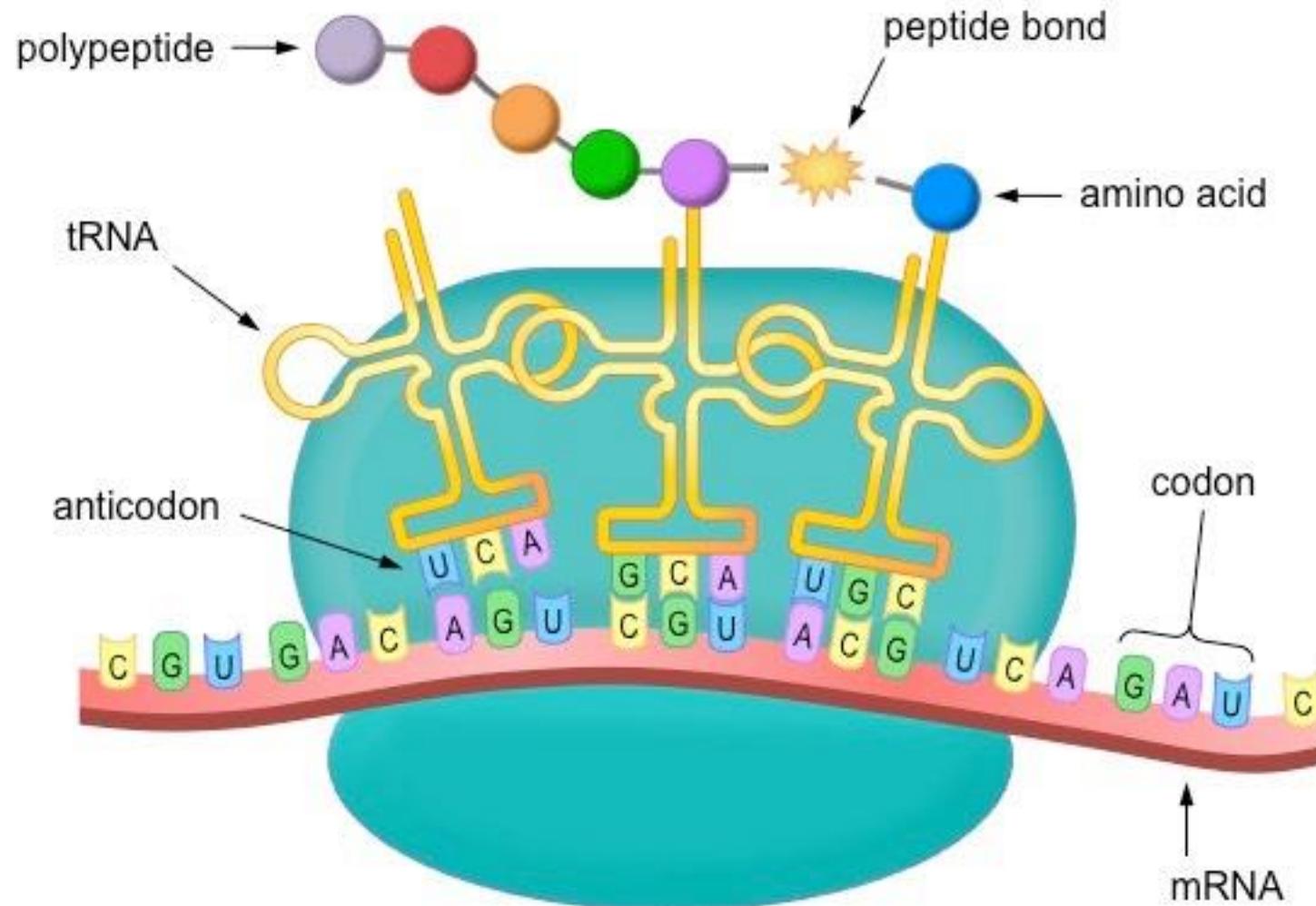
Ribose

[Mun.ca](#)

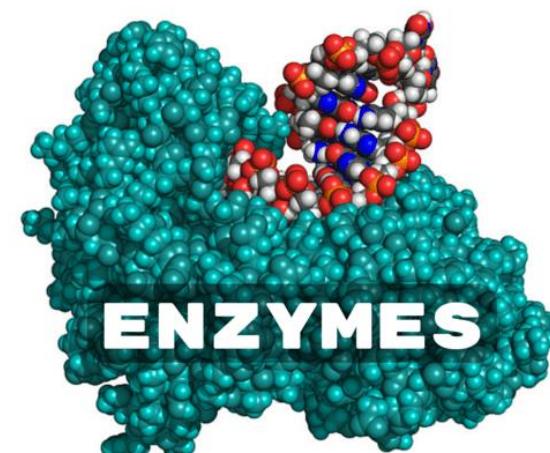
# Transcription



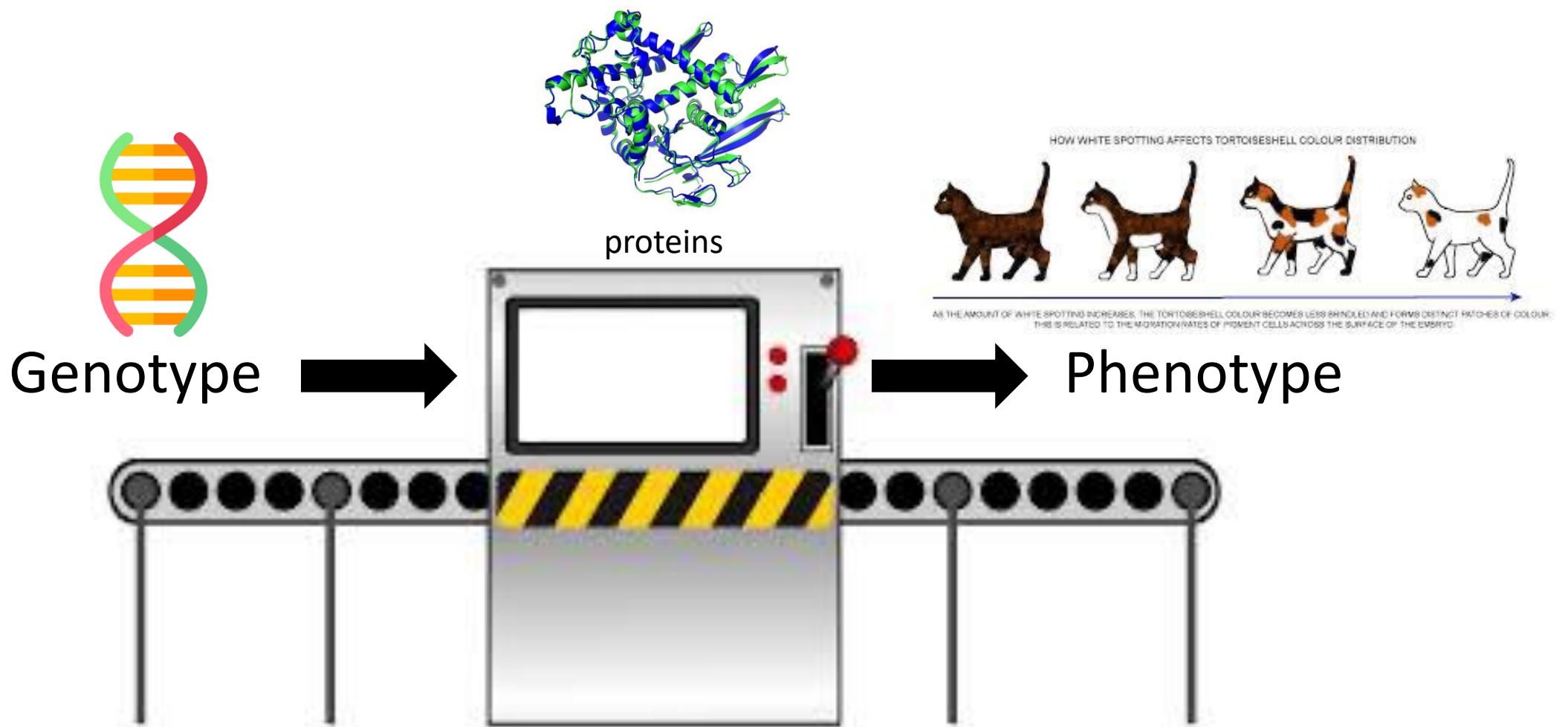
# Translation



[Bioninja](#)



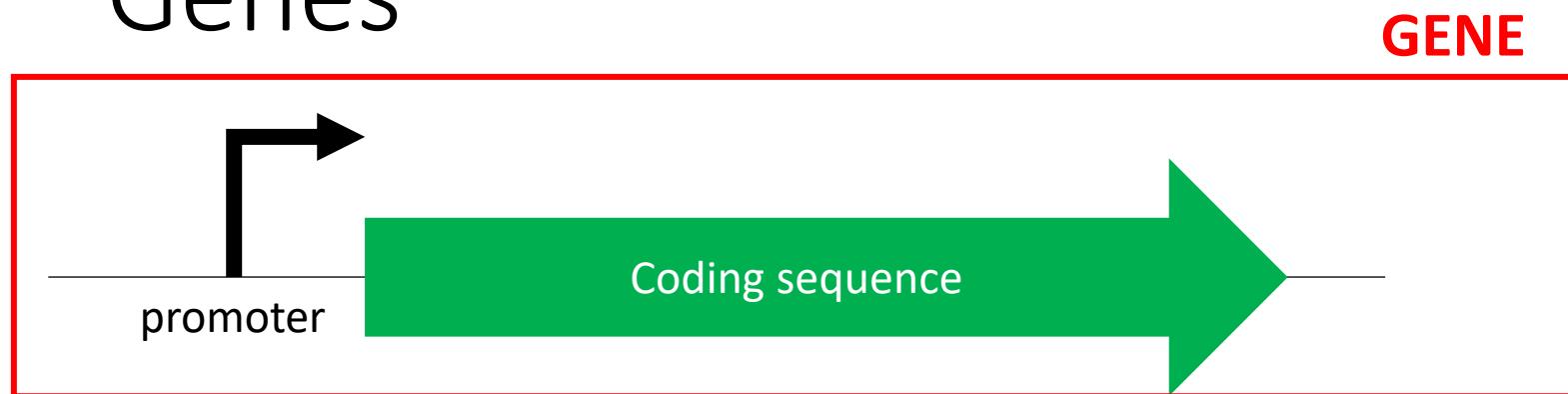
# Genotype VS Phenotype



# Quiz 1

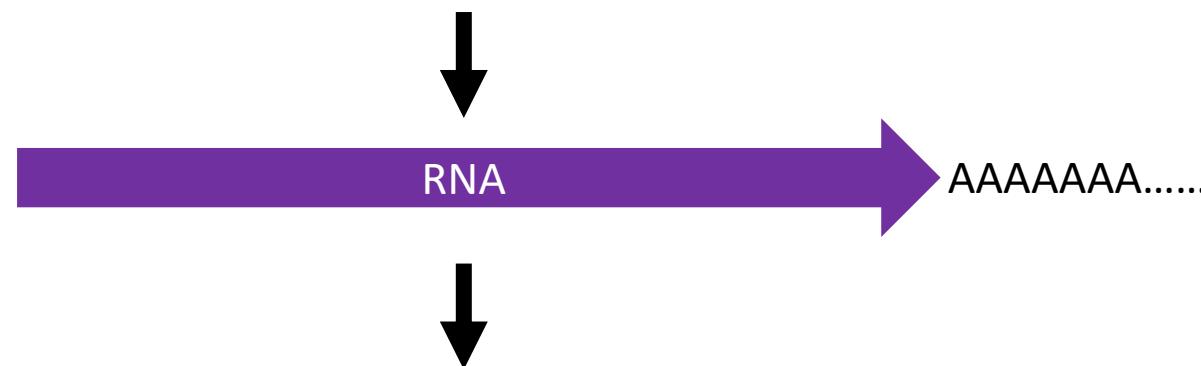
# Part 2: Genes and Diseases

# Genes



**GENE**

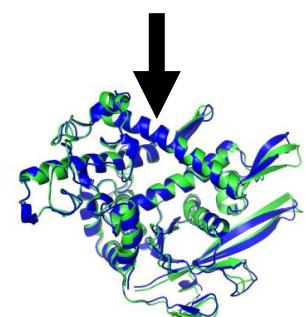
DNA



RNA → **Phenotype**

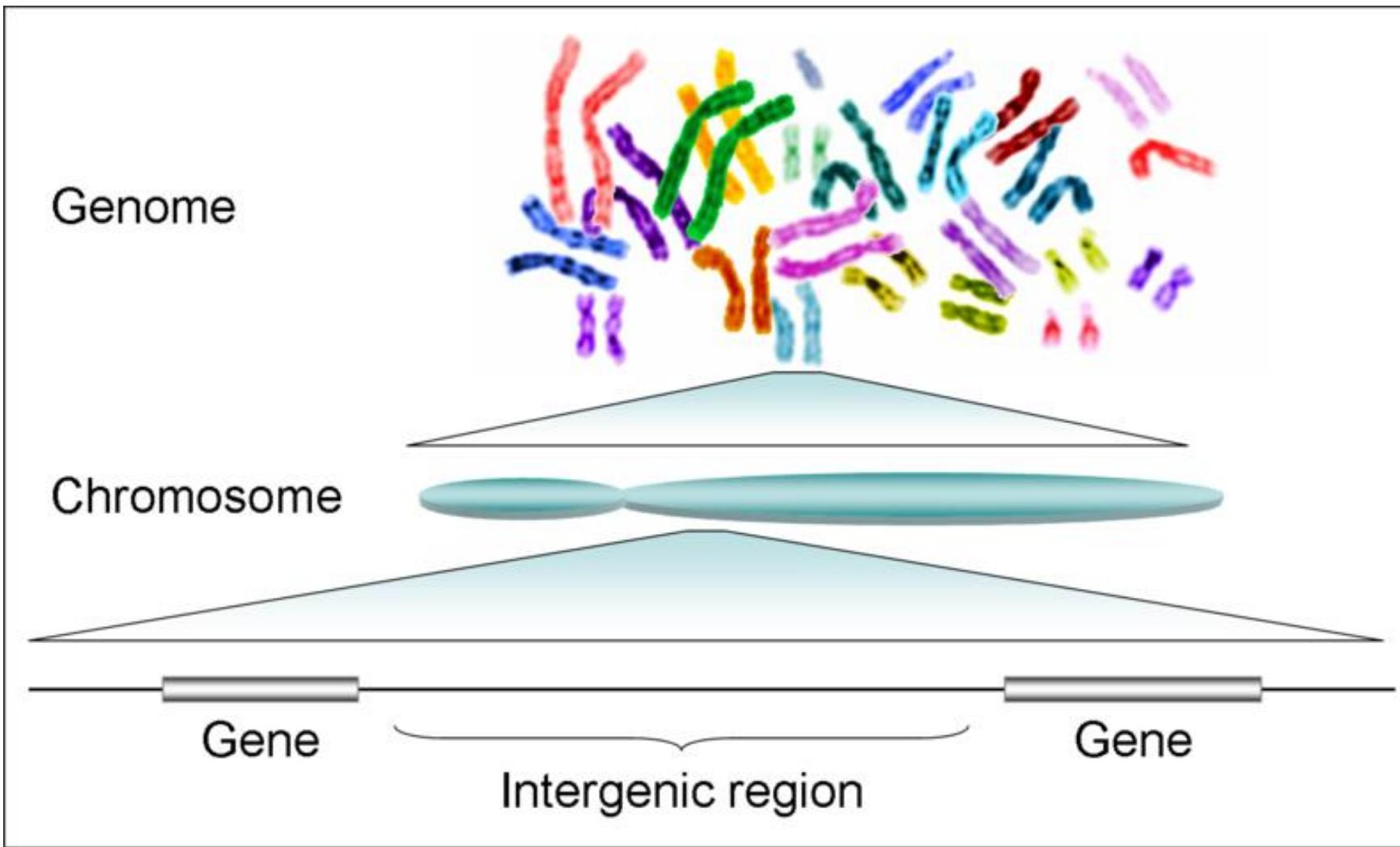


polypeptide



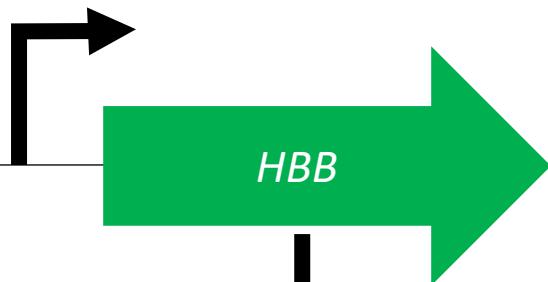
protein → **Phenotype**

# Genome



# Variations in humans – Genetic Disease

## Sickle Cell Disease



*HbA*  
Normal

Variation

*HbS*

Sickle cell

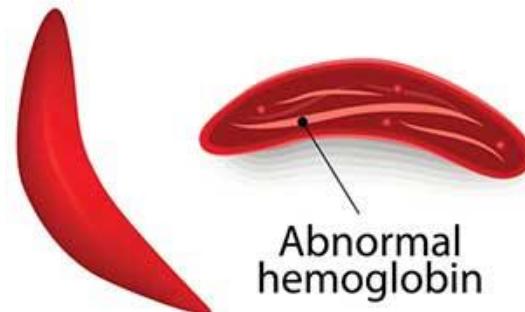
CCT GAG GAG

Pro Glu Glu

CCT GTG GAG

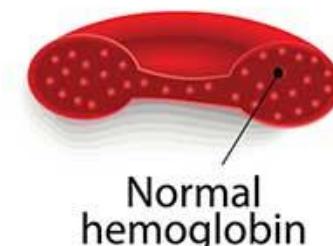
Pro Val Glu

Sickle cell



Abnormal hemoglobin

Normal red blood cell



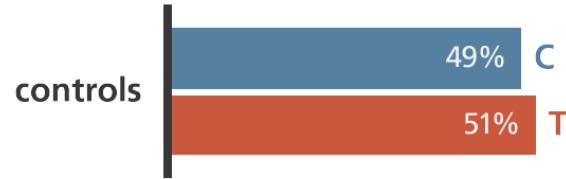
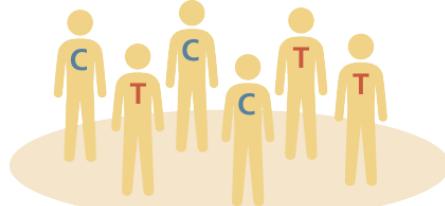
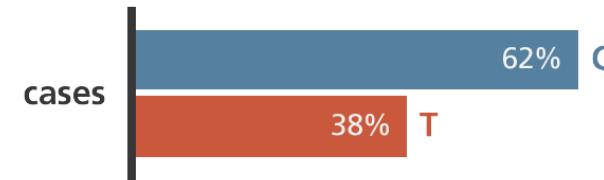
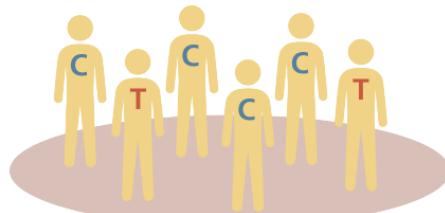
Normal hemoglobin



Froedtert

# Single Nucleotide Polymorphism (SNP)

## Genome-Wide Association Study (GWAS)



[YourGenome](#)



*HbA*  
Normal

CCT GAG GAG  
Pro Glu Glu

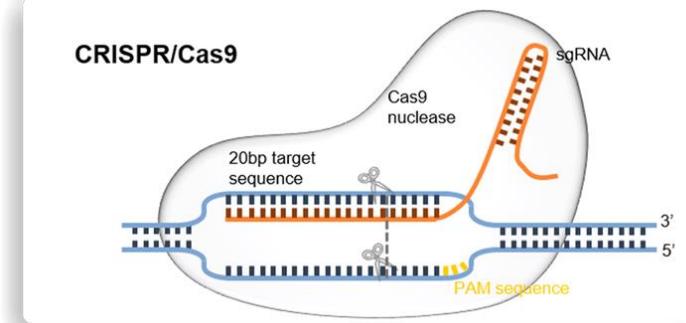
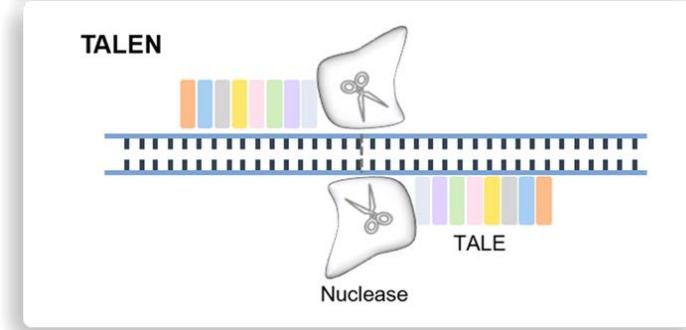
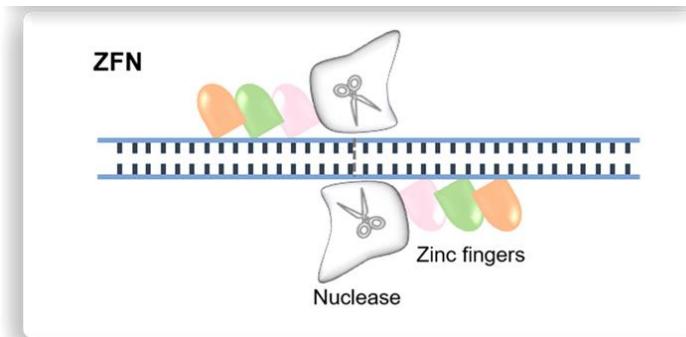
*HbS*  
Sickle cell

CCT GTG GAG  
Pro Val Glu

# Quiz 2

# Part 3: CRISPR-Cas Genome Editing Tool

# DNA “Molecular Scissors”



{

DNA-binding protein modules

{

DNA-binding RNA  
(complementary base-pairing)

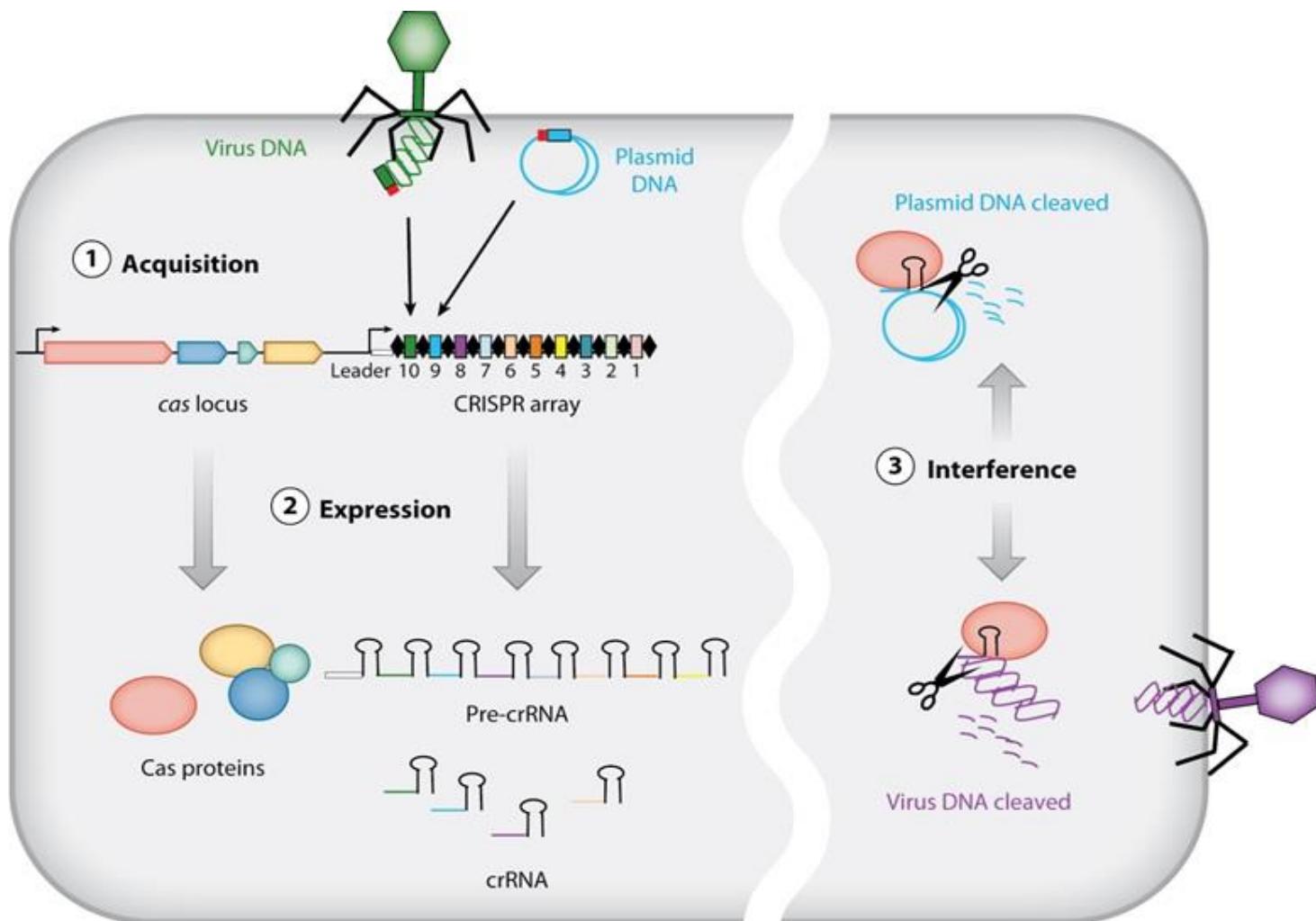
**Zinc Finger Nuclease (ZFN)**

**Transcription Activator-Like  
Effector Nuclease  
(TALEN)**

**Clustered Regularly Interspaced  
Short Palindromic Repeats  
(CRISPR)**

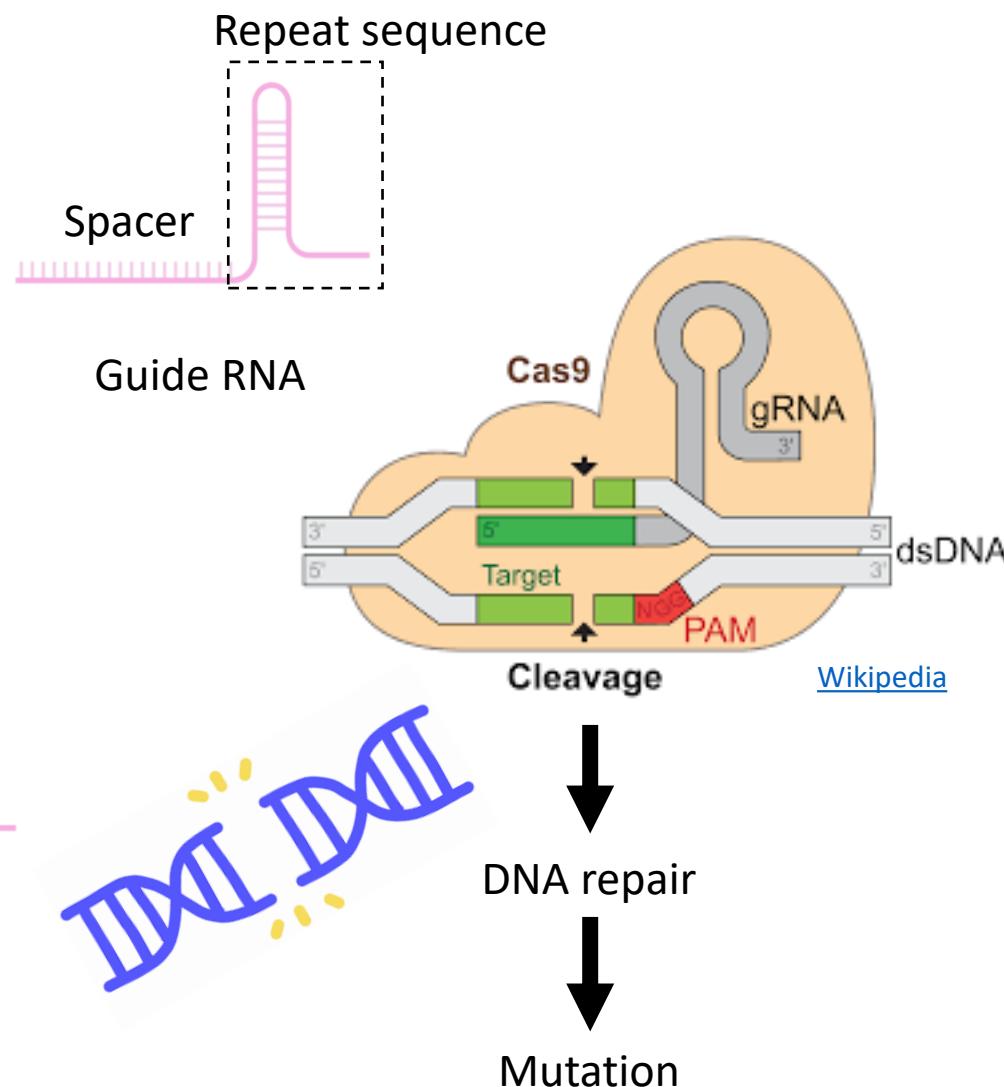
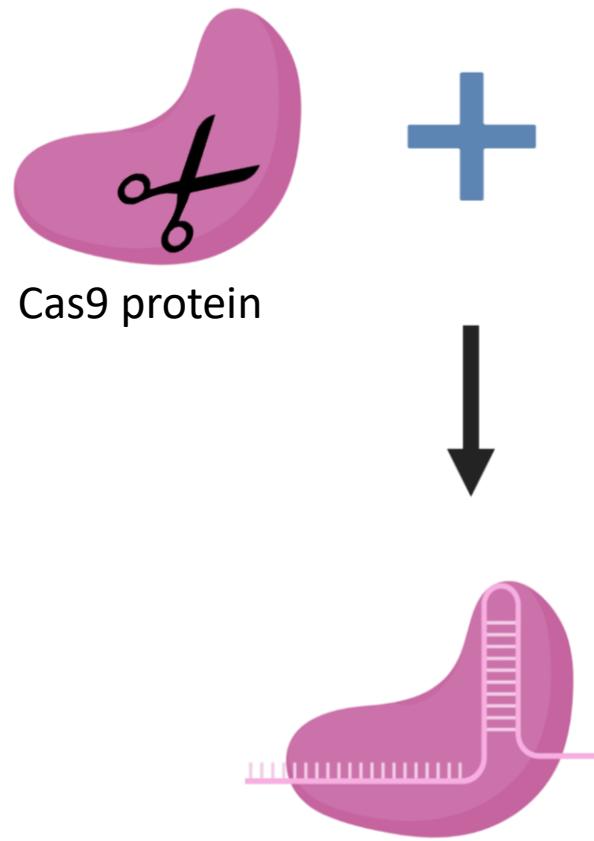
**2012**

# CRISPR-Cas9



- Adaptive immunity in bacteria
- Discovered as early as 1993
- 2 crucial components:
  - Cas locus
  - CRISPR array

# CRISPR-Cas9 as a Genome Editing Tool



Jennifer Doudna & Emmanuel Charpentier



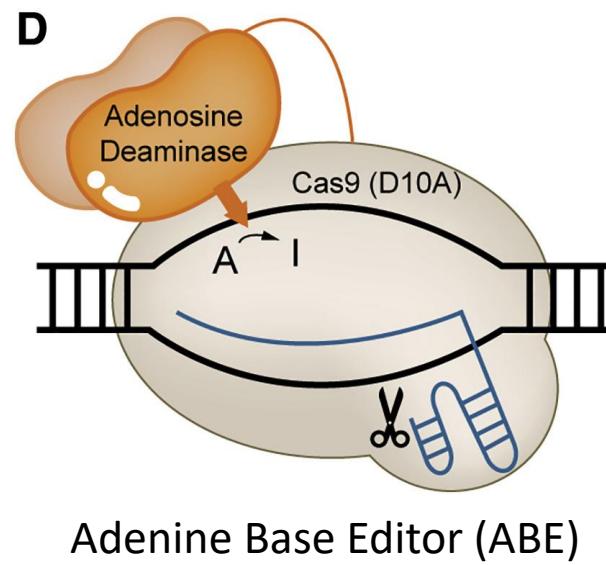
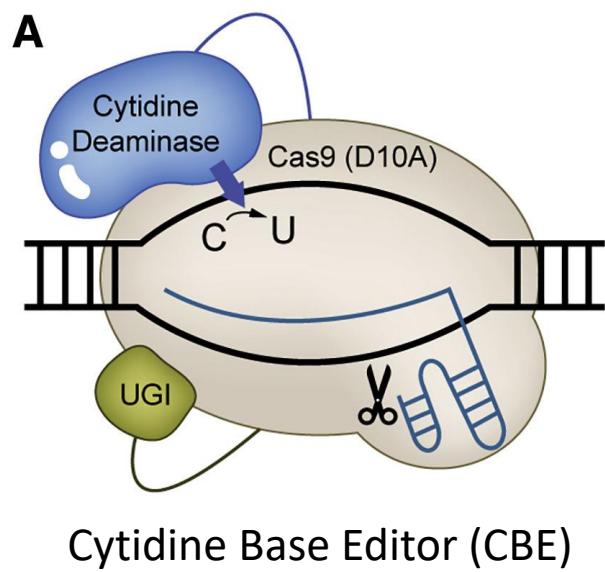
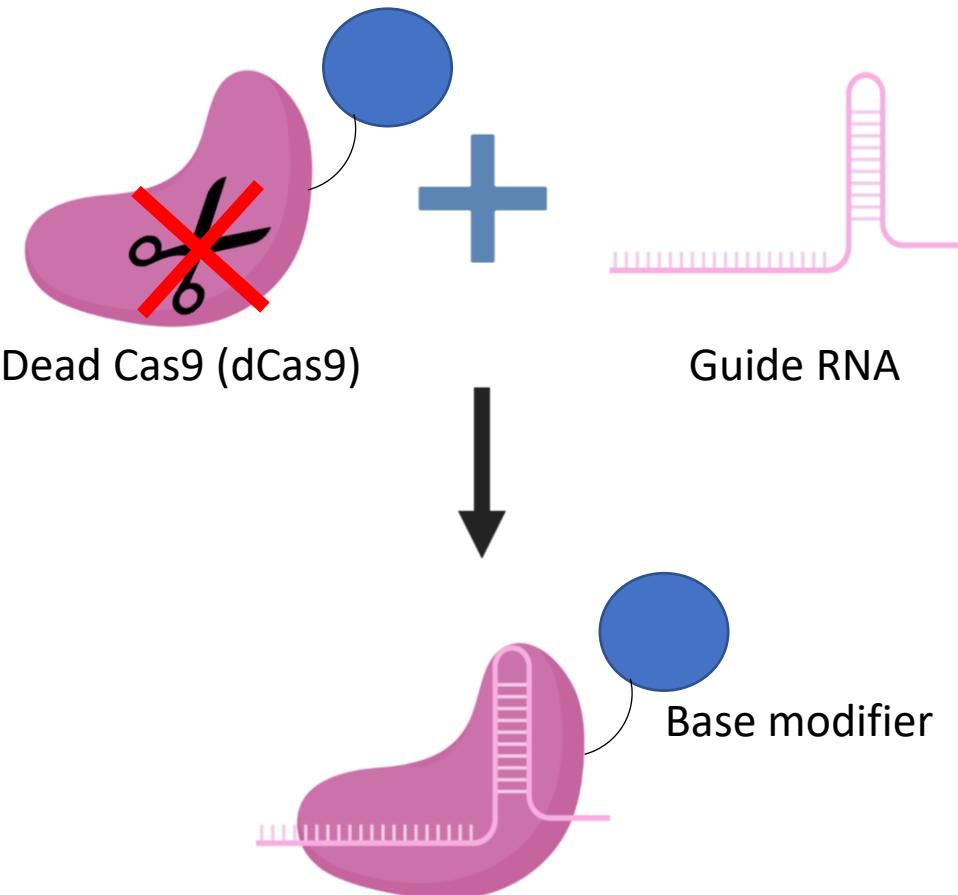
Nature

**Nobel Prize in Chemistry 2020**

# DNA Base Editor



Alexis Komor



**Targeted, specific base editing**

# Quiz 3

# Part 4: Ethics and Safety of CRISPR-Cas

# Health VS Aesthetic



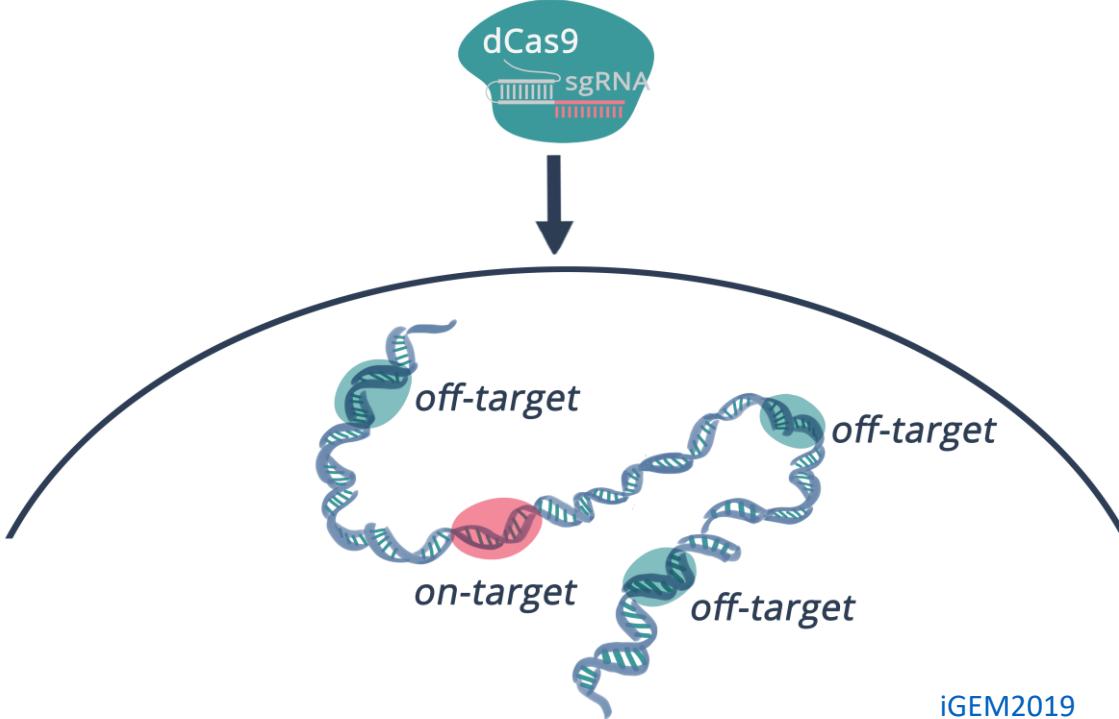
Health Improvement

VS

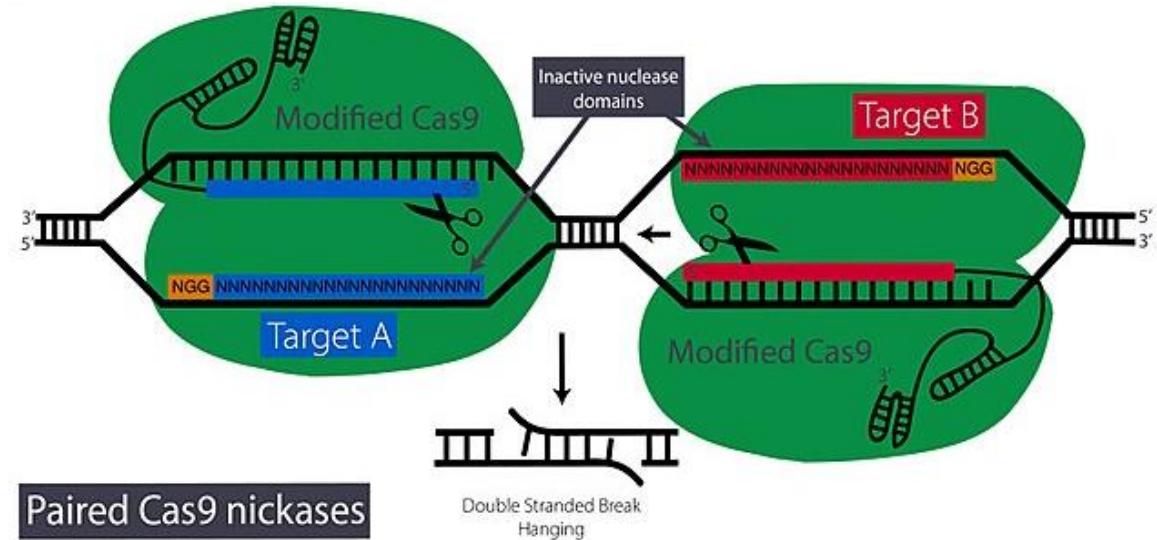


Aesthetic Enhancement

# Safety concerns



iGEM2019

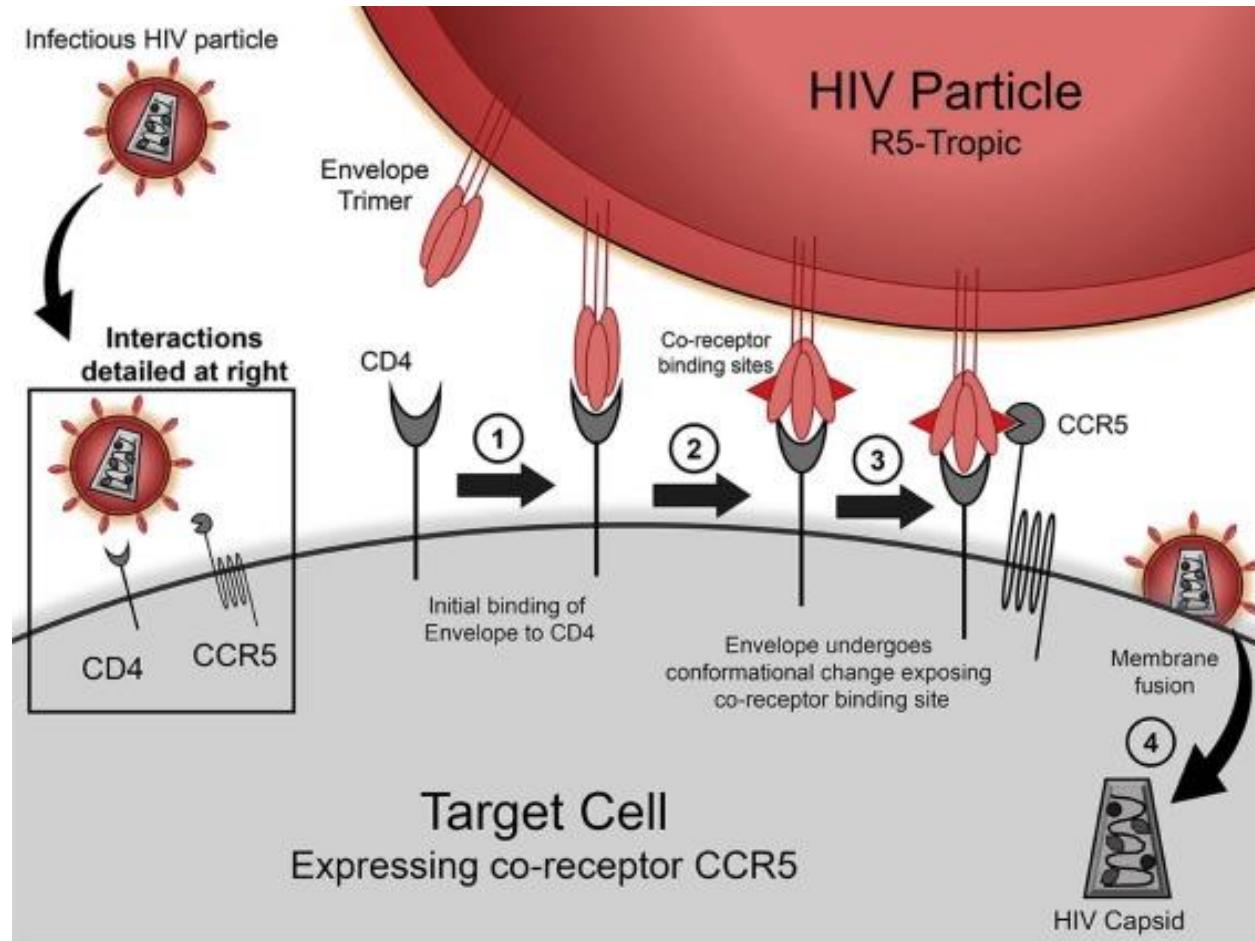


Off-target effects

Delivery of genome editing tools

Genome editing in germline

# World's first gene-edited babies – Lulu & Nana



He Jiankui

[Wikipedia](#)

Disrupt *CCR5* gene to confer innate resistance to HIV infection

# Summary Video



# Potential of CRISPR in Genetic Engineering

