Alnylam® PHARMACEUTICALS

RNAi Science Activity Booklet

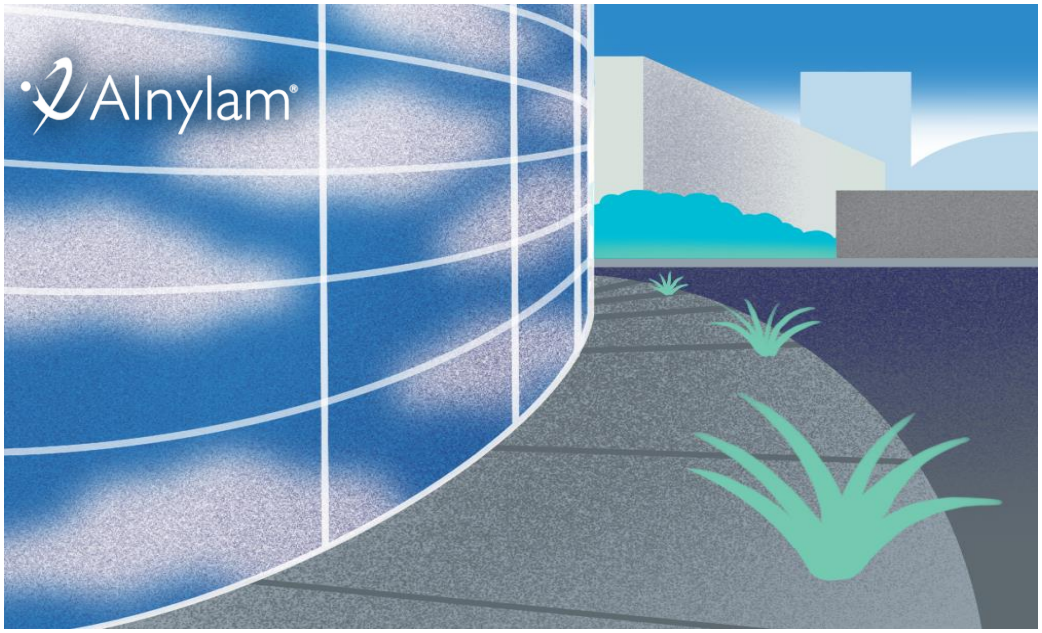
2026 Edition

ULearn with Alnylam® PHARMACEUTICALS

About Alnylam

Alnylam Pharmaceuticals has led the translation of RNAi (RNA interference) from Nobel Prize-winning discovery into an entirely new class of medicines. Alnylam's RNAi therapeutics make it possible to target and "silence" the production of genes that cause specific diseases.

In 2018, our first medicine, ONPATTRO® (patisiran), became the world's first approved RNAi therapeutic. Since then, five more Alnylam-discovered medicines – GIVLAARI® (givosiran), OXLUMO® (lumasiran), Leqvio® (inclisiran) and AMVUTTRA (vutrisiran) have been approved by the U.S. FDA. We are advancing a robust pipeline of innovative RNAi-based medicines in four therapeutic areas: genetic medicines, cardio-metabolic diseases, hepatic infectious diseases, and central nervous system (CNS) and ocular diseases.



What's in a Name?

AL-NY-LAM. Our name may not be the easiest to pronounce, but once you learn it, you'll never forget it. Alnylam is derived from "Alnilam," the center star in Orion's belt, which has been used by explorers for navigation for thousands of years. It is also loosely translated to "string of pearls" in Arabic.

The Discovery of RNAi



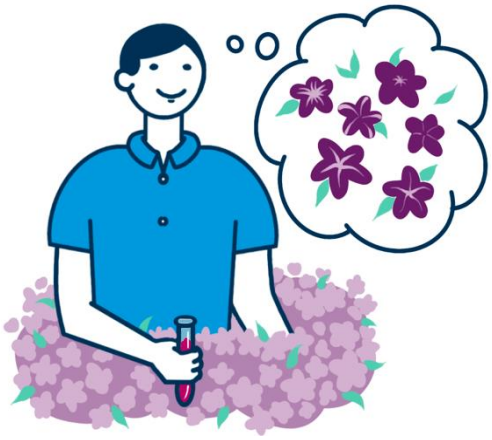
At Alnylam, we each play a role in making medicine for sick patients. But you might be surprised how the story of RNAi began…

It all started with a flower...
A purple petunia.





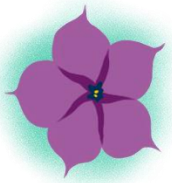
There was a scientist who studied flowers.
He wanted to make his petunias more purple.



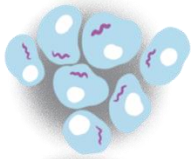
He found out which **gene** gave the petunias their purple color. He made a lot of this gene in his lab to give to his petunias, to make them very, very purple.

The Discovery of RNAi

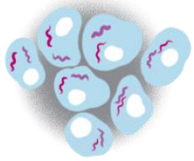




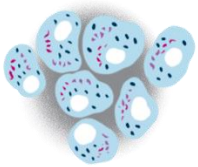
The scientist went back into his lab to understand why his petunias turned white instead of purple.



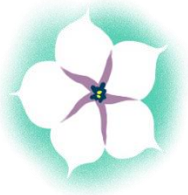
The **cells** of the purple petunias had genes that caused their petals to have a purple color.



When the scientist gave the petunias genes that were made in his lab, those genes looked slightly different.



The cells thought the new genes were dangerous. So they destroyed all the color genes, and in the process destroyed their own.



Since all of the purple genes were destroyed, the petunias petals turned white. This is an example of RNAi. (i = interference)

The Discovery of RNAi

Scientists around the world were fascinated by the petunias turning white. They wondered if other genes besides color would also change. They tested it on plants and animals. Then, they wondered if this would work in people…





The scientists at Alnylam found a gene in humans that causes disease. They invented a medicine, based on this gene, that can treat this disease.

The Discovery of RNAi



This scientist wants to create colorful flowers. For each flower, she mixes two gene colors to create a new color. Help her color in each flower with the new color!











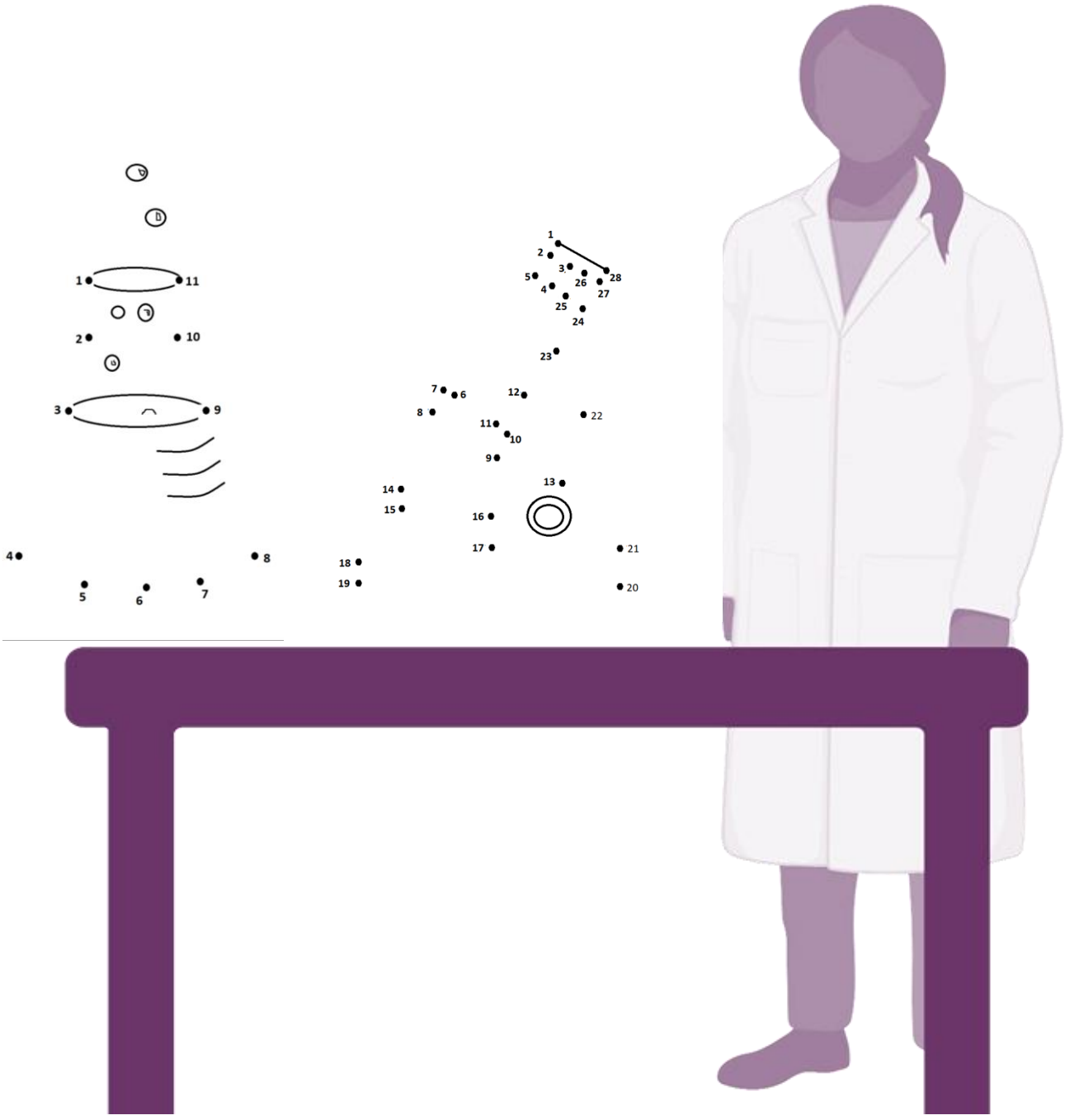


1. Green 2. Purple 3. Orange

Science is Fun and For Everyone!

Do you want to learn about how things work?
Do you want to learn how our bodies function?

You are a Scientist! Scientists ask questions about the world around them, and anyone can be a Scientist!



See answers on page 20

Science is Fun and For Everyone!

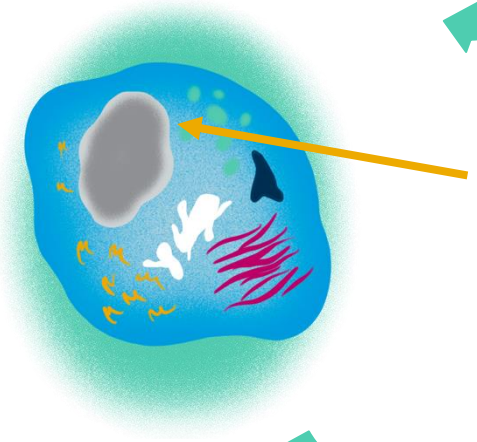
What is
RNA
and why is it important?



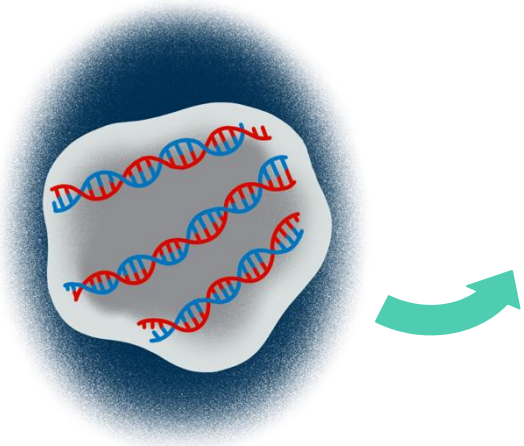
Our bodies are made up of **cells**.



There are many cell types: hair cells, blood cells, skin cells, muscle cells, bone cells…



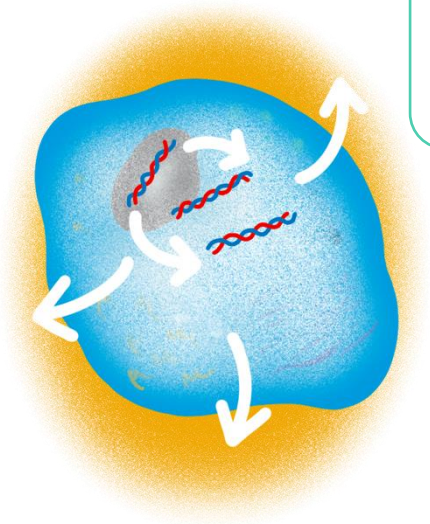
Inside every single cell, there is a **nucleus**.

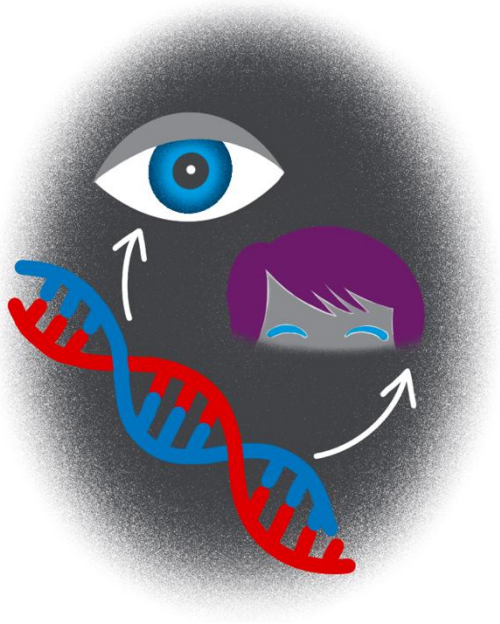


Inside every nucleus, there is DNA & RNA, or **genes**. Every person's genes are unique only to them.

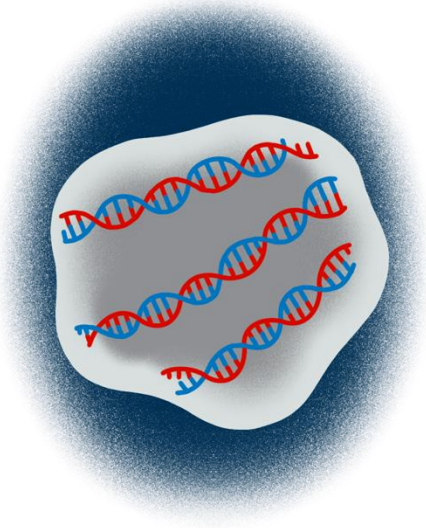
Science is Fun and For Everyone!

DNA copies its code into **RNA**, which leaves the nucleus to instruct the cells of our unique traits.



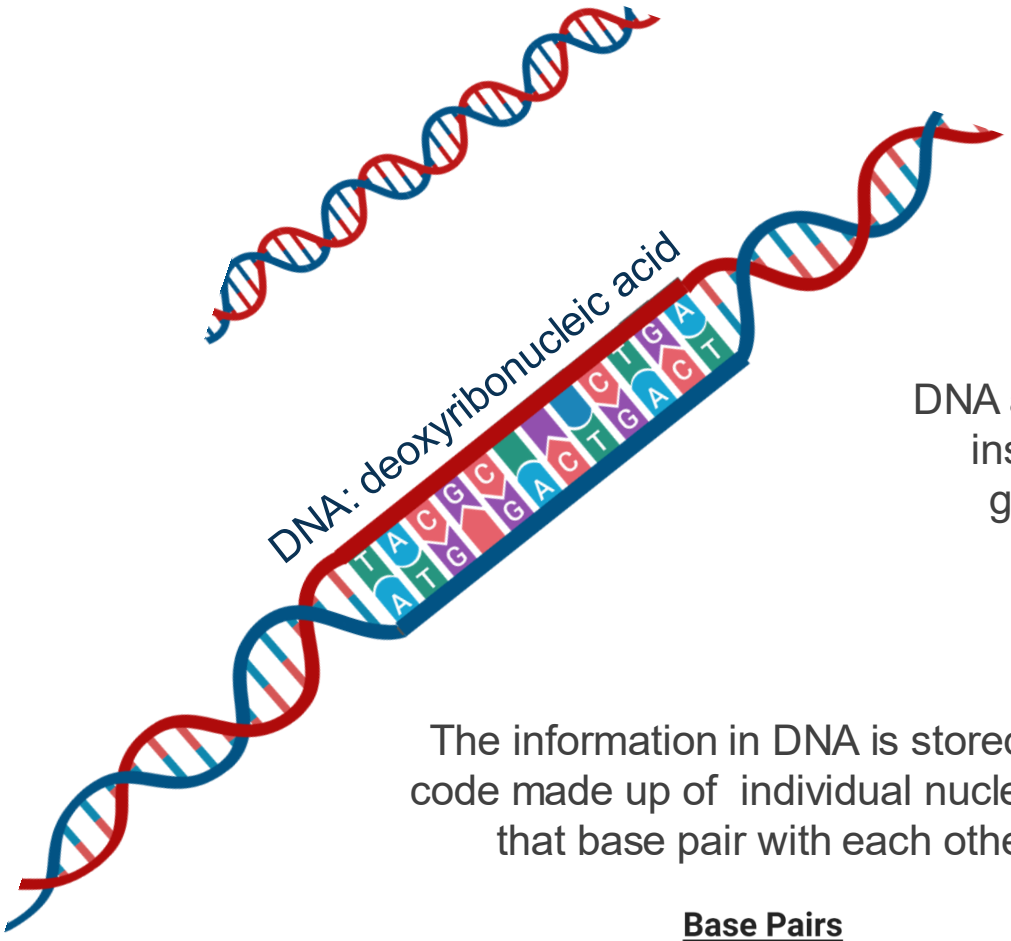


DNA contains genetic code such as eye color, hair color, height, etc.



Inside every nucleus, there is DNA & RNA, or **genes**. Every person's genes are unique only to them.

What is RNA/DNA Made of?



DNA and RNA carry the genetic instructions for how our cells grow, develop and function.

The information in DNA is stored as a code made up of individual nucleotides that base pair with each other.

Base Pairs

adenine A U uracil

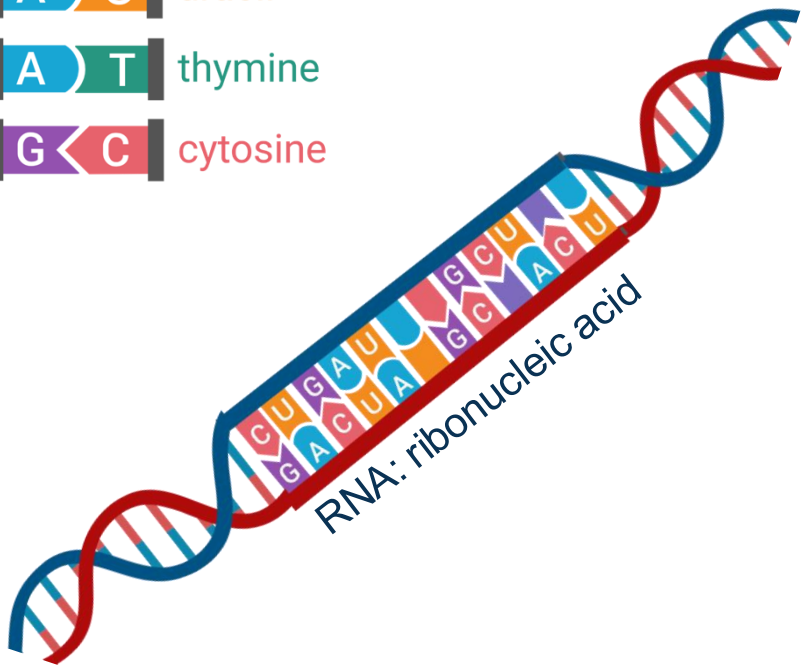
A T thymine

guanine G C cytosine

Challenge

Can you fill in the missing nucleotides?

What's the difference between RNA and DNA base pairs?



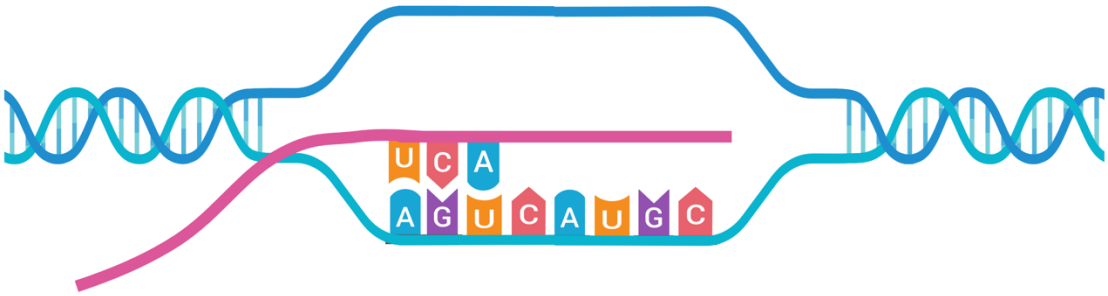
See answer on page 21

DNA top: T, G, A,Bbottom: C RNA top: A, C, G, A Bottom: U, G
RNA contains Uracil and DNA contains Thymine

Transcription

Transcription is the process by which the information in a strand of DNA is copied into a new molecule of messenger RNA (mRNA).

The double helix of DNA (blue line) has opened to allow transcription of mRNA (pink line). The bases on the new strand are added one at a time in the appropriate pairings. Can you fill in the next bases on the pink line?



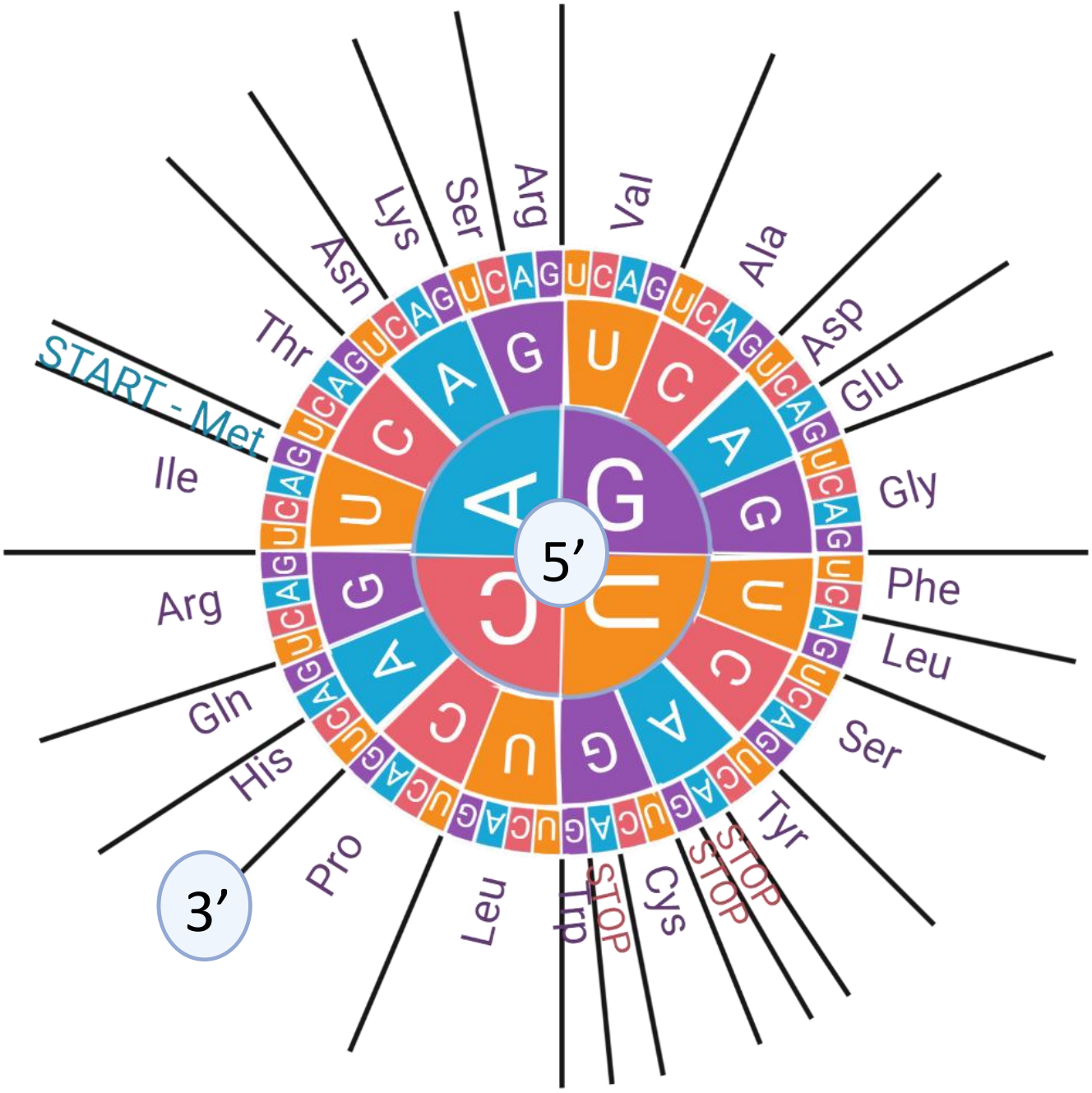
Uh-oh! This strand of mRNA isn't following the instructions from the DNA! Can you circle the incorrect base pairing? (hint: there are 4 mistakes)



See answers on page 22

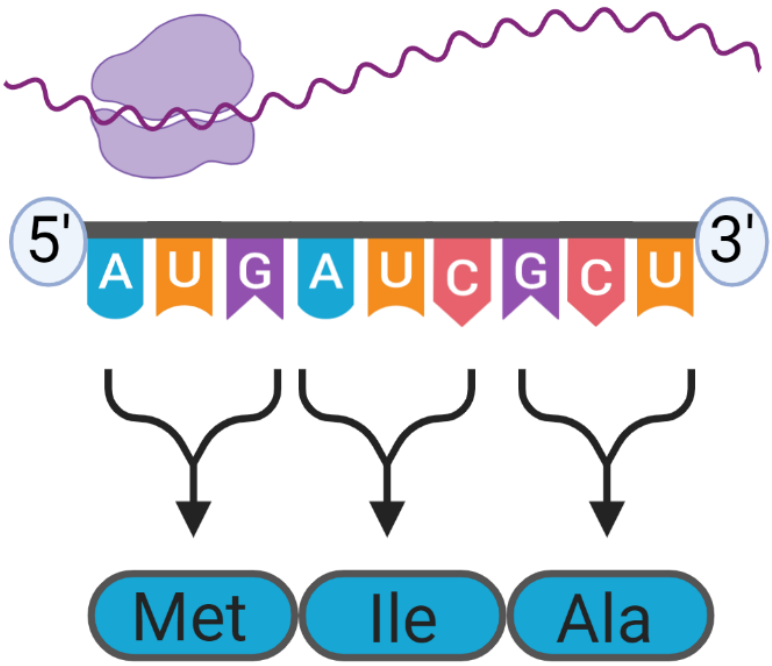
What is Translation?

In translation, mRNA is decoded into amino acid chains.

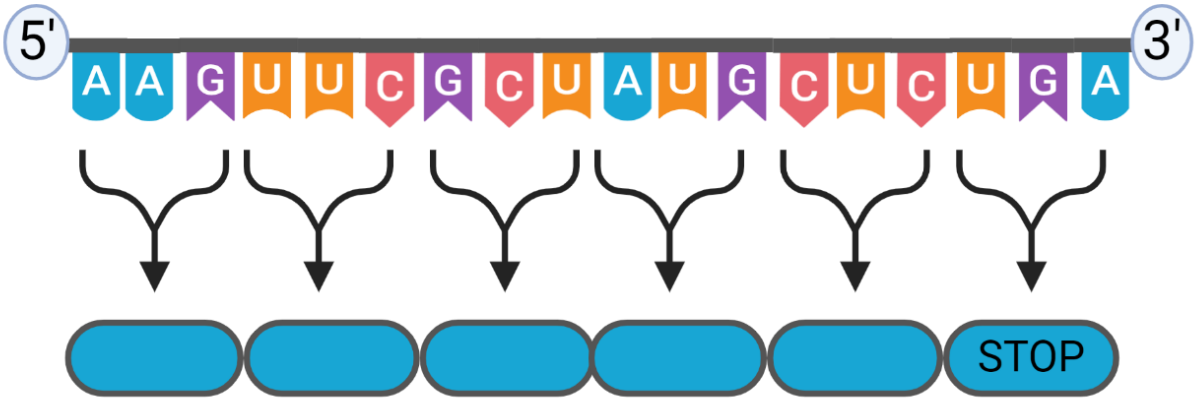


This wheel is like the "decoder" used by our ribosomes (molecular machinery) during translation. Every three nucleic acids are translated into a single amino acid!

mRNA Decoder



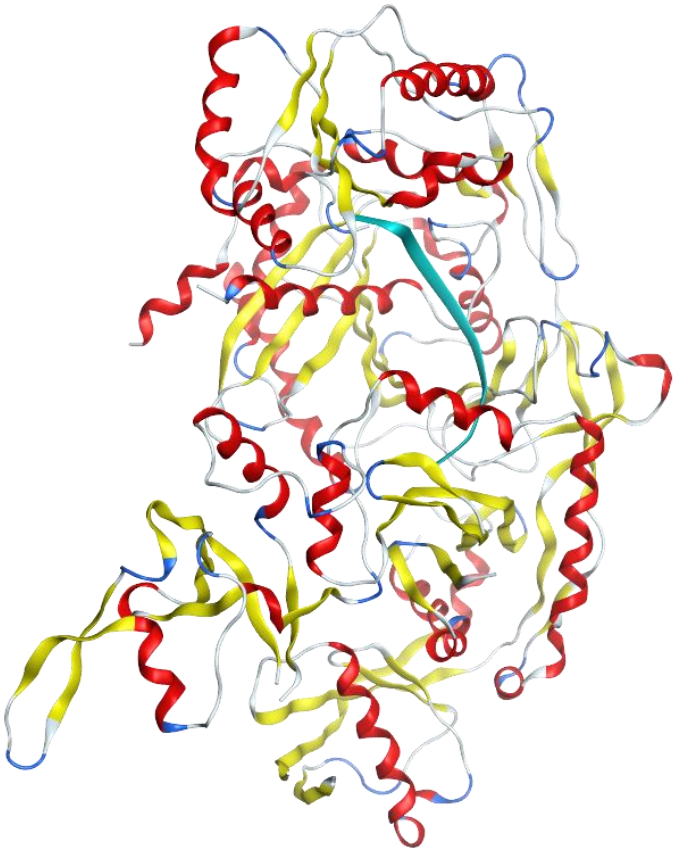
Can you use the decoder to determine the amino acids created by this mRNA sequence?

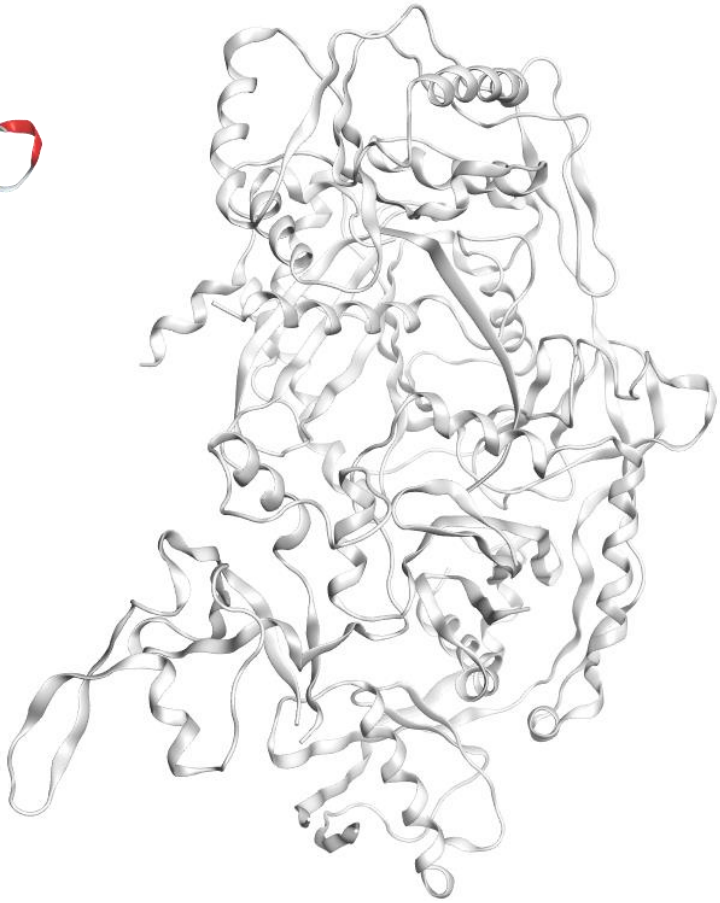


Answer: Lys-Phe-Ala-Met-Leu-

Color a Protein

The order and shape of their amino acid building blocks, give proteins a secondary structure! Can you color the secondary structures on next page?







Beta Sheets

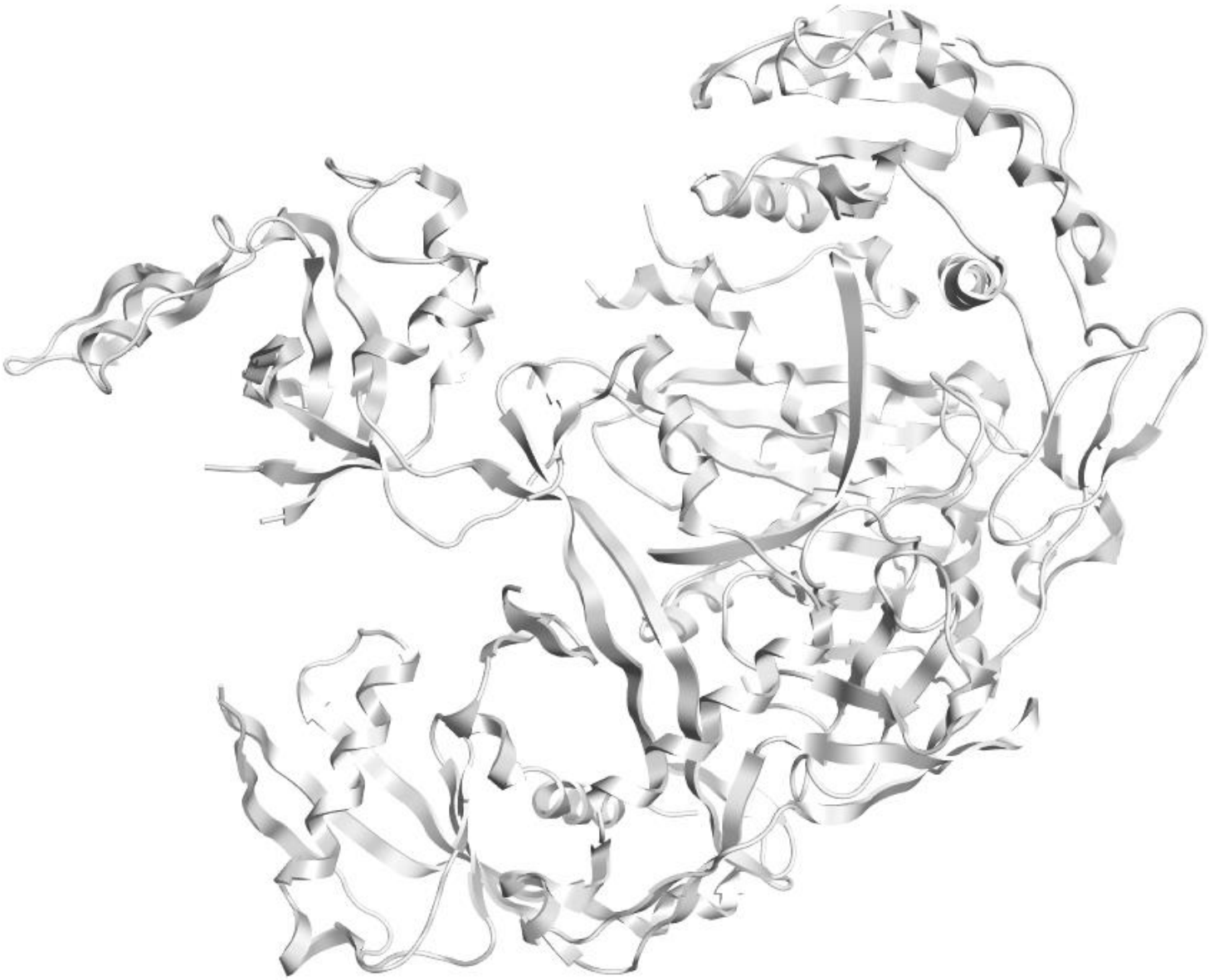


Bound RNA



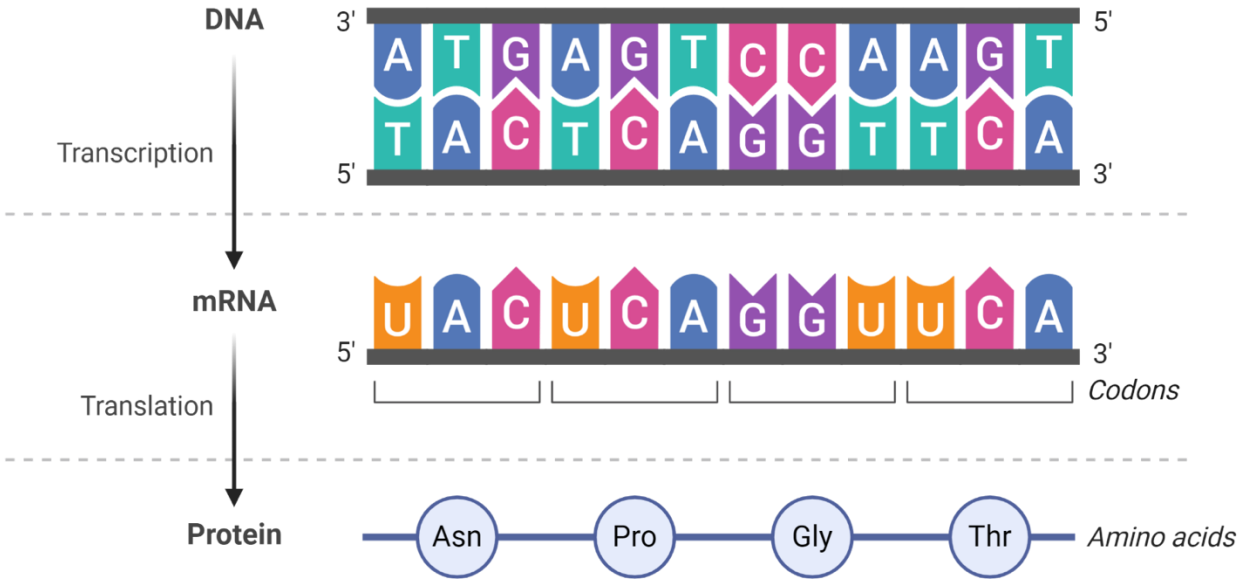
Alpha Helix

Color a Protein

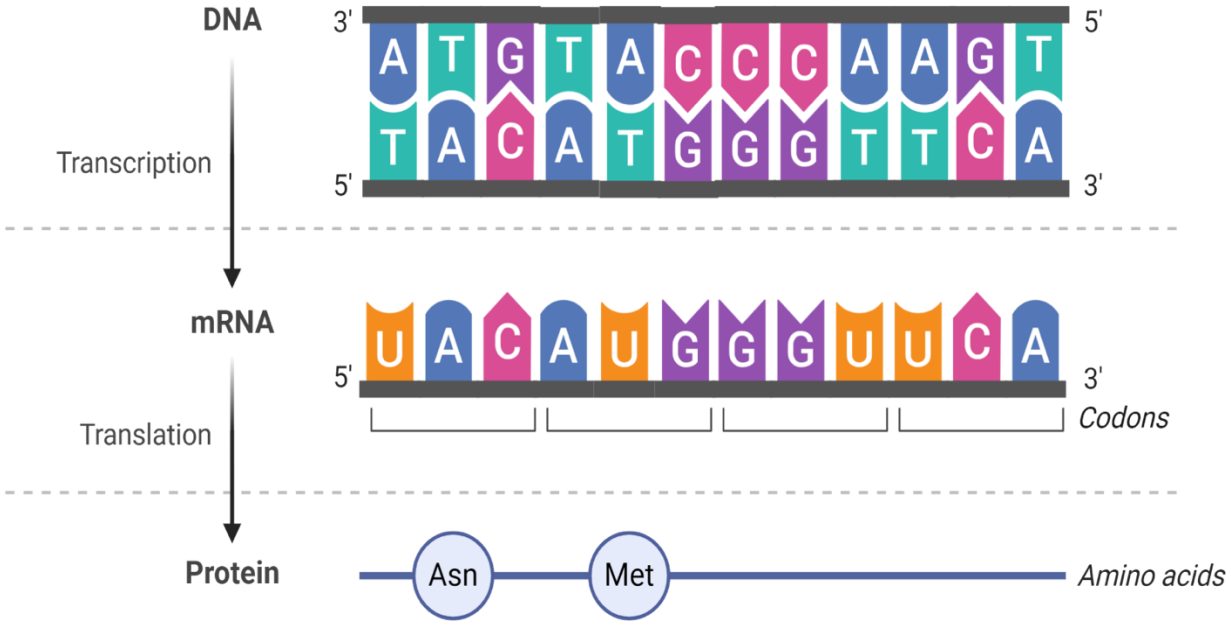


See answers on page 19

Disease Causing Mutations



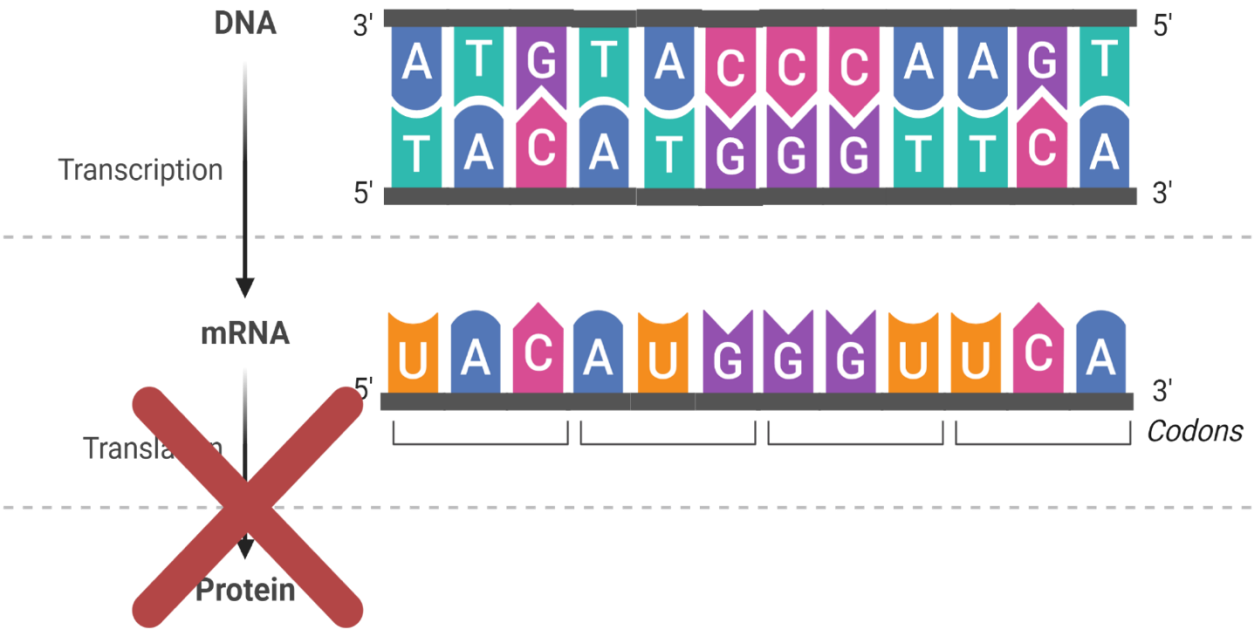
Small changes in our DNA or RNA can be transcribed and translated into proteins that cause diseases. Mutations can occur spontaneously!



For example, a mutation to Met ends the protein translation, resulting in a truncated (shorter) protein.

What is RNAi?

Diseases caused by proteins can be treated by preventing the translation of mRNA into protein.



Using the body's own tools to prevent translation of a gene is called RNA interference (RNAi).

Genetic Diseases

Errors in our genes can lead to many different diseases!

D P U H L Z Q R E N D L F F F J U I G I
U H I M P R X E A X X Y N V E E I T C Q
N L T E D R B U U C W A H L I D E O G W
Q U H S A N G C T W P H B N B G V B B R
V T V I X T N M Z K Y L J B A I V J E S
G K K P S C R W A M Y L O I D O S I S T
G H X D Y W V K J Y J W Q Y I O E C O E
Q Y E A M W A X M F A N O I T C E F N I
F I V I K H E M O P H I L I A H E E A J
Q P S R H E P A T I T I S B X S J N I O
H Q F U M R J U H K A T S C W E R B L P
B E C L R O L A G V U A F V H O X S G O
I Y M A D K C N T E O T I A P T G E E R
G M R X D Z L R N N M R M O J Q S L L P
V N Y O K D J E X G U D Q N G K T Y E H
L E R R B Z H A H S W O U F A H E W L Y
F K K E L M E W Q X V N L J C H G Y M R
M D X P P C J X S E Q Q T G V A L D J I
A X K Y D N O I S N E T R E P Y H J H A
G A C H R L O R E T S E L O H C W P G F

AMYLOIDOSIS
PORPHYRIA
HEMOPHILIA
HYPEROXALURIA

VIRUS
RNAI
NASH
COVID

HYPERTENSION
HEPATITISB
CHOLESTEROL
INFECTION

See answers on page 23

Knowledge Check Quiz

Fill in the blanks with the genetic terms contained in the word bank

WORD BANK

nucleotides

DNA (deoxyribonucleic acid)

1._____ carries the genetic instructions for how our cells grow, develop and function. This molecule is made up of individual _____ that base pair with each other.

____ 2. A mutation:

A. can change the amino acid sequence of a protein

B. is an alteration in your DNA sequence

C. may arise spontaneously

D. All of the above

____ 3. What are the possible combinations of nucleotide base pairs?

A. G-A, T-C

B. A-T, G-C

C. A-B, C-D

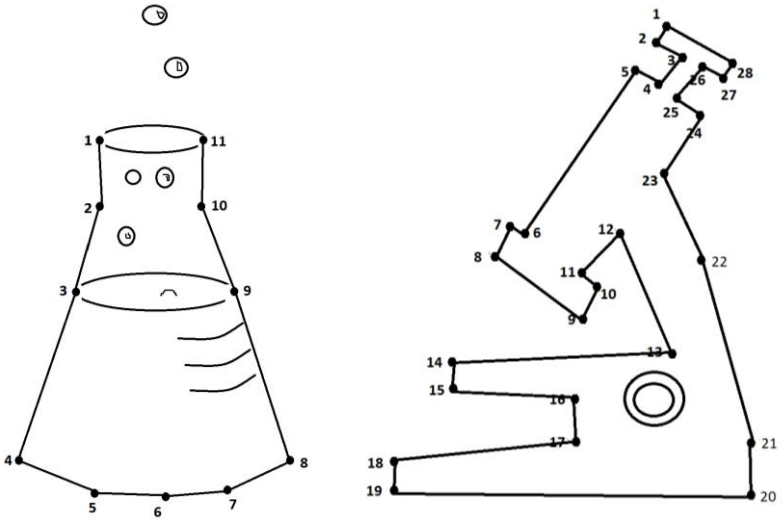
D. A-T, G-C, D-H

1. DNA (deoxyribonucleic acid), nucleotides 2. D 3. B

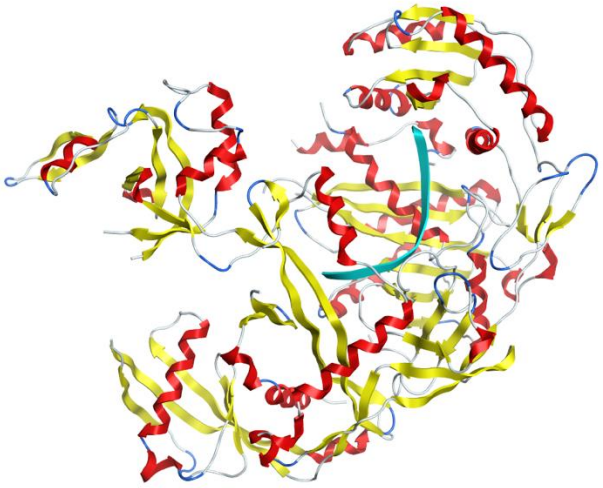
Answers Pages

Connect the dot

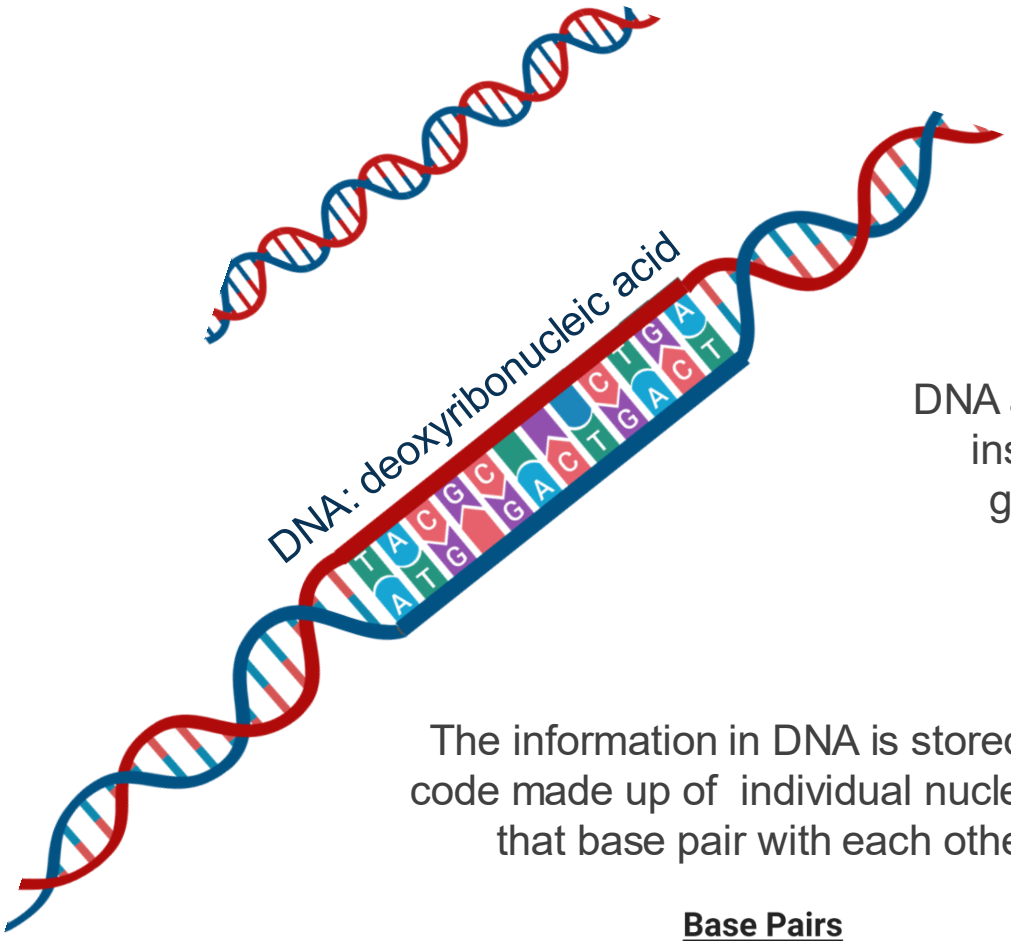
A beaker and a microscope!



Color a protein



Answers Pages



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The information in DNA is stored as a code made up of individual nucleotides that base pair with each other.

Base Pairs

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A T thymine

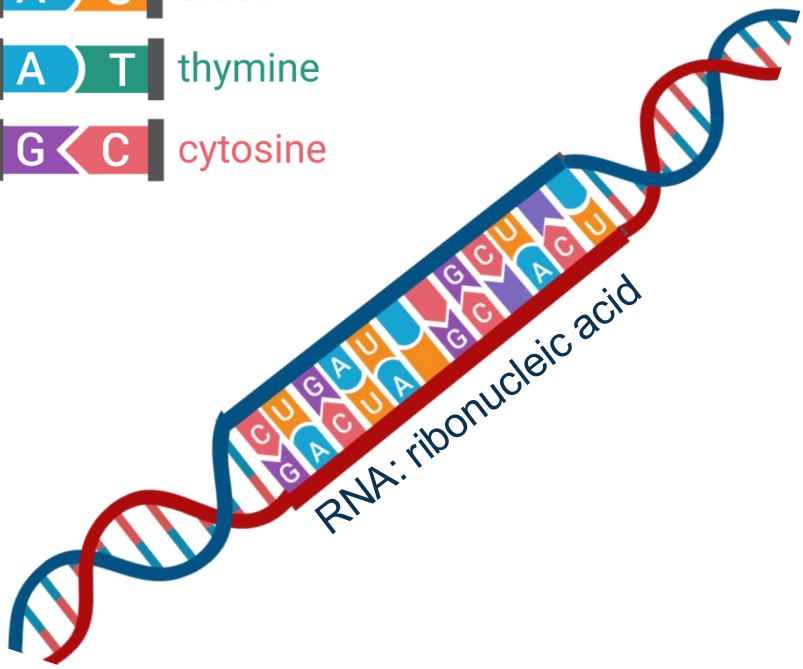
guanine G C cytosine

Challenge

Can you fill in the missing nucleotides?

What's the difference between RNA and DNA base pairs?

RNA has U as a base while DNA has T as a base



Answers Pages

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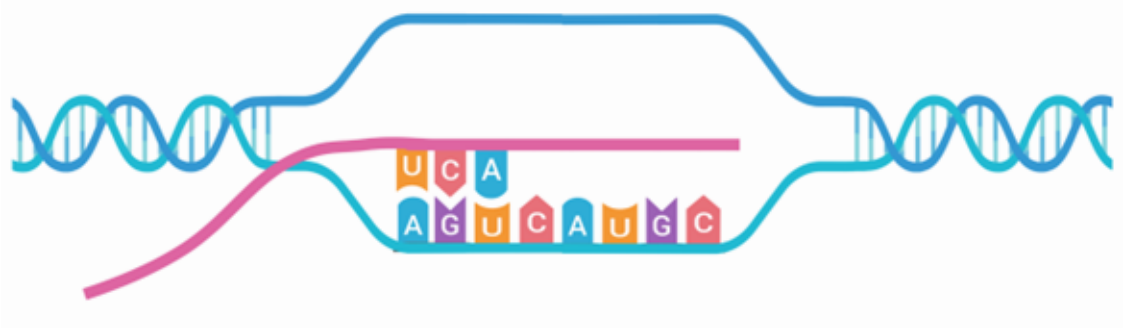

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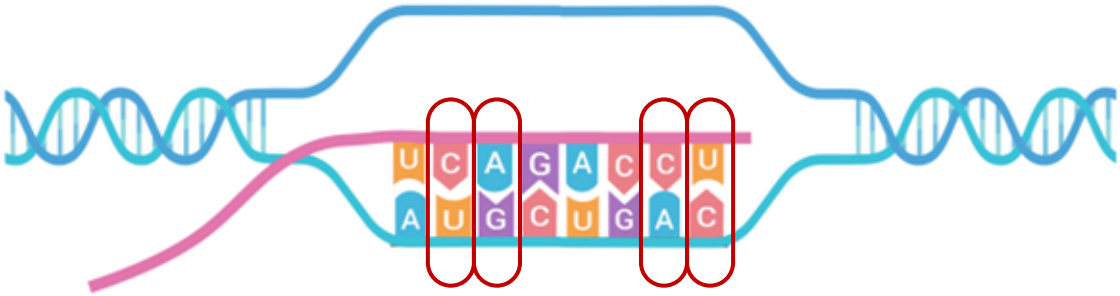

Answers Pages

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AMYLOIDOSIS
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HEMOPHILIA
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RNAI
NASH
COVID

HYPERTENSION
HEPATITISB
CHOLESTEROL
INFECTION

See answers on page 23

Alnylam® PHARMACEUTICALS

To those who say “impossible, impractical, unrealistic,” we say:

CHALLENGE ACCEPTED

Notes/References

1. Illustrations on pages 14 & 15 – Source: PDB (protein database) ID: 5JS1, PDB: https://www.rcsb.org/structure/5JS1
2. Images on pages 10, 11, 13, 16, 17 - Created with BioRender.com

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