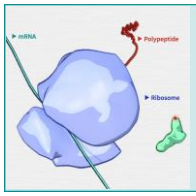

Central Dogma and Genetic Medicine

hhmi | BioInteractive

Click & Learn
Student Worksheet

OVERVIEW

This worksheet complements the Central Dogma and Genetic Medicine Click & Learn.

PROCEDURE

As you proceed through the Click & Learn, follow the instructions below and answer the questions in the spaces provided.

1. *Let's review!* The central dogma of molecular biology refers to the process of gene expression. Write the definition of gene expression in your own words.

2. *Click on the "Central Dogma" menu tab at the top of the screen*.

The table below outlines the steps in eukaryotic gene expression. Click on each tab or scroll through the page and briefly summarize each step below.

Gene Expression Steps	**Molecules Involved** What molecules and proteins are involved in this step?	**Summary** What happens during this step?
Transcription		
RNA Splicing		
mRNA Transport		
Translation		
Protein Processing		

3. Mutations in the DNA can affect the structure and function of proteins. Some mutations may even cause genetic diseases. Scientists and doctors can intervene at different points during gene expression to develop treatments for such genetic diseases—or genetic medicine. Let's learn about the genetic medicines that are being developed.

Select the "Genetic Medicine" tab located on the top right of the screen. **Click on the tab corresponding to the genetic medicine(s) that your instructor assigns to you, or scroll through the interactive and click on the pink "+" sign labeled with that genetic medicine.** Read the "Genetic Medicine" tab material, watch the video, and read the information in the "Learn more" link. **Then, complete the appropriate row(s) below.**

Genetic Medicine	**Short Summary** Write a one-sentence summary of how this genetic medicine works.	**Detailed Description** Describe how this genetic medicine would be used to treat a genetic disease. (For example, mention how it would fix the disease-causing mutation and/or result in a functioning protein.)
CRISPR-Cas9		
Gene Therapy		
Gene Switches		
Exon Skipping		
RNA Interference		
Small Molecule Drug		

4. Now let's learn about some of the diseases that may be treated using these genetic medicines.

Scroll through the interactive and click on the pink "+" sign that is labeled with the disease(s) that your instructor has assigned to you. Next, click on the "Case Study" tab at the top to reveal information about the disease. Read the "Case Study" material, watch the video, and read the information in the "Learn more" link. **Then fill in the appropriate row(s) in the table below.**

Name of Disease	**What are the key characteristics of the disease and whom does it affect? How can the featured genetic medicine be used to treat the disease?**
Leber Congenital Amaurosis	
Sickle Cell Disease	
Duchenne Muscular Dystrophy	
Huntington's Disease	
Cystic Fibrosis	

APPLY WHAT YOU HAVE LEARNED

5. You are a researcher working on a treatment for Hutchinson-Gilford progeria syndrome, an extremely rare genetic disorder that causes accelerated aging in children. Children with progeria generally appear healthy at birth but soon start growing more slowly than other children and lose their hair. Additional symptoms include stiffness of joints, heart problems, and stroke. These children typically die of heart disease at an average age of 13 years.

   Progeria is caused by a mutation in a single gene, called lamin A. Scientists have identified over 1,400 mutations in the lamin A gene that result in changes in transcription, RNA splicing, and/or protein production. Lamin A codes for a protein required for the structural support of the nuclear envelope in cells. Without a functional protein, the nuclear envelope becomes unstable, eventually damaging the nucleus and causing cells to die.

   Based on what you learned in this Click & Learn, propose a genetic medicine strategy you could develop to treat patients with progeria. Describe which step in gene expression you might target and why you would target that step, the intervention tool you would use, and explain how this strategy would treat the disease.