The Science and Fundamentals of mRNA Technology

modernatx.com/mrna-technology/science-and-fundamentals-mrna-technology

What does mRNA do? mRNA produces instructions to make proteins that may treat or prevent disease

mRNA medicines aren't small molecules, like traditional pharmaceuticals. And they aren't traditional biologics (recombinant proteins and monoclonal antibodies) – which were the genesis of the biotech industry. Instead, mRNA medicines are sets of instructions. And these instructions direct cells in the body to make proteins to prevent or fight disease.

It's actually basic human biology.

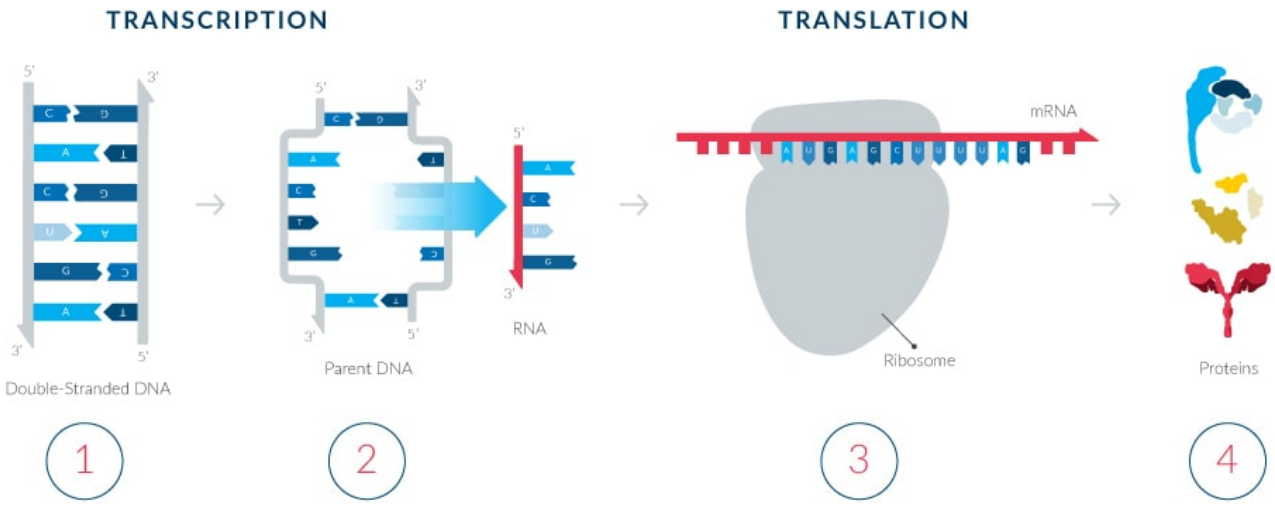
DNA (deoxyribonucleic acid) is a double-stranded molecule that stores the genetic instructions your body's cells need to make proteins. Proteins, on the other hand, are the 'workhorses' of the body. Nearly every function in the human body – both normal and disease-related – is carried out by one or many proteins.

mRNA is just as critical as DNA.

Without mRNA, your genetic code would never get used by your body. Proteins would never get made. And your body wouldn't – actually couldn't – perform its functions. Messenger ribonucleuc acid, or mRNA for short, plays a vital role in human biology, specifically in a process known as protein synthesis. mRNA is a single-stranded molecule that carries genetic code from DNA in a cell's nucleus to ribosomes, the cell's protein-making machinery.

Learn about messenger RNA's role in human biology, the instructions it provides that direct cells in the body to make proteins, and why we believe mRNA medicines may have the potential to treat a broad array of diseases.

mRNA's role in protein synthesis



1. Through a process known as transcription, an RNA copy of a DNA sequence for creating a given protein is made.
2. This copy – mRNA – travels from the nucleus of the cell to the part of the cell known as the cytoplasm, which houses ribosomes. Ribosomes are complex machinery in the cells that are responsible for making proteins.
3. Then, through another process known as translation, ribosomes 'read' the mRNA, and follow the instructions, creating the protein step by step.
4. The cell then expresses the protein and it, in turn, carries out its designated function in the cell or the body.

Using mRNA to develop a new category of medicines.

At Moderna, we are leveraging the fundamental role that mRNA plays in protein synthesis. We have developed proprietary technologies and methods to create mRNA sequences that cells recognize as if they were produced in the body. We focus on diseases where enabling targeted cells to produce – or turn 'on' – one or more given proteins will enable the body to fight or prevent a given disease.

- We start with our desired sequence for a protein.
- We design and synthesize the corresponding mRNA sequence – the code that will create that protein.
- Before synthesis, we also engineer that mRNA sequence to optimize the mRNA's physical properties, as well as those of the encoded protein.
- We deliver the mRNA sequence to the cells responsible for making that protein via one of several modalities. Reaching different types of cells requires different delivery methods.
- And, once the mRNA – the instructions – are in the cell … human biology takes over. Ribosomes read the code and build the protein, and the cells express the protein in the body.

Using mRNA as a drug opens up a breadth of opportunities to treat and prevent disease. mRNA medicines can go inside cells to direct protein production, something not possible with other drug approaches. We have the potential to treat or prevent diseases that today are not addressable – potentially improving human health and impacting lives around the world.

Learn about the intrinsic features of mRNA, how it is used in cells throughout the body and the diversity of potential applications for using mRNA to develop new medicines.