

Beyond COVID vaccines: What else could mRNA technology do for our health?

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Many people first became familiar with the term "[mRNA](#)" when Pfizer's and Moderna's COVID vaccines were rolled out. In the simplest terms, mRNA, which stands for messenger ribonucleic acid, is a type of genetic

material that gives cells in our bodies instructions to make specific proteins.

More recently the 2023 Nobel prize in physiology or medicine was awarded to Katalin Karikó and Drew Weissman from the University of Pennsylvania for their discoveries in mRNA biology.

These scientists' work has underpinned multiple successful COVID vaccines, which undoubtedly shifted the course of the pandemic. But their discoveries have likewise opened the door to a range of possible therapeutics which, until recently, remained elusive.

The promise of mRNA

Within each of our [cells](#) are [ribosomes](#), micro-machines that manufacture proteins, which in turn make up everything from muscle and bone to enzymes and hormones.

mRNA is the intermediate chemical "message" that carries the [genetic code](#) locked in the chromosomes of our DNA to the cytoplasm, the fluid that fills our cells and where proteins are made.

The ability to deliver genetic information directly into a cell has been one of medicine's most obstinate challenges. While mRNA was theoretically the most attractive way to achieve this, it was of little use as a therapy. This is because our immune system mistakes the foreign RNA as being an invading virus, mounting a powerful and toxic immune response. Injecting [naked mRNA](#) therefore can make you very sick.

So it was pivotal when Karikó and Weissman [pioneered a technique](#) to "cloak" mRNA from the immune system, alongside lipid nanoparticles to protect the RNA and allow it to be delivered safely to our cells.

This paved the way for mRNA COVID vaccines which instruct our cells to make spike proteins, proteins on the surface of SARS-CoV-2 (the virus that causes COVID). This in turn primes our immune system to make anti-spike antibodies that then block SARS-CoV-2 from infecting our cells.

Their discovery has opened up new possibilities for how we treat common infectious illnesses as well as genetic diseases that have previously defied treatment.

Flu vaccines

Influenza kills up to 650,000 people globally each [year](#). At the moment, [seasonal vaccines](#) need to be made annually once the main circulating strain has been identified. Manufacture takes about six months, by which time the original flu strain may have evolved. At best the seasonal vaccine is about [60% effective](#).

We need a better vaccine and mRNA technology offers the potential of a universal influenza vaccine, with [multiple candidates](#) currently undergoing human [clinical trials](#). A vaccine, if successful, could replace the current seasonal shots.

The mRNA vaccines are based on a specific part of the influenza protein, called hemagglutinin, teaching the cells to recall it and therefore inducing broad immunity across many influenza strains. In this [vaccine](#), hemagglutinin is the equivalent target the spike protein is in the COVID vaccines.

Cancer treatments

Targeting cancer is another promising avenue for mRNA technology,

with mRNA-based cancer immunotherapies already at the [trial stage](#).

One technique uses mRNA to mimic "neoantigens" (short bits of tumor proteins on the surface of the tumor cells) identified from an individual patient's tumor cells. Once delivered to the patient's [immune system](#), their body should produce powerful killer cells called cytotoxic T cells, eliciting a strong anti-tumor immune response.

Chimeric antigen receptor T cells (CAR-T) therapy is a form of [cancer immunotherapy](#) currently in use around the world to treat certain forms of leukemia. It uses immune cells called T cells that are genetically altered in a lab to help them locate and destroy cancer cells more effectively.

Traditionally CAR-T therapy has required a patient's T cells to be harvested from [white blood cells](#), modified, and then injected back into the patient. With mRNA technology the time consuming and most expensive steps are [could be eliminated](#) by delivering the CAR gene directly to T cells in the bloodstream.

Genetic diseases

mRNA technology is also transforming our response to some genetic diseases. [Hereditary angioedema](#) is a rare and potentially fatal genetic disorder where patients suffer severe and repeated attacks of swelling in their organs and tissues.

Scientists had discovered that a specific liver gene called KLKB1 prompts these swelling attacks. Researchers developed mRNA as a system to genetically edit and in turn "silence" the offending gene, with [initial results](#) positive for patients.

A similar trial using mRNA to edit the liver gene transthyretin alleviated

symptoms in patients suffering a life-threatening hereditary condition called [ATTR amyloidosis](#) which affects the nerves and heart.

The path ahead

Therapeutics based on mRNA technology are still in their infancy and hurdles remain. For example, mRNA is short-lived in cells and [protein](#) is only made for a short time. Increasing the life-span of mRNA in cells would reduce the amount of mRNA required (the dosage). Scientists are working on this and a couple of methods have [shown promise](#).

These caveats aside, the ability to deliver [genetic information](#) directly into cells could be a new frontier for medical therapeutics.

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