Moderna

Advancing mRNA Medicines to Fight and Prevent Disease

Moderna is driven by our mission to deliver on the promise of mRNA science to create a new generation of transformative medicines for patients. We are currently advancing mRNA clinical studies on our development candidates to address serious unmet needs across several therapeutic areas and multiple diseases, including Propionic Acidemia (PA), Methylmalonic Acidemia (MMA) and other rare genetic diseases that are caused by defects or deficits in proteins expressed by liver cells.

Moderna is taking a different approach to address the underlying cause of these diseases by delivering mRNA therapeutics intravenously (IV) to potentially stimulate production of therapeutic proteins in the liver in ways that cannot be achieved with other technologies. Our approach aims to help the body make its own missing or defective protein (in this case, PCC).mRNA technology does not change the genetic information of the cell, and it is short-acting. It acts like traditional drugs that can be adjusted over time based on the dose and frequency needed. In simple terms, we are working to provide physicians and patients with a "controllable" way to start and manage their therapy over time.

As we continue our work at Moderna to advance the development of mRNA-based therapeutics, we are pleased to share that our first program in PA, the Paramount Study, is now enrolling study participants in the United States.

People who are diagnosed with PA are missing the propionyl-CoA carboxylase (PCC) enzyme, which is responsible for the breakdown of certain proteins and fat. When this enzyme is missing, it is difficult for cells in the body to turn food into energy, leading to toxins building up in the bloodstream. Currently, PA is treated by lowering the amount of protein eaten daily, taking dietary supplements or antibiotics, or getting a liver transplant.

moderna | paramount study

The Paramount Study is a Phase 1/2 study. It is designed to evaluate if an investigational treatment called mRNA-3927 is safe and effective in reducing the symptoms of PA in individuals one year of age and older. mRNA 3927 is an investigational intravenous (IV) infusion treatment that instructs a persons' body to make a PCC enzyme that works.

More information including full trial inclusion and exclusion criteria can be found at https://trials.modernatx.comor by visiting https://clinicaltrials.gov

"As a company, we often talk about the societal responsibility we feel to deliver on the promise of mRNA science for patients. That sense of responsibility is felt with great intensity, and urgency, by the Rare Diseases team. We understand that, for so many patients suffering with a debilitating rare disease, there are no approved treatments. We are committed to leveraging our mRNA platform to advance medicines for some of these diseases and bring new hope to patients and their families."

- Paolo Martini, Chief Scientific Officer, Rare Disease