



Phase 1/2 Study of mRNA-3704 in Patients with MMA MUT Deficiency

Methylmalonic acidemia (MMA) is a genetic disease that is primarily caused by an enzyme named methylmalonyl CoA mutase (MUT) that does not work properly. MUT helps to break down foods that we eat. When MUT does not work, this causes a buildup of other substances (such as an acid called methylmalonic acid) in the body that can be harmful. Moderna is developing an investigational mRNA medicine (mRNA-3704) that provides instructions to cells in the liver to make normal MUT protein. It is hoped that by increasing the MUT proteins, the buildup of harmful substances in the liver and blood that are causing symptoms can be reduced.

A Phase 1/2 study will look at how safe the investigational drug, mRNA-3704, is and whether it works for children 1-18 years of age with MMA who have not received a liver or kidney transplant. Up to 34 children with MMA are expected to take part in the entire study. This study will take place at multiple research institutions throughout the United States.

Moderna is a biotechnology company focused on developing a new class of medicines using messenger RNA (mRNA). mRNA plays a critical role in human biology, providing a set of instructions to cells in the body to make proteins. Moderna is developing therapeutics and vaccines for infectious diseases, immuno-oncology, rare diseases and cardiovascular diseases, independently and with strategic collaborators. The Company currently has 20 development candidates in its pipeline, with 11 in clinical trials.

To learn more about this Phase 1/2 study, please contact your doctor, visit

<https://clinicaltrials.gov/ct2/show/NCT03810690> or email clinicaltrials@modernatx.com.

To learn more about Moderna, please visit [modernatx.com](https://www.modernatx.com).