

## **NON-TECHNICAL ABSTRACT**

We are performing a study to test rAAV-hAAT, a gene therapy, on humans. The test drug has been created to replace a malfunctioning gene in the human body. The gene normally makes a protein used by the lungs. People who can't make this protein develop problems with their breathing which can get very severe as they grow older.

Although other medicine is available which can help the body make up for this missing protein, patients must have injections of this treatment every week for the remainder of their lives. This is both painful and expensive.

In this study, volunteers will have three (3) shots of the gene that will cause their bodies to make this missing protein on its own. Different tests will then be done on the volunteer's blood and breathing to see if the drug both works and does not cause new problems.