

NON-TECHNICAL ABSTRACT

Cystic fibrosis (CF) is a common inherited disease, effecting about 1 in 1600 Caucasians. Most patients with cystic fibrosis die before age 30. The disease can effect many parts of the body (including the pancreas, reproductive glands, and sweat glands), but the cause of death is usually due to lung failure from thick secretions and chronic infection of the lungs.

Human genes carry functions which are necessary for healthy human life. Cystic fibrosis is caused when one of these genes, called the cystic fibrosis gene, cannot function properly. As a result of this dysfunction, tissues in the bodies of CF patients (and the cells which are the building blocks of these tissues) cannot move salts properly. For example, the high content of salt in the sweat of CF patients, and the thick respiratory mucous in the lungs of CF patients, have both been associated with difficulties moving salts such as sodium and chloride.

The purpose of this study is to determine whether normal, functional copies of the CF gene can be delivered to the cells lining the nose of CF patients. This study will use small amounts of molecules of fat (also called "lipids") to carry the normal gene into these cells.

This study has two goals. The first goal is to determine whether lipids can be used to safely carry the normal gene into the cells lining the nose. The second goal is to determine whether after receiving the normal CF gene, the inside of the nose will develop an improved ability to move salts.

The use of genes to treat human diseases has been called "gene therapy". Because the serious complications of CF occur in parts of the body other than the nose (for example, in the lungs and pancreas), it is unlikely that the delivery of a normal CF gene to the inside of the nose will make participants in this study healthier. On the other hand, the overall goal of this research study will be to allow the possibility of attempting the same type of normal gene delivery to CF lungs, with the hope of developing better treatments for cystic fibrosis.