



Gene Therapy for Cystic Fibrosis

The University of Florida is seeking a company interested in commercializing a gene therapy for treating Cystic Fibrosis (CF). CF is a widespread genetic disease that affects approximately 30,000 people in the United States, with about 1,000 new cases of CF diagnosed every year. More than 10 million Americans are unknowing carriers of a defective CF gene. Approximately 500 CF patients die each year, usually from lung damage due to the disease. Our new therapy addresses this problem by targeting the readily accessible airway cells within the lungs of patients.

Applications

Gene therapy tool to treat Cystic Fibrosis

Advantages

- ◆ Offers a competitive advantage over alternative therapies by targeting the underlying cause of lung damage instead of only treating its symptoms
- ◆ Improves the delivery of the gene to cells and is less likely to trigger the body's immune response than alternative therapies
- ◆ Prevents the progression of lung damage, increasing patients' lifespan and improving overall quality of life
- ◆ Decreases patients' predisposition to respiratory infection which protects lungs from further damage

The Technology

CF is caused by a defect in a single gene that codes for a protein called the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR). This protein regulates the salt concentration of bodily fluids in a variety of organs. Problems with salt concentration in the lungs leads to an increased frequency, duration, and severity of lung infections. The body's attempts to eliminate these infections result in severe damage to the lungs. Our technology involves the delivery of a functional version of the CFTR gene into the airway cells which produces functional protein that either slows or stops the progression of CF before severe lung damage occurs.

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