

**Docket #:** S18-325

# **Transplantation of Airway Stem Cells to Treat Cystic Fibrosis and Other Lung Disorders**

Researchers at Stanford have demonstrated the first method of its kind for treating cystic fibrosis (CF) using regenerated airway stem cells embedded on a biocompatible scaffold. Their recently released study supports further development of this novel concept, i.e., using genetically corrected autologous airway stem cell transplant as a treatment for lung disorders. CF is a debilitating disease that results in early death and high morbidity. Improved therapies for CF and other lung disorders remain a tremendous unmet clinical need. Currently there are no stem cell-based regenerative medicine therapies for solid organs. This research advances a new concept of regenerating airway tissue via transplantation of genome edited sinus airway stem cells, as well as the concept of embedding those cells in a biocompatible membrane. There are no previous reports of transplanting gene corrected airway basal stem cells.

## **Stage of Development**

The researchers used Cas9 and adeno-associated virus 6 to correct a mutation in readily accessible upper-airway basal stem cells (UABCs) obtained from CF patients. On average, they achieved 30%-50% allelic correction in UABCs and bronchial epithelial cells (HBECs) from 10 CF patients and observed 20%-50% CFTR function relative to non-CF controls in differentiated epithelia. They also successfully embedded the corrected UABCs on a FDA-approved porcine small intestinal submucosal membrane (pSIS), and they retained differentiation capacity.

## **Applications**

- Transplantation of airway cells into the airways to treat/prevent lung failure due to various causes

- Transplantation of airway cells engineered to express other proteins/factors to treat other indications
- Treatment of cystic fibrosis sinusitis and lower airway disease
- Transplantation of gene corrected airway stem cells to treat cystic fibrosis

## Advantages

- Novel and high potential
- Addresses unmet clinical need

## Publications

- Vaidyanathan S, Salahudeen AA, Sellers ZM, et al. High-Efficiency, Selection-free Gene Repair in Airway Stem Cells from Cystic Fibrosis Patients Rescues CFTR Function in Differentiated Epithelia. *Cell Stem Cell*. 2020;26(2):161-171.e4. [doi:10.1016/j.stem.2019.11.002](https://doi.org/10.1016/j.stem.2019.11.002)

## Patents

- Published Application: [WO2020132248](https://patents.google.com/patent/WO2020132248)

## Innovators

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