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

Patents

- Published Application: WO2020132248

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