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Improved airway-targeted gene delivery in a pig model of cystic fibrosis

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Cystic fibrosis (CF) is characterized by accumulation of thick mucus in the lungs and is associated with a high incidence of bacterial infection. Mutations in the gene encoding CF transmembrane conductance regulator (CFTR) underlie the disease. Gene therapy to deliver a normal copy of the *CFTR* gene has shown promise in both pre-clinical models and clinical trials; however, current gene delivery methods are inefficient and do not result in sustained expression of functional *CFTR* in the airways.

Two studies in this issue of *JCI Insight* report the development and use of viral vector-based delivery of *CFTR* in pig models of CF.

A team led by Patrick Sinn and Paul McCray Jr. of the University of Iowa developed a lentiviral-based vector that was delivered through the nose to newborn CF pigs. Analysis of these animals revealed evidence of functional *CFTR* expression in the airways.

A second team led by Joseph Zabner of the University of Iowa and David Schaffer of the University of California, Berkeley, generated an adeno-associated virus (AAV) that homes to the pig airway to mediate expression of functional *CFTR* in the airways of 1 week-old CF pigs. Together, these reports indicate that viral vector-based approaches could potentially improve the effectiveness of *CFTR* gene therapy.

More information: Benjamin Steines et al. CFTR gene transfer with AAV improves early cystic fibrosis pig phenotypes, *JCI Insight* (2016).

DOI: 10.1172/jci.insight.88728

Ashley L. Cooney et al. Lentiviral-mediated phenotypic correction of cystic fibrosis pigs, *JCI Insight* (2016). DOI: 10.1172/jci.insight.88730