Gene replacement in pigs ameliorates cystic fibrosis-associated intestinal obstruction

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Cystic fibrosis (CF) is caused by mutations in CFTR and is characterized by dysfunction of the lungs, liver, pancreas, and intestines. Approximately 15% of babies with CF are born with an obstruction of the small intestine known as meconium ileus, frequently the first sign of CF. Unlike in humans, meconium ileus occurs in 100% of newborn CF pigs.

n this issue of the *Journal of Clinical Investigation*, Michael Welsh and colleagues at the University of Iowa demonstrate that transgenic expression of normal CFTR in the intestine of CF pigs alleviated meconium ileus. Over time, the pigs still exhibited other manifestations of CF, including liver and lung disease, reduced weight gain, and pancreatic destruction.

These findings provide insight into the pathophysiology of CF and indicate that tissue-specific, partial gene replacement can ameliorate intestinal symptoms of CF.

More information: Intestinal CFTR expression alleviates meconium ileus in cystic fibrosis pigs, *J Clin Invest*. doi:10.1172/JCI68867

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