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CFTR-knockout neonatal ferrets model may be useful in the understanding of CF pathogenesis and developing therapies

There is no adequate animal model of cystic fibrosis (CF) airways disease. This study investigated using CFTR-knockout neonatal ferrets as they show similarities to newborn humans with CF. These similarities include meconium ileus (MI), pancreatic disease, liver disease, severely impaired nutritional status, absent or degenerate vas deferens at birth, defective airways and a predisposition of lung infection. There is a higher prevalence of MI and intestinal complications in CFTR-knockout kits than in humans with CF. This limitation is overcome by using transgenic CFTR-knockout kits expressing CFTR in the intestine. Oral administration of ursodeoxycholic acid normalised liver function and a proton pump inhibitor improved the nutrition status and survival in the CF ferrets. Despite improved nutrition, CFTR-knockout neonatal ferrets had increased bacterial counts in the airways that subsided after the first week.

The findings of CF phenotype in various organs in CFTR-knockout neonatal ferrets and their similarities to human CF may be useful in the understanding of CF pathogenesis and the future development of organ-specific therapies.

► **Sun X**, Sui H, Fisher JT, *et al.* Disease phenotype of a ferret CFTR-knockout model of cystic fibrosis. *J Clin Invest* 2010;**120**:3149–60.

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