Intervention in infants with cystic fibrosis key to slowing progression

December 5 2008

Early detection of lung disease in cystic fibrosis (CF), combined with aggressive treatment in infants, may be the key to controlling the progression of the disease, according to a recent study. New research shows that contrary to previous scientific opinion, progressive lung damage in CF patients can begin as early as infancy even though lung function shortly after diagnosis is normal.

"We might be able to stop some of the lung function reduction we're identifying in the first months of life," said Dr. Sarath Ranganathan, Ph.D., consultant respiratory physician at the Royal Children's Hospital in Melbourne, and lead author of the paper, which appeared in the second issue for December of the American Thoracic Society's *American Journal of Respiratory and Critical Care Medicine*.

"We don't know what's going to work, but we have to target those patients in the first six months of life if we're going to be effective," added Dr. Ranganathan.

In the Australian study, 68 infants with CF were compared to 49 infants without the disease. The children were between six weeks and 30 months of age. Forced expiratory volume (FEV) measurements were obtained for the children at baseline, and 16 of the children with CF were measured again one year later. FEV was equivalent for all children both with and without CF at baseline. But by six months of age, the mean FEV score was significantly lower in infants with CF compared to controls—and the deficit increased with each month of age. "This

finding indicates that lung function declines sooner than previously thought," said Dr. Ranganathan.

The study also found that diminished lung function occurred even in the absence of clinical symptoms and irrespective of CF genotype. But, notably, infants measured soon after birth and within the first six months of life had normal lung function. Newborn screening for CF has been in existence in Australia for nearly 30 years, but was introduced only recently in the United Kingdom and the United States. This finding validates the early-screening approach taken in Australia and strongly suggests that other nations should adopt similar approaches.

"Ninety percent of those with CF can't absorb the fat in their diet, and people with this condition who are better nourished live longer," said Dr. Ranganathan. "[Those patients] should take manufactured pancreatic enzymes with every meal that contains fat, so they can have a good high-fat diet with lots of calories," he added. "Although the patients in our study were well-nourished, they still had diminished lung function. However, we can't be certain whether or not fat malabsorption that occurs prior to starting enzymes at diagnosis contributes to diminished lung function later on in early childhood."

While the FEV tests were conducted according to standardized guidelines recently developed by the American Thoracic Society and the European Respiratory Society, if better tests for lung function in newborns could be developed, it might be possible to determine that lung function is not in fact normal during the first six months, Dr. Ranganathan said. "Our paper really indicated that lung function is diminished after the first six months, but appears to be normal in the first six, which is good news and bad news," he said. "It's bad news because even with the best current treatment we don't seem able to prevent diminished lung function occurring in later infancy. The good news is that if we can diagnose infants with this condition early by

newborn screening, we have shown for the first time that there appears to be a window of opportunity in the first six months of life to intervene to prevent diminished lung function." The findings may inform future research—and, it is hoped, clinical practices.

As a result of this research, Dr. Ranganathan and colleagues have developed a research team called The Australian Respiratory Early Surveillance Team for Cystic Fibrosis (AREST-CF).

"We are looking into the possibility of an intervention trial using macrolides as these drugs have proved useful in older children and adults. Anticipating that we would conduct the trial in one or two years' time, we think we need to recruit close to 80 patients in each arm of the trial," said Dr. Ranganathan, who is chair of the Melbourne wing. "Our main aim would be to optimize what an appropriate study design for an intervention trial should be so that we can move as quickly as possible from research to treatment. We'd like to be very aggressive about treatments from the very day an infant is diagnosed," he added.

Source: American Thoracic Society

Citation: Intervention in infants with cystic fibrosis key to slowing progression (2008, December 5) retrieved 25 April 2024 from https://medicalxpress.com/news/2008-12-intervention-infants-cystic-fibrosis-key.html

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.