Researchers find test to ID children at higher risk for cystic fibrosis liver disease

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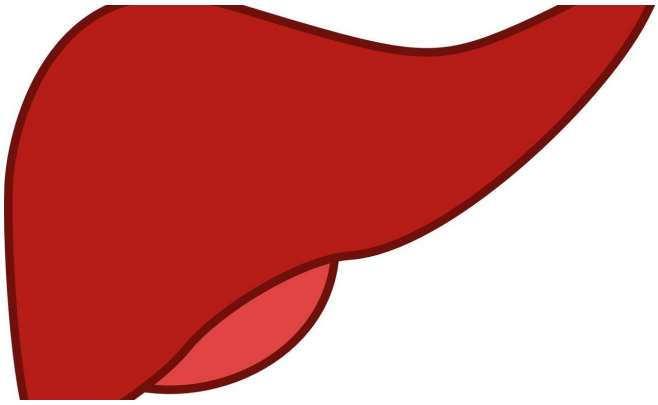

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A major multi-center investigation of children with cystic fibrosis has identified a test that allows earlier identification of those at risk for cystic fibrosis liver disease.

The study, which includes 11 clinical sites in North America, was led by Michael Narkewicz, MD, professor of pediatrics from the University of Colorado School of Medicine and Children's Hospital Colorado. The findings of the study are published today ahead of print in The *Journal of Pediatrics*.

Cystic fibrosis is a genetic disorder that primarily affects the lungs and the pancreas, but can also create problems in the liver and other organs. Advanced cystic fibrosis liver disease refers to advanced scarring of the liver, which occurs in about 7 percent to 10 percent of patients with cystic fibrosis. The exact cause of advanced cystic fibrosis liver disease is not known, but only individuals with cystic fibrosis develop it.

"As we develop new therapies for cystic fibrosis and for other liver diseases, it is critical that we better understand which patients with cystic fibrosis are at higher risk for cystic fibrosis liver disease," said Narkewicz. "Our study is a vital first step in identifying those patients and should help in the creation of targets for therapies that could prevent cystic fibrosis liver disease."

The results reported in the article show that researchers could identify children at higher risk for advanced liver disease by using research-based ultrasound screening with consensus grading by four radiologists. The report is a four-year interim analysis conducted as part of a projected nine-year study.

The study covers 722 participants recruited between January 2010 and February 2014. Children from 3 years to 12 years of age who had been diagnosed with cystic fibrosis were eligible to enroll in the study. Using research-based ultrasound screening, researchers sought to determine whether the participants had a heterogeneous pattern on their livers that could indicate a higher risk for advanced cystic fibrosis liver disease. The study found that of the participants with the heterogeneous pattern were at a nine-fold higher risk than those participants with a normal pattern to develop advanced cystic fibrosis liver disease.

"This is the first large multicenter study of ways to predict which children with cystic fibrosis are at higher risk for advanced liver disease," Narkewicz said. "The findings show that we have found an important way to project which patients might develop cystic fibrosis liver disease. It also gives us clinical therapeutic targets for interventional therapies that could prevent the development of this liver disease. "

These research-based ultrasound exams relied on multiple radiologists reviewing the results. Narkewicz said clinically based liver ultrasound exams may not have the same predictive value. The research-based ultrasound tests are a next

step after liver enzyme monitoring and could indicate an increased risk for developing advanced cystic fibrosis liver disease. Researchers are continuing to study the magnitude of the risk.

More information: Marilyn J. Siegel et al, Heterogeneous Liver on Research Ultrasound Identifies Children with Cystic Fibrosis at High Risk of Advanced Liver Disease: Interim Results of a Prospective Observational Case-Controlled Study, *The Journal of Pediatrics* (2020). DOI: 10.1016/j.jpeds.2019.12.033

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