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Cystic Fibrosis Research News

Title:
Heterogeneous liver on research ultrasound identifies children with cystic fibrosis at high risk of advanced liver disease

Lay Title:
Children and Adolescents with CF who have a patchy liver on a research ultrasound (US) are at increased risk of developing advanced CF liver disease

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What was your research question?

Does a heterogeneous (patchy) pattern of the liver on US identify in children with CF identify a cohort at increased risk for the development of advanced liver disease.

Why is this important?

Advanced liver disease affects about 5-10% of children and adolescents with CF. Individuals with CF and advanced liver disease have worse outcomes compared to those who do not have advanced liver disease. To pursue studies to try and prevent the development of advanced liver disease, there is a need to identify a cohort with a higher risk of advanced liver disease to help design appropriate clinical trials. This work lays the groundwork for designing trials for prevention and for early identification of individuals with advanced liver disease.

What did you do?

We enrolled 722 children with CF and pancreatic insufficiency and did a research liver US. Each US study was interpreted by 3 different study radiologists. The pattern was determined by the majority of the 3 radiologists. We followed the 55 children with a patchy pattern on US (HTG) and 116 with a normal pattern (NL) for 6 years with an US every 2 years and lab and clinical visits every year.

What did you find?

We found that 33% of children with a HTG US developed a nodular (NOD) liver that indicates advanced liver disease by 6 years, compared to 3.4% who had a NL US. Adding in the age at

the initial US and an index of scarring called GPR (using 2 blood tests: GGTP and platelet count) was able to predict very well who would develop NOD US.

What does this mean and reasons for caution?

This study shows that using research US and GPR can identify children with a high risk for advanced liver disease that affects about 7% of children with CF. However, when we just used the interpretation of one radiologist, we missed many children who developed NOD. Thus, a clinical US finding of HTG does not mean that the risk is as high as we found in this study. We also found that the finding of a normal liver with a normal GPR means that the risk for advanced liver disease is likely low.

What's next?

We hope to study machine learning to improve how the US findings are determined and reduce variability and we studying for new blood tests to help identify children at risk for advanced liver disease. We hope that this would allow trials of new therapies to prevent advanced liver disease in the future.

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