

Targeting the EGFR and FGFR cellular pathways for bile duct cancer

February 14 2014, by Steve Yozwiak

Researchers at the Translational Genomics Research Institute (TGen) and physicians at Mayo Clinic's Individualized Medicine Clinic have personalized drug treatments for patients with cholangiocarcinoma using genomic sequencing technologies.

Potential new treatment approaches are being validated to develop new tests that physicians can use to guide therapy for this aggressive [cancer](#) of the bile ducts that progresses quickly and is difficult to treat.

Clinically important findings suggest that targeting the EGFR and FGFR cellular pathways may benefit thousands of patients with this disease, according to the study published today in the journal *PLOS Genetics*.

Half of the patients treated in this study responded to either ponatinib (typically used for certain types of leukemia) or pazopanib (a kidney cancer drug), depending on the [genetic alterations](#) identified through sequencing.

"In 3 out of the 6 patients we analyzed, we found compelling, treatable and unexpected genetic alterations that would never have been found by normal testing methods for cholangiocarcinoma," said Dr. Mitesh Borad, a Mayo Clinic oncologist and lead author of the paper. "We treated these three patients with drugs that attack these genetic alterations and saw tumor shrinkage. This gives us hope for better treatments for this aggressive, hard-to-treat cancer."

Because of these encouraging early results, the team of 49 doctors and researchers proposes large-scale clinical trials to test EGFR and FGFR inhibitors as possible treatments for biliary tract cancers that harbor mutations in these genes and pathways.

"Our results demonstrate that if we find the right molecular context, more appropriate therapies can be chosen that improve outcomes," said Dr. John Carpten, TGen Deputy Director of Basic Science and Director of TGen's Integrated Cancer Genomics Division, and the study's senior author. "We now hope to design larger clinical studies to treat patients' tumors harboring these novel genomic aberrations to further explore the precise extent of clinical benefit for patients with primary or advanced [cholangiocarcinoma](#)."

Bile duct cancer is a rare cancer that occurs mostly in people older than age 50. Surgical approaches, such as resection and liver transplantation, represent the only curative treatment approaches, said Dr. Borad. Most [patients](#), however, present with surgically unresectable or metastatic disease at the time of diagnosis. Standard-of-care chemotherapies are not curative and there is an unmet need for newer approaches, Dr. Borad added.

Provided by Translational Genomics Research Institute

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