

Cancer drug may benefit patients with inherited form of kidney disease

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A cancer drug called bosutinib may inhibit the growth of cysts in patients with autosomal dominant polycystic kidney disease (ADPKD), according to a study appearing in an upcoming issue of the *Journal of the American Society of Nephrology (JASN)*. The findings point to a potential new treatment strategy for affected patients, but the long-term

benefits remain to be determined.

ADPKD is an inherited disorder that affects up to 1 in 1000 people and is characterized by cysts in the [kidney](#) and other organs. As patients' kidney volume increases due to cyst growth, they gradually lose their [kidney function](#) and often develop [kidney failure](#). Current treatments are primarily supportive, such as focusing on hypertension and other secondary complications.

The inherited mutations that cause ADPKD affect a protein involved in various signaling pathways that often involve enzymes called [tyrosine kinases](#). Therefore, a team led by Vladimir Tesar, MD, PhD (Charles University and General University Hospital, in the Czech Republic) tested the potential of an investigational drug called bosutinib that inhibits a particular tyrosine kinase called Src/Bcr-Abl.

The phase 2 study included patients with ADPKD who were randomized 1:1:1 to bosutinib 200 mg/day, bosutinib 400 mg/day, or placebo. Of 172 patients enrolled, 169 received at least one treatment. The higher dose of bosutinib was not well tolerated.

The annual rate of kidney enlargement was reduced by 66% for patients receiving bosutinib 200 mg/day vs. those receiving placebo (1.63% vs. 4.74%, respectively) and by 82% for all patients receiving bosutinib vs. those receiving placebo (0.84% vs. 4.74%, respectively). The study was not powered to demonstrate a treatment effect on kidney function, but there was no evidence of a benefit associated with bosutinib compared with placebo over the 2-year treatment period.

"The reduction in growth of cysts through [treatment](#) with bosutinib was confirmed, although gastrointestinal side effects (primarily diarrhea), which were partly dose-dependent, may represent a substantial drawback for the further development of the drug for [patients](#) with ADPKD," said

Prof. Tesar.

More information: "Bosutinib Versus Placebo for Autosomal Dominant Polycystic Kidney Disease," *Journal of the American Society of Nephrology* (2017). [DOI: 10.2215/CJN.01530217](https://doi.org/10.2215/CJN.01530217)

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