

Epilepsy Drug Shows Promise in Treating Kidney Disease

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(PhysOrg.com) -- An anti-convulsant drug commonly used to treat epilepsy reduces cysts in mice that are associated with polycystic kidney disease (PKD), a difficult to treat ailment that afflicts 600,000 people in the United States, Yale University researchers report.

“This is exciting because the drug (valproic acid) is also in clinical trials as a potential cancer drug and has a known safety profile,” said Zhaoxia Sun, associate professor of genetics and senior author of the paper scheduled to be published online this week in the [Proceedings of the National Academy of Sciences](#).

Sun’s lab began searching for [chemical compounds](#) that would suppress the effects of one of two genes known to cause the most common form of PKD, which is the fourth leading cause of kidney failure. The screens identified a class of molecules called HDAC inhibitors as potential [drug candidates](#). These inhibitors eliminated the curve tail in zebrafish, a defect associated with the PKD-causing gene.

The epilepsy drug valproic acid, marketed under brand names such as Depakote, is also a HDAC inhibitor. So Sun’s collaborator Stefan Somlo, professor of internal medicine and genetics at Yale School of Medicine, used the drug to treat mice with PKD. The team noted that treated mice showed a marked reduction of cysts and improved [kidney function](#).

Sun observed that multiple HDAC inhibitors are being developed as anti-cancer drugs and each would be a potential candidate to treat PKD.

“Currently there is no directed therapy, but it is a slow progressing disease, which frequently takes decades to develop symptoms,” Sun said. “Therefore, a treatment of even modest efficacy could have significant clinical impact.

Other Yale authors of the paper were Ying Cao, Nichole Semanchik, Seung Hun Lee, Paolo Emilio Barbano, and Ronald Coifman.

Provided by Yale University ([news](#) : [web](#))

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