

A promising treatment for an incurable, deadly kidney disease

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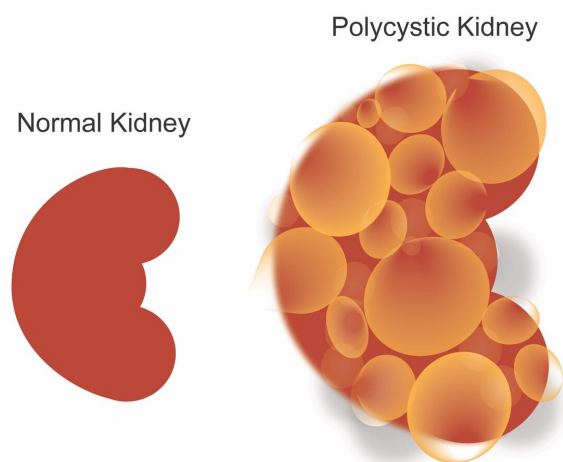
Dr. Vishal Patel. Credit: UTSW

A potential treatment for polycystic kidney disease—a genetic disorder that causes the kidneys to swell with multiple cysts and can eventually lead to organ failure—has shown promising results in animal testing.

A study describing the drug's development and testing appears today in *Nature Communications*. The study shows an approximately 50 percent reduction in [kidney](#) size in afflicted mice following treatment. The drug is now in early clinical trials in [human subjects](#), said Dr. Vishal Patel, Associate Professor of Internal Medicine at UT Southwestern and senior author of the study.

Autosomal dominant [polycystic kidney disease](#) (ADPKD) affects about 12 million people worldwide, with half developing end-stage kidney disease by age 60, according to the study. "Once the kidneys have failed, the only options for survival are dialysis or a kidney transplant," Dr. Patel said. "A large percentage of ADPKD patients on dialysis die each year while waiting for a donated kidney."

The only drug currently approved to treat ADPKD, called Jynarque (generic name tolvaptan), carries the FDA's highest warning in its prescribing information, a box notifying prescribers and users of the possibility of "serious and potentially fatal liver injury."



Credit: UT Southwestern Medical Center

The new treatment cooperatively developed at UT Southwestern and Regulus Therapeutics Inc., a biopharmaceutical company based in California, showed no evidence of toxicity in animals or in human cell tests, according to the study. It is preferentially delivered to kidneys rather than the liver after being administered, according to the *Nature Communications* study.

"We earlier showed that levels of a tiny RNA fragment called microRNA-17 are increased in models of ADPKD," Dr. Patel said. "MicroRNA-17 interferes with the normal function of other, beneficial RNAs, causing kidney cysts to grow. RGLS4326, as the new drug is called in development, works by blocking the harmful

microRNA-17."

Early phase one clinical trials began last year, conducted by Regulus Therapeutics. The FDA has asked for additional toxicity information from [animal testing](#) before human trials can move to the next step, Dr. Patel said.

More information: Edmund C. Lee et al, Discovery and preclinical evaluation of anti-miR-17 oligonucleotide RGLS4326 for the treatment of polycystic kidney disease, *Nature Communications* (2019). [DOI: 10.1038/s41467-019-11918-y](https://doi.org/10.1038/s41467-019-11918-y)

Provided by UT Southwestern Medical Center

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