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Opinion Article

Exploring Alternative Treatments for Autosomal Dominant Polycystic Kidney Disease through Drug Repurposing

Michael Pove*

Department of Medicine, University of California, San Francisco, United States of America

DESCRIPTION

Autosomal Dominant Polycystic Kidney Disease (ADPKD) is a common genetic disorder characterized by the formation of numerous fluid-filled cysts in the kidneys, leading to progressive kidney failure. Currently, there is no cure for ADPKD, and treatment options are limited to managing symptoms and slowing disease progression. ADPKD is a disease which affects millions of people worldwide. It is caused by mutations in the genes encoding Polycystin-1 (PC1) or Polycystin-2 (PC2), leading to the formation of numerous fluid-filled cysts in the kidneys, which can cause renal failure, hypertension, and other complications. Currently, there is no cure for ADPKD, and treatment is focused on managing symptoms and slowing disease progression. However, recent advances in drug repurposing have provided new hope for the development of effective therapies for ADPKD.

Drug repurposing, also known as drug repositioning or drug reprofiling, is the process of identifying new uses for existing drugs that have already been approved by regulatory agencies for other indications. The advantage of drug repurposing is that it can significantly reduce the time and cost associated with drug development, as the safety and pharmacokinetic profiles of these drugs are already known. Furthermore, drug repurposing can offer new treatment options for diseases that currently lack effective therapies.

Several drugs that were originally developed for other indications have shown promise in preclinical and clinical studies for the treatment of ADPKD. For example, several inhibitors of the Mammalian Target of Rapamycin (mTOR), including rapamycin and everolimus, have been shown to reduce cyst growth and improve kidney function in animal models of ADPKD. These drugs are already approved for the treatment of other conditions, such as cancer and organ transplantation, and their safety profiles are well-established.

Another promising drug for the treatment of ADPKD is tolvaptan, a vasopressin V2 receptor antagonist that has been shown to slow the rate of kidney function decline in patients with ADPKD. Tolvaptan works by reducing the production of cyclic Adenosine Monophosphate (cAMP), a signaling molecule that promotes cyst growth in ADPKD. Tolvaptan was approved by the US Food and Drug Administration (FDA) for the treatment of ADPKD in 2018 based on the results of two randomized clinical trials that demonstrated its efficacy in slowing disease progression.

In addition to mTOR inhibitors and tolvaptan, other drugs have shown promise in preclinical studies for the treatment of ADPKD. For example, statins, which are commonly used to lower cholesterol levels, have been shown to reduce cyst growth in animal models of ADPKD by inhibiting the mevalonate pathway, a metabolic pathway that is involved in the regulation of cell growth and proliferation. Similarly, inhibitors of the Renin-Angiotensin-Aldosterone System (RAAS), such as Angiotensin-Converting Enzyme (ACE) inhibitors and Angiotensin Receptor Blockers (ARBs), have been shown to reduce cyst growth and improve kidney function in animal models of ADPKD.

Despite the promising results of preclinical and clinical studies, several challenges remain in the development of effective therapies for ADPKD. One of the major challenges is the heterogeneity of the disease, as different mutations in the PC1 and PC2 genes can lead to different disease phenotypes. Therefore, it is important to identify biomarkers that can predict the response to specific therapies and stratify patients based on their disease phenotype.

Another challenge is the lack of validated animal models that recapitulate the human disease phenotype. Most animal models of ADPKD rely on the overexpression or deletion of the PC1 or PC2 genes, which may not accurately reflect the complex genetic and environmental factors that contribute to disease development and progression in humans. Therefore, it is important to develop more relevant animal models that can better predict the efficacy and safety of potential therapies for ADPKD.

Drug repurposing, the process of using an existing drug for a new indication, has emerged as a promising strategy for developing new treatments for ADPKD. Several drugs have been identified as

Correspondence to: Michael Pove, Department of Medicine, University of California, San Francisco, United States of America, E-mail: michaelpove@gmail.com

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potential candidates for repurposing based on their ability to target cellular pathways and molecular mechanisms involved in ADPKD pathogenesis. Among these drugs, the vasopressin V2 receptor antagonist tolvaptan has been approved for the treatment of ADPKD in several countries, including the United States, Japan, and Europe. Tolvaptan has been shown to slow kidney function decline in ADPKD patients, but its use is associated with adverse effects, such as liver toxicity, which limit its widespread use. Other drugs that have shown promise for repurposing in ADPKD include somatostatin analogs, mTOR inhibitors, and statins.

CONCLUSION

In conclusion, the concept of drug repurposing represents a promising approach for developing new treatments for ADPKD. Tolvaptan is currently the only drug approved for ADPKD, but other drugs hold promise for future development.

However, further research is needed to identify and evaluate new drug candidates, and to address the challenges associated with drug development in rare diseases like ADPKD.