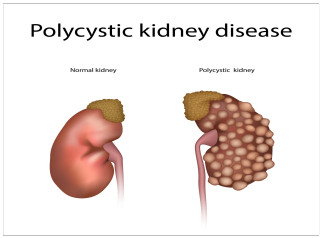
TMEM16A inhibitors as a Polycystic kidney disease (PKD) treatment option

Polycystic Kidney Disease (PKD) comprises a group of inherited disorders that lead to multiple fluid-filled renal cysts. The most common form, autosomal dominant PKD (ADPKD), affects 1 in 1000 people, and accounts for 10% of end-stage renal disease, which often necessitates long term treatment, dialysis and/or kidney transplantation.



TMEM16A inhibitors include approved and well tolerated drugs

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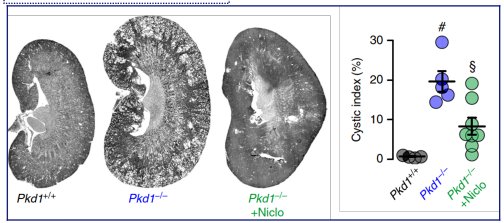
PKD is characterized by increased TMEM16A expression in kidney cells. Inhibition of TMEM16A can be achieved by using biologically active derivatives of benzbromarone or niclosamide.

01 TMEM16A inhibitors include approved and well-tolerated drugs such as niclosamide and benzbromarone
02 Significant suppression of renal cysts
03 Combinable with other strategies

REFERENCES:

https://doi.org/10.1038/s41467-020-18104-5



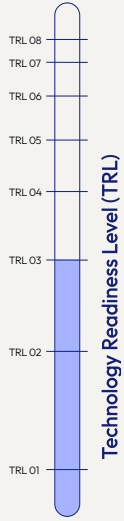
Niclosamide inhibits polycystic kidney disease in a mouse model for ADPKD
Representative images of kidneys from non-induced (Pkd1+/+) and induced (Pkd1−/−) mice, untreated or treated with niclosamide. Similar results were obtained with Benzbromaron and by tubule-specific deletion of Tmem16a

CHALLENGE

The standard treatment for early stages of PKD is usually symptomatic. Approximately 50% of ADPKD patients require dialysis treatment before the age of 60 , which is associated with reduced life expectancy. While a recently approved vasopressin antagonist has been shown to reduce cyst growth, dialysis and kidney transplantation are still required in advanced stages of PKD. Therefore, therapeutic options for delaying or preventing dialysis and targeting the advanced stages of PKD, are urgently needed.

INNOVATION

The Ca2+-regulated chloride ion channel TMEM16A is central to ADPKD. Inhibition of TMEM16A by inhibitors such as the FDA-approved and well-tolerated drugs niclosamide and benzbromarone largely suppress cyst development, as demonstrated in preclinical studies in-vivo. A large number of patients would be likely to benefit from this novel therapeutic concept for the treatment of ADPKD. It could strongly reduce the costs for public health care and lower the patient's burden caused by invasive medical treatments.



01 Basic principles observed · 02 Technology concept formulated · 03 Experimental proof of concept · 04 Technology validated in lab · 05 Technology validated in relevant environment · 06 Technology demonstrated in relevant environment · 07 System prototype demonstrated in operational environment · 08 System complete and qualified

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Bayerische Patentallianz GmbH
Dr. Katrin Bercht
kbercht@baypat.de
+49 (0)89 5480177-16
Prinzregentenstr. 52
80538 München