TMEM16A inhibitors as a treatment option for polycystic kidney disease (PKD)

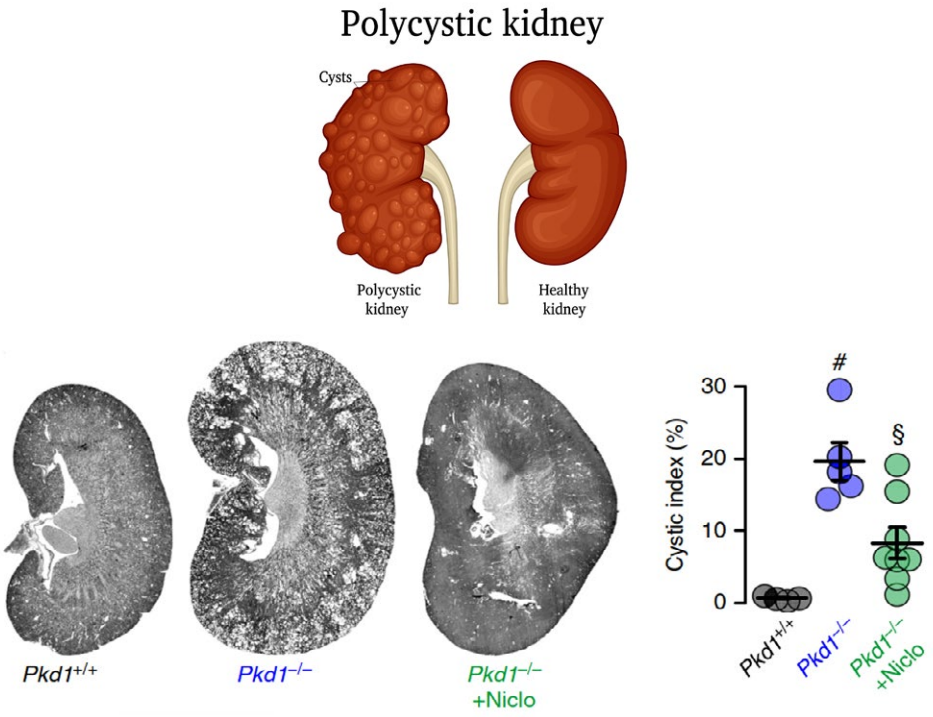
Reference No: B79076

CHALLENGE

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.

INNOVATION

The Ca^{2+}-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.



Niclosamide inhibits polycystic kidney disease in a mouse model for ADPKD

Representative images of kidneys from non-induced (Pkd1+/+) (n=4 animals) and induced (Pkd1−/−) mice, untreated (n =5 animals) or treated with niclosamide (n=8 animals). Scale bar 5000 µm.

COMMERCIAL OPPORTUNITIES

- TMEM16A inhibitors include FDA approved and well-tolerated drugs such as niclosamide and benzbromarone
- TMEM16A inhibitors represent a causative treatment for ADPKD
- Significant suppression of renal cysts

DEVELOPMENT STATUS

Efficacy shown *in-vivo*. Currently seeking partners for further development and licensing.

REFERENCES:

1 Cabrita I et al. "*Cyst growth in ADPKD is prevented by pharmacological and genetic inhibition of TMEM16A in-vivo*". Nat Commun. 2020 Aug 28;11(1):4320.

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