OCR 7602: New modality for the treatment of Autosomal Dominant Polycystic Kidney Disease

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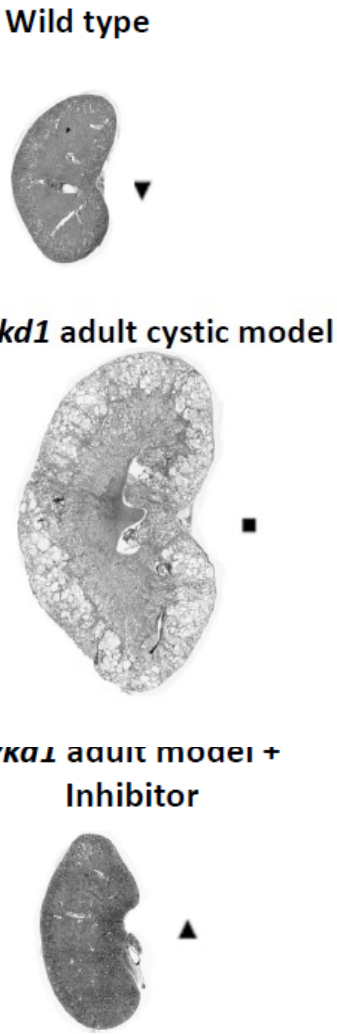
Background

- Autosomal Dominant Polycystic Kidney Disease (ADPKD) affects >600,000 in US population; 12.5 M worldwide
- ~4% of prevalent End-Stage Renal Disease (ESRD)
- ADPKD has **orphan condition designation** (2012) with estimated prevalence in US 1:2000
- One **approved therapy**: Tolvaptan (Jinarc) – approved April, 2018
- Targets low level proliferation and secretion in cysts originating from collecting duct; unknown long term efficacy and significant side-effects including liver toxicity (Hy's law)

Innovation

- Identified the Ireα-Xbp1 pathway as a modulator of cyst growth
- Inhibition of this pathway at the genetic level slows down disease progression in orthologous animal models through specific apoptosis of mutant cells
- Generated a pre-clinical efficacy package around a novel use for an Ireα inhibitor previously tested in human trials



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