

Mouse kidney with PKD Image: Dr Allara Zylberberg

ADVANCING POLYCYSTIC KIDNEY DISEASE TREATMENTS: THE RISE OF XCYSTENCE BIO

SMALL MOLECULE THERAPEUTICS

Groundbreaking research into Polycystic Kidney
Disease (PKD) —an inherited condition that often
leads to renal failure and requires dialysis or
transplant, has led to the creation of xCystence Bio, a
new biotech spin-out from Monash University. This
venture builds on discoveries made by researchers
from the Monash Biomedicine Discovery Institute
(BDI) and the Monash Institute of Pharmaceutical
Sciences (MIPS), who identified a key cell signalling
pathway driving the formation and growth of kidney
cysts. Founders include BDI's Prof Ian Smyth and Dr
Denny Cottle, along with Prof Paul Stupple and Dr
Yichao Zhao from MIPS.

TIA's Pipeline Accelerator scheme played a pivotal role by providing access to the Australian Translational Medicinal Chemistry Facility and the Centre for Drug Candidate Optimisation for efficacy testing and drug interaction studies. This support enabled the team to translate their findings into targeted therapeutic candidates with the potential to slow or halt disease progression by advancing the project toward Phase I trials and supporting the development of a diversified portfolio of compounds.

Backed by a \$500,000 CUREator grant from the Medical Research Future Fund, a \$1.1M NHMRC Ideas Grant and a \$891,000 NHMRC Development Grant, xCystence Bio is working to develop new

treatments for PKD. The team recently received an additional \$250,000 CUREator top-up funding, recognising the substantial progress made by the team and further empowering their efforts to translate innovations into better health outcomes. The company's mission is clear: to bring safe and effective therapies to patients and improve their quality of life.

With the additional support of the Phenomics
Australia Monash Genome Modification Platform
node, the team will be able to accelerate their
therapeutic screening process by studying
pharmacodynamics in CRISPR-engineered mice. TIA's
early support has been instrumental in helping the
team move from proof-of-concept research to
establishing a viable commercial venture with
promising clinical potential.

"The TIA Voucher Program was critical in facilitating the transition from biological discovery to drug development. It allowed us to collaborate with talented scientists expert in medicinal chemistry and compound profiling who have helped us to realise our translational aspirations."

Prof Ian Smyth

Impact:















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