Market-Ready Description: Targeted disruption of PKD1 gene inactivation

Early Intervention for Polycystic Kidney Disease

This technology is an early interventional therapeutic used to block the initiation of autosomal dominant polycystic kidney disease (ADPKD). This therapeutic destabilizes DNA structural changes called G4 DNA that cause mutations and kidney cyst formation. ADPKD is a lethal disease characterized by large numbers of steadily growing kidney and liver cysts with no effective treatment to stop progression to kidney failure. This is an autosomal dominant inherited disease, so there is a 50% chance of a child inheriting it from an affected parent. Patients generally become symptomatic at 30-40 years of age, but genetic screening is used to identify patients earlier in families where ADPKD is common. The treatment may be useful to prevent cysts forming at any time, with the highest efficacy predicted to be during fetal development or early childhood. ADPKD affects more than 600,000 patients in the US and 12.4 million globally.

Potential Benefits

- **Prevent cyst formation –** This therapeutic could block the initial stages of ADPKD, compared to current therapy that attempts to slow cyst growth.
- **Improve patient outcomes –** By reducing or eliminating cysts, this technology could preserve kidney function in ADPKD patients.

Potential Applications

- ADPKD
- Additional forms of the technology can be applied to genes where G4 DNA is associated with cyst formation and other defects caused by G4 quadruplex structures.