Market-Ready Description: Fluorescent Microparticle Assay for Protein Aggregation

Unique Assay Quickly Identifies Protein Aggregates, Qualitatively and Quantitatively

This is a novel assay that leverages monoclonal antibody specificity to efficiently identify protein aggregates' amount, and size, providing both qualitative and quantitative information to investigators. The technology is a microparticle immunocapture fluorescence assay to identify and quantify protein aggregates faster with more accurate results. Work to date has successfully shown the method to be highly reproducible with sensitivity comparable to or exceeding existing standards, while providing a broader range of size quantification. The cost-effective assay is an all-in-one method or kit that uses flow cytometry and can be a tool used by investigators at the bench to collected enhanced data. Another application for pharmaceutical manufacturers, is as a component of the quality control process to show proteins have not precipitated or aggregated in therapeutic preparations.

Clinically, this technology can be introduced for diagnosis and monitoring of disease associated aggregates, especially for early-stage diagnosis of proteinopathies. This assay can be applied to patients' cerebral spinal fluid, peripheral blood, and tissue preparations as part of routine annual lab work and could increase the likelihood that protein aggregates associated with neurodegenerative diseases would be identified earlier than with the existing standards, and without a significant increase to healthcare costs as the technological resources required to complete the assay are already in place in most clinical research lab settings.

Potential Benefits

- **Faster –** singular assay provides qualitative and quantitative data within a day
- **Accurate –** provides specificity for protein of interest and determines protein aggregates size
- **Early Diagnosis –** may identify protein aggregates associated with neurodegenerative diseases earlier if assay is adopted into routine diagnostic lab orders

Potential Applications

- Research tool in basic and translational research
- Diagnostic tool for multiple proteinopathies

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.