The scope of this proposed biomarker collaborative research for NAFLD and NASH will encompass development of diagnostic and staging biomarkers that can separate NASH from NAFLD, and more specifically, identify the presence and severity of inflammation and fibrosis. This will serve to help identify the most appropriate population for a particular treatment. Secondly, development of biomarkers that can predict progression. It is recognized that in general, NASH progresses slowly toward End-Stage Liver Disease (ESLD). However, there do appear to be subgroups that progress fairly rapidly. Accordingly, there is a need to qualify biomarkers that can identify “fast progressors” amongst those who meet criteria for a diagnosis of NASH. Finally, the ultimate goal of this biomarker research is to provide evidence supporting the regulatory acceptance of non-invasive biomarkers as surrogates for clinically important endpoints. It will be a long-term goal of this collaborative research to validate biomarkers that can reliably identify and predict risk for cirrhosis and complications of ESLD (e.g. varices, ascites) and that can reliably identify and predict risk for hepatocellular carcinoma (HCC).

The central challenge to be addressed is to establish and validate non-invasive markers for classifying and evaluating subjects with NAFLD, and in particular to identify amongst those with NAFLD, individuals who have Nonalcoholic steatohepatitis (NASH). Identifying and validating these biomarkers will be crucial in furthering an understanding of the factors that drive the heterogeneity of NAFLD and in enabling accurate diagnosis of those with NASH. The ultimate challenge that will be addressed is to validate one or more NAFLD biomarkers that can serve as surrogates for development of clinically meaningful outcomes.

It is expected that this program to identify and qualify non-invasive biomarkers for NAFLD and NASH will be transformative for clinical management of patients and profoundly enabling for drug development for treatment of NASH. Accurate diagnosis and effective treatment are the twin pillars that support medical practice and the unmet need that is present with regard to NAFLD and NASH cannot be effectively addressed without the elucidation of validated biomarkers.