Targeted Diagnostics for Neurodegenerative Diseases

**DISEASE FOCUS:** Neurology

**TECHNOLOGY PLATFORM:** Brain-enriched microRNA biomarker signatures in blood

**ONE PLATFORM, MULTIPLE INDICATIONS:** enabling early, differential diagnosis of neurodegenerative and neurological (including rare) diseases; strong potential for other disease areas and aging

**LEAD PRODUCT:** CogniMIR™ for early detection of Mild Cognitive Impairment – clinical assay in development

**COLLABORATIONS:** 4 leading US-based academic centers

**IP:** U.S. Patent No. 8,648,017 (issued in 2014) provides broad coverage for DiamiR methods for diagnosis and monitoring of neurodegenerative diseases; five additional families of patent applications are in prosecution

**VALUE DRIVERS:** Data from several studies are expected in the next 12 – 18 months

**FUNDING TO DATE:** Founders, non-dilutive funding from National Institute on Aging, Michael J Fox Foundation for Parkinson’s Research, a rare disease foundation

**YEAR FOUNDED:** 2009

**COMPANY:** DiamiR develops minimally invasive tests for early detection and monitoring of Alzheimer’s and other neurodegenerative and neurological diseases.

**APPLICATIONS:** Patient screening / stratification for clinical trials; prediction of disease progression; monitoring treatment response.

**MILD COGNITIVE IMPAIRMENT (MCI), ALZHEIMER’S DISEASE (AD):**

5.4M Americans have AD; AD is sixth leading cause of death in the US; current US healthcare cost of AD and other dementia: $220B/yr. 10-20% of 65+ year olds have MCI, 15% of MCI patients convert to AD each year. 99.6% of clinical drug candidates for AD fail, likely due to massive neuronal death occurring by the advanced stage of AD; stratified analyses of Phase III trials reveal promising results for earlier stage patients. Minimally invasive screening and predictive tests are urgently needed.

**COMMERCIAL OPPORTUNITY:** (i) Clinical trials are increasingly large, long, expensive, and focused on preclinical dementia; 70+ clinical trials on MCI/AD, ~240 patients/trial, initiated in the US each year. (ii) Large numbers of subjects are screened to enroll for trials; high screen failure rate (~50% for mild to moderate AD, >80% for preclinical AD) results in lengthy recruitment; significantly increases the cost of trials. (iii) Current diagnostic tools: neuroimaging, cerebrospinal fluid analysis are expensive, invasive, and not suitable for primary screening. (iv) Potential partners: pharmaceutical, biotech, diagnostics companies; clinical CROs.

**TECHNOLOGY:** DiamiR’s approach is focused on detecting pathology at synaptic dysfunction stage, which precedes clinical symptoms. The approach involves: (1) extensive data mining for synapse / brain-enriched microRNAs (miRNAs) detectable in plasma; (2) RT-qPCR of plasma levels of pre-selected 30–50 brain-enriched miRNAs; (3) selection of effective miRNA pairs using proprietary software; (4) biomarker signatures validation in independent cohorts of patients and controls; and (5) test development under Clinical Laboratory Improvement Amendments (CLIA) regulations.

**CogniMIR™ VALIDATION:** Pilot, feasibility, small longitudinal, and validation studies with independent plasma sample cohorts completed; two miRNA biomarker families, miR-132 and miR-134, differentiating MCI from age-matched control with 96% and 87% accuracy (p < 1e-11) identified; progression to MCI is predicted with 84% accuracy 1 – 5 years prior to diagnosis. Several studies on early MCI detection and MCI to AD progression are on-going.

**MANAGEMENT:** CEO: Kira Sheinerman, PhD, MBA, formerly served as Managing Director, Healthcare Investment Banking at Burrill & Company and Rodman & Renshaw. CSO: Samuil Umansky, MD, PhD, is an experienced MDx entrepreneur and inventor; formerly co-founder and CSO of Trovagene. VP Informatics: Vladimir Tsvinsky, PhD, is an expert in biostatistics and software development with 30 years experience.