

Courtagen Life Sciences Announces Collaboration with Raptor Pharmaceuticals for Leigh Syndrome and Other Mitochondrial Disorders

Courtagen Life Sciences to Provide Genetic Profiling of Patients Enrolling in the RP103-MITO-001 Clinical Trial

WOBURN, MA, March 25, 2014 – Courtagen Life Sciences announced today an agreement with Raptor Pharmaceuticals Inc. to provide **genetic testing** in the **clinical trial** for RP103 as a potential treatment for **Leigh syndrome** and other **mitochondrial disorders**. Leigh syndrome is a severe neurological disorder caused by genetic defects in mitochondrial or nuclear DNA affecting respiratory chain function that typically results in death within the first decade of life.

“The genetic profiling of patients is expected to elucidate important information beneficial to Raptor’s clinical trial for RP103,” said Brian McKernan, CEO of Courtagen. “We are pleased to work with Raptor Pharmaceuticals and believe that Courtagen’s mtSEEK[®] and nucSEEK[®] Next Generation Sequencing tests offer the most comprehensive mitochondrial analysis for such clinical trial work.”

About Courtagen Life Sciences, Inc.

Courtagen is a privately-held life science company with a world-class Next Generation Sequencing and multiplex immunoassay operation that converts genomic data into actionable clinical information for critical pediatric neurological and metabolic disorders. Specifically, Courtagen focuses on mitochondrial disorders, epilepsy, and autism spectrum disorders. Courtagen’s CLIA laboratory integrates genotype, phenotype, and disease mechanism data using cloud-based computing and custom analytical methods to provide the most comprehensive results for clinicians and patients to better understand and treat disease. For additional information, please visit www.courtagen.com.

About Raptor Pharmaceuticals

Raptor Pharmaceuticals Inc. is a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat rare, debilitating and often fatal diseases. The company's first product, PROCYSBI[®] (cysteamine bitartrate) delayed-release capsules, is FDA approved for the management of nephropathic cystinosis in adults and children 6 years and older. The product is also approved by the European Commission for marketing in the EU as PROCYSBI gastro-resistant hard capsules of cysteamine (mercaptamine bitartrate), for the treatment of proven nephropathic cystinosis. Raptor's pipeline also includes RP103 in a Phase 2/3 trial for Huntington's disease and a Phase 2b trial in nonalcoholic fatty liver disease in children. PROCYSBI was granted orphan designation and exclusivity for nephropathic cystinosis in the U.S.

and EU, and RP103 has received U.S. orphan drug designation for Huntington's disease. For additional information, please visit www.raptorpharma.com.

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