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QR Pharma Reports Results from SBIR Grant by NIA/NIH to Study Posiphen® and Metabolites in Various Models

Berwyn, PA, October 13, 2011 QR Pharma, Inc., a clinical-stage specialty pharmaceutical company committed to developing therapeutics with novel approaches for the treatment of cognitive impairment and Alzheimer's disease (AD), along with the Medical University of South Carolina (MUSC) report on the results obtained from the Small Business Innovation Research (SBIR) phase I grant study on Posiphen®. The grant complements QR's study in mildly cognitive impaired (MCI) patients and shows that in all systems tested: tissue culture cells, AD transgenic mice and MCI patients Posiphen reproducibly has the same effects.

Posiphen is a small orally available molecule with a high brain to plasma ratio (7:1), which inhibits the synthesis of amyloid precursor protein (APP) and Tau. QR and MUSC's data show that Posiphen lowers the levels of APP and Tau by approximately 50% in tissue culture cells, AD transgenic mice and MCI patients consistent with its mechanism of action. The effects last for over 12 hours consistent with its half-life in brain and CSF.

Posiphen is distinct from other AD drugs currently in development, because it inhibits the formation of the toxic proteins leading to AD. After 10 days of Posiphen administration CSF levels of secreted APP- α and - β , together with the AD biomarkers Tau and phosphorylated Tau (pTau) and inflammatory markers were lowered back to the levels found in healthy volunteers. Comparable data was found in AD transgenic mice treated with Posiphen for 10 days.

"APP mutations and duplications cause Familial Alzheimer's disease. APP triplication causes Down syndrome and high levels of APP after head trauma, stroke and brain injury lead to neurodegeneration," said Kumar Sambamurti, PhD, Professor of Neurosciences at MUSC. "Posiphen, an APP-lowering compound, targets all the toxic fragments cleaved from APP and therefore provide benefits beyond those realized by compounds that target A β . Posiphen also targets Tau, which further contributes to the toxicity in AD".

About Posiphen®, QR's Lead Compound Posiphen® is a small orally active compound with high blood brain barrier permeability, which lowers levels of toxic protein aggregates. It targets the mRNA of a number of proteins that are overexpressed in several neurological disorders such as Alzheimer's disease, Parkinson's disease and Down syndrome. It is in clinical development as an oral treatment for Alzheimer's disease. Posiphen reduces the rate of synthesis of amyloid precursor protein (APP) in cell cultures, normal, transgenic and trisomic mice. APP is cleaved into a number of toxic peptides, including A β 42, which is cleaved from the middle while the others are cleaved from the N- and C- terminal ends. These peptides attack multiple pathways of neuronal cell life leading to synaptic loss and nerve cell death. This degeneration of the brain induces dysfunction, neuroinflammation and leads to cognitive impairment and neurodegeneration.

QR conducted a trial in patients with mild cognitive impairment to confirm Posiphen's mechanism of action in humans and correlate it with the pharmacokinetics of the compound and its metabolites in CSF and plasma. Posiphen lowers levels of APP, tau and inflammation by about 50%, approaching the levels found in healthy volunteers.

We conducted a trial in MCI patients to confirm this mechanism of action (reduced rate of APP synthesis) in humans and correlate it with the pharmacokinetics of the drug and its metabolites in CSF and plasma. We found that Posiphen® and metabolites enter the brain readily and show a 2 to 2.5 time longer half-life in brain than in plasma, leading to prolonged

efficacy and possible once a day dosing.

About QR Pharma, Inc. Headquartered in Berwyn, Pennsylvania, QR Pharma, Inc. is a clinical-stage specialty pharmaceutical company committed to developing therapeutics with novel approaches for the treatment of cognitive impairment, Alzheimer's disease (AD), Parkinson's disease (PD) and Down syndrome (DS). QR currently has three product development programs - Posiphen for early stage AD and PD and BNC for advanced AD. For more information on QR Pharma, please visit the company's website, www.qrpharma.com.

About the Neurosciences Laboratory at MUSC The Sambamurti laboratory has a long-standing interest in understanding the biochemical basis of Alzheimer disease and its relationship to other neurodegenerative diseases. The laboratory has proposed a hypothesis that failure of membrane protein turnover (including APP) is the triggering event in Alzheimer disease pathogenesis. Thus, rather than viewing the amyloid peptide as a target for reduction, the lab focuses on facilitation of the turnover pathways for APP and other membrane proteins. Posiphen constitutes a novel category of agents that aim to reduce the synthesis of APP, thereby reducing the load of this protein on its turnover pathways. The hypothesis extends to other degenerative diseases, including age-related macular degeneration and glaucoma. Dr. Sambamurti is also the Co-Director of the Carol Campbell brain bank.

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