

QR Pharma Awarded SBIR Grant by NIA/NIH to Test Posiphen® and Metabolites in Various Models

September 21, 2010, [QR Pharma, Inc.](#) (QR), Radnor, PA : QR a clinical-stage specialty pharmaceutical company committed to developing therapeutics with novel approaches for the treatment of cognitive impairment and Alzheimer's disease and the Medical University of South Carolina (MUSC) announced today that they received a Small Business Innovation Research (SBIR) phase I grant in the amount of \$220,000 to study Posiphen® and its metabolites in cell culture and in transgenic Alzheimer mice.

[Posiphen](#), a small orally active compound, has been shown in cell cultures and in normal mice, AD transgenic mice and Down syndrome (DS) mice as well as in humans to reduce the synthesis of amyloid- β precursor protein (APP), which is cleaved into a number of toxic peptides. These peptides include N- and C-terminal fragments and amyloid-beta 42 (A β 42) that is widely believed to be responsible for Alzheimer disease pathogenesis.

Posiphen was found to be metabolized into three major metabolites. The primary activity is APP inhibition; it is exhibited by Posiphen as well as all three metabolites. The other two activities, acetylcholinesterase inhibition and neuro-regeneration seem to be properties of one or another metabolite, but not of Posiphen. This grant will help evaluate the absolute and relative activities of Posiphen and metabolites as well as test their activities in transgenic mice.

"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, should target all the toxic fragments cleaved from APP and therefore provide benefits beyond those realized by compounds that target A β ".

"Finding these two additional activities of Posiphen and better defining them will help QR Pharma to better understand the efficacy of the compound in humans," said [Maria Maccecchini](#), PhD, CEO of QR Pharma. "The original APP inhibitory activity should give us disease modification, while acetylcholinesterase inhibition should lead to better short term improvements in cognition and neuro-regeneration could lead to a halt in the cognitive decline."

About QR Pharma, Inc. Headquartered in Radnor, PA, QR Pharma, Inc. is a clinical-stage specialty pharmaceutical company committed to developing therapeutics with novel approaches for the treatment of cognitive impairment and Alzheimer's disease (AD). QR currently has two product development programs based on oral small-molecule, blood-brain barrier passable therapeutics that target two distinct pathways for the treatment of AD. 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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