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## Massachusetts General Hospital and QR Pharma Start a Collaboration on Posiphen® and Parkinson's Disease

Radnor, PA, June 16, 2010: QR Pharma, Inc. (QR) and [Massachusetts General Hospital](#) announced today a new collaborative research agreement to work on a treatment for Parkinson's disease (PD).

Initially the collaboration will focus on testing QR's drug candidates in [Dr. Jack Rogers'](#)  $\alpha$ -Synuclein-based drug discovery assay for the treatment of [Parkinson's disease](#) (PD). Dr. Catherine Cahill, a Harvard faculty member and co-investigator, will conduct experiments in animal models of PD and assays on human clinical samples. [Posiphen®](#), QR's lead compound, inhibits amyloid precursor protein (APP) and, therefore, a major pathway leading to Alzheimer's disease (AD); it is in clinical development for the treatment of AD. The same compound was shown by Drs. Rogers and Cahill to inhibit  $\alpha$ -Synuclein, a major pathway leading to PD. "To further explore the mechanism," Dr. Cahill said: "We will take this observation a step further and explore Posiphen's effect in a mouse model of PD and then proceed to clinical trials. This is a new approach for the treatment of PD and we are looking forward to conduct in vivo studies."

Dr. Rogers' expertise is in regulation of protein synthesis via the iron regulatory element on 5'untranslated regions (5'UTR) of messenger RNA (mRNA).  $\alpha$ -Synuclein, amyloid precursor protein (APP), super oxide dismutase and prions are copper/zinc superoxide scavengers that can all form aggregates associated with neurodegeneration. These proteins share similar translation mechanisms with homologous 5'UTRs under iron regulatory element control. By inhibiting protein synthesis via this common mRNA regulatory pathway, [Posiphen®](#) may have a beneficial therapeutic effect by lowering the accumulation of these toxic proteins in the brain.

Dr. Rogers explains: "An inter-relationship exists between the Parkinson's disease causative protein,  $\alpha$ -Synuclein, and the Alzheimer's APP. We will pursue [Posiphen](#), a well-tolerated inhibitor of APP translation and amyloid burden, which also inhibits  $\alpha$ -Synuclein expression in cultured neural cells. Since  $\alpha$ -Synuclein mediates neurotoxicity in PD, we will test if [Posiphen®](#) exerts therapeutic action by acting as an inhibitor of  $\alpha$ -Synuclein expression in vivo. [Posiphen®](#) represses  $\alpha$ -Synuclein mRNA translation by its 5' leader sequence by interfering with the selective binding of an essential Iron Regulatory Protein, IRP-1, to the functional RNA enhancer in front of the  $\alpha$ -Synuclein messenger. This pathway is similar to the one established for [Posiphen's](#) inhibition of APP mRNA translation for anti-amyloid efficacy in AD. This novel pathway could provide therapeutic efficacy in Parkinson's disease."

"QR Pharma's technology targets the mRNA of a number of proteins that are overexpressed in several neurological disorders, such as AD, PD and Down Syndrome (DS), in a manner that has the potential to impact disease progression and provide symptomatic relief," said Maria Maccicchini, Ph.D., CEO of QR Pharma, a successful biotechnology veteran in the region. "In the last six months we entered [Posiphen®](#) into a clinical study in AD patients that measures whether [Posiphen®](#) enters the brain and once there, whether it shows the same mechanism of action as seen in tissue culture cells and in mice. At the same time we are progressing [Bisnorcymserine \(BNC\)](#), a centrally active, reversible butyrylcholinesterase inhibitor, into its first clinical safety trial."

About QR Pharma, Inc.: Headquartered in Radnor, Pennsylvania, 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. 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.

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