

QR Pharma's Posiphen® was shown in a Clinical Mechanism of Action Study to Enter the Brain and to Inhibit Amyloid Precursor Protein

Radnor PA July 14, 2010: [QR Pharma, Inc.](#), a developer of novel drugs to treat Alzheimer's disease (AD), is presenting a poster today on positive clinical data from its recent mechanism of action study in mild cognitive impaired (MCI) patients at the International Congress on Alzheimer's Disease ([ICAD 2010](#)).

[Posiphen®](#) is in clinical development as an oral treatment for Alzheimer's disease (AD). It is a small orally active compound that in rodents shows high blood brain barrier permeability. In cell cultures, normal, transgenic and trisomic mice Posiphen lowers amyloid- β precursor protein (APP) levels by inhibiting the rate of APP messenger RNA synthesis.

APP is cleaved into a number of toxic peptides, one of them being amyloid- β_{42} (A β_{42}), the others being 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 induces dysfunction, neuroinflammation, and leads to cognitive impairment and neurodegeneration.

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.

We were also able to show that Posiphen lowers the levels of APP in plasma and CSF proving that its mechanism of action in humans is inhibition of APP. The study allowed us to confirm the mechanism of action in humans and to narrow down potential dose levels and dosing regimens.

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, in diseases such as Alzheimer's disease (AD), Parkinson's disease (PD) and Down Syndrome (DS). 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

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