

QR Pharma Awarded Funding by Michael J. Fox Foundation to Test Posiphen® as a Treatment for Parkinson's Disease

Berwyn, PA, March 6, 2012 [QR Pharma, Inc.](#) (QR) a clinical stage specialty pharmaceutical company committed to developing therapeutics with novel approaches for the treatment of [Alzheimer's disease](#), [Parkinson's disease](#) (PD) and other neurodegenerative disorders, announced today that The [Michael J. Fox Foundation](#) for Parkinson's Research (MJFF) awarded the company \$468,000 to conduct research for the development of Posiphen® to treat PD. QR will collaborate with [Robert Nussbaum](#), MD, Professor, Department of Medicine and Chief, Division of Medical Genetics at the University of California, San Francisco and [Jack T. Rogers](#), PhD, Associate Professor Psychiatry (Neuroscience) at the Massachusetts General Hospital, Genetics and Aging Research Unit.

Mutations and overexpression of alpha-synuclein (a-SYN) have been shown to cause familial PD while genetic association studies indicate a-SYN is a key risk factor in sporadic PD. Evidence suggests that therapies that can reduce a-SYN expression may block its pathogenic actions and therefore be useful in treating PD. In vitro studies conducted at Massachusetts General Hospital demonstrate that Posiphen lowers a-SYN levels by inhibiting a-SYN translation.

To establish efficacy in PD, Posiphen will be tested in transgenic mice engineered by Dr. Nussbaum's group at UCSF that express mutant human a-SYN. These mice exhibit early gastrointestinal dysfunction at three-months and motor abnormalities later in life, mimicking what is found in PD patients. Studies will be conducted to test, among other things, Posiphen's ability to reverse these abnormalities to establish pre-clinical efficacy as a basis for future testing in humans.

"Our animal model is based on the initial work we did 15 years ago identifying alpha-synuclein as the first human gene which, when mutated, causes Parkinson disease," said Dr. Robert Nussbaum. "Our model recapitulates the early signs of the disease and is well suited to test therapies directed toward regulating alpha-synuclein expression either on the gene or on the protein level."

"Alpha-synuclein is a high-priority target for our Foundation, as there is evidence that it plays an important role in both genetic and idiopathic cases of PD," said [Kuldip Dave, PhD](#), associate director of research programs at MJFF. "QR Pharma's drug Posiphen has been shown to block the synthesis of alpha-synuclein. By decreasing alpha-synuclein levels in the brain, Posiphen could potentially be a novel treatment for PD."

"We are pleased that The Michael J. Fox Foundation for Parkinson's Research sees the potential of Posiphen in the treatment of Parkinson's disease," said [Maria Maccacchini, Chief Executive Officer](#) of QR. "We look forward to working with the excellent research teams led by Dr. Nussbaum and Dr. Rogers."

About Posiphen®. QR's lead [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 as well as in humans. Posiphen also inhibits the synthesis of tau and alpha-synuclein in mice and humans. These neurotoxic aggregating proteins induce dysfunction, neuroinflammation and lead to cognitive impairment and neurodegeneration.

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 Michael J. Fox Foundation for Parkinson's Research.

As the world's largest private funder of Parkinson's research, The Michael J. Fox Foundation is dedicated to accelerating a cure for Parkinson's disease and improved therapies for those living with the condition today. The Foundation pursues its goals through an aggressively funded, highly targeted research program coupled with active global engagement of scientists, Parkinson's patients, business leaders, clinical trial participants, donors and volunteers. In addition to funding more than \$285 million in research to date, the Foundation has fundamentally altered the trajectory of progress toward a cure. Operating at the hub of worldwide Parkinson's research, the Foundation forges groundbreaking collaborations with industry leaders, academic scientists and government research funders; increases the flow of participants into Parkinson's disease clinical trials with its online tool, Fox Trial Finder; promotes Parkinson's awareness through high-profile advocacy, events and outreach; and coordinates the grassroots involvement of thousands of Team Fox members around the world. Now through December 31, 2012, all new and increased giving to The Michael J. Fox Foundation, as well as gifts from donors who have not given since 2010 or earlier, will be matched on a dollar-for-dollar basis with the \$50-million Brin Wojcicki Challenge, launched by Sergey Brin and Anne Wojcicki. For more information, visit: www.michaeljfox.org; www.facebook.com/michaeljfoxfoundation.