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Intra-Cellular Therapies Announces Initiation of Phase I/II Clinical Trial for ITI-007 in Healthy Geriatric Subjects and Patients with Dementia, Including Alzheimer's Disease

NEW YORK, March 3, 2014 /PRNewswire/ -- Intra-Cellular Therapies, Inc. (NASDAQ: ITCI), a biopharmaceutical company focused on the development of therapeutics for central nervous system (CNS) disorders, today announced the initiation of ITI-007-200, a Phase I/II clinical trial designed to evaluate the safety, tolerability and pharmacokinetics of low doses of its lead drug candidate, ITI-007, in healthy geriatric subjects and in patients with dementia, including Alzheimer's disease. The commencement of this study marks an important milestone in our strategy to develop low doses of ITI-007 for the treatment of behavioral disturbances associated with dementia and related disorders.

About the Phase I/II Clinical Trial

The ITI-007-200 trial is planned to be conducted in two parts. Part 1 is a randomized, double-blind, placebo-controlled multiple ascending dose evaluation of ITI-007 in healthy geriatric subjects. In each cohort in Part 1, we anticipate that ten subjects will be randomized to receive ITI-007 (N=8) or placebo (N=2) for seven days. In Part 2, we anticipate that twelve patients with dementia will be randomized to receive ITI-007 (N=9) or placebo (N=3) for seven days. The number of cohorts in each part may be adjusted based on results. Safety, tolerability and pharmacokinetic data will be determined. Exploratory pharmacodynamic endpoints will be included to assess feasibility of measuring agitation, sedation, sleep and cognition in potential future trials. We expect that initial data from the trial will be available in the second half of 2014.

About ITI-007

ITI-007 is our lead product candidate, whose mechanisms of action, we believe, have the potential to yield a first-in-class antipsychotic therapy and, at lower doses, a first-in-class therapy for the behavioral disturbances associated with dementia. In our pre-clinical and clinical trials to date, ITI-007 combines potent serotonin 5-HT_{2A} receptor antagonism, dopamine receptor phosphoprotein modulation (DPPM), glutamatergic modulation and serotonin reuptake inhibition into a single drug candidate. At dopamine D₂ receptors, ITI-007 has been demonstrated to have dual properties and to act as both a post-synaptic antagonist and a pre-synaptic partial agonist. ITI-007 has also been demonstrated to stimulate phosphorylation of glutamatergic NMDA NR2B, or GluN2B, receptors in a mesolimbic specific manner.

At the lowest doses studied to date, ITI-007 has been demonstrated to act primarily as a potent 5-HT_{2A} serotonin receptor antagonist. As the dose is increased, additional benefits are derived from the engagement of additional drug targets, including modest dopamine receptor modulation and modest inhibition of serotonin transporters. We believe that combined interactions at these receptors may provide additional benefits above and beyond selective 5-HT_{2A} antagonism for treating agitation, aggression and sleep disturbances in diseases that include dementia, Alzheimer's disease and autism spectrum disorders, while avoiding many of the side effects associated with more robust dopamine receptor antagonism. As the dose of ITI-007 is further increased, leading to moderate dopamine receptor modulation, inhibition of serotonin transporters, and indirect glutamate modulation, these actions complement the complete blockade of 5-HT_{2A} serotonin receptors. In this dose range, we believe that ITI-007 may be useful in treating the symptoms associated with schizophrenia, bipolar disorder, major depressive disorder and other neuropsychiatric diseases.

About Behavioral Disturbances in Dementia, Including Alzheimer's Disease

It has been estimated that 44.4 million people worldwide were living with dementia in 2013 including over five million patients with Alzheimer's disease in the United States. This number is expected to nearly double to 75.6 million by 2030 and to 135.5 million by 2050. While the diagnostic criteria for Alzheimer's disease and other dementias mostly focus on the related cognitive deficits, it is often the behavioral and psychiatric symptoms that are most troublesome for caregivers and lead to poor quality of life for patients. Several behavioral symptoms are quite prevalent in patients with dementia, including patients with Alzheimer's disease. Rates of depression in Alzheimer's disease are estimated to be up to 87%, although most estimates are between 30% and 50%. Agitation and aggression are present in approximately 60% of patients. Sleep disturbances, particularly as an increased likelihood of day-night reversal, are present in up to approximately 60% of patients. In view of the potential multiple effects of ITI-007 on aggression, agitation, sleep disorders and depression, and its safety profile to date, we believe that ITI-007 may provide a novel therapy for treating the behavioral disturbances accompanying dementia, including Alzheimer's disease.

The FDA has not approved any drug to treat the behavioral symptoms of dementia, including Alzheimer's disease. As symptoms progress and become more severe, physicians often resort to off-label use of antipsychotic medications in these

patients. Current antipsychotic drugs are associated with a number of side effects, which can be problematic for elderly patients with dementia. In addition, antipsychotic drugs may exacerbate the cognitive disturbances associated with dementia. There is a large unmet medical need for a safe and effective therapy to treat the behavioral symptoms in patients with dementia, including Alzheimer's disease.

About Intra-Cellular Therapies

Intra-Cellular Therapies (the "Company") is developing novel drugs for the treatment of neuropsychiatric and neurodegenerative disease and other disorders of the central nervous system ("CNS"). The Company is developing its lead drug candidate, ITI-007, for the treatment of schizophrenia, behavioral disturbances in dementia, bipolar disorder and other neuropsychiatric and neurological disorders. In December 2013, the Company announced positive topline results from the Company's randomized, placebo- and active-controlled Phase II clinical trial of ITI-007 in patients with acutely exacerbated schizophrenia. This study showed a statistically significant improvement in symptoms associated with schizophrenia at the 60 mg dose on the trial's pre-specified primary endpoint and a favorable safety profile. We believe ITI-007 may be able to improve the quality of life of patients with schizophrenia and enhance social function to allow them to integrate more fully into their families and their workplaces. The Company is also utilizing its phosphodiesterase platform and other proprietary chemistry platforms to develop drugs for the treatment of cognitive deficits in schizophrenia and other CNS disorders. The Company has partnered the lead compound, ITI-214, and backups from this PDE1 platform with the Takeda Pharmaceutical Company. ITI-214 has finished the first Phase 1 clinical trial and is now in subsequent Phase 1 trials. The Company is also developing inhibitors against additional targets for CNS indications such as Alzheimer's disease, Parkinson's disease and depression and non-CNS indications such as cardiovascular disease.

Forward-Looking Statements

This news release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 that involve risks and uncertainties that could cause actual results to be materially different from historical results or from any future results expressed or implied by such forward-looking statements. Such forward-looking statements include statements regarding, among other things, our plans to develop low doses of ITI-007 for the treatment of behavioral disturbances associated with dementia and related disorders; the plans for our Phase I/II clinical trial of ITI-007 in healthy geriatric subjects and in patients with dementia, including Alzheimer's disease; the potential for ITI-007 to yield a first-in-class antipsychotic therapy and, at lower doses, a first-in-class therapy for the behavioral disturbances associated with dementia, along with other statements about the potential for ITI-007 under the caption "About ITI-007"; the potential for ITI-007 to provide a novel therapy for treating the behavioral disturbances accompanying dementia, including Alzheimer's disease; the potential for ITI-007 to improve the quality of life of patients with schizophrenia and enhance social function to allow them to integrate more fully into their families and their workplaces; and our research and development efforts and plans under the caption "About Intra-Cellular Therapies." All such forward-looking statements are based on management's present expectations and are subject to certain factors, risks and uncertainties that may cause actual results, outcome of events, timing and performance to differ materially from those expressed or implied by such statements. These risks and uncertainties include, but are not limited to the following: our current and planned clinical trials for ITI-007 and our other product candidates may not be successful or may take longer and be more costly than anticipated; product candidates that appeared promising in earlier research and clinical trials may not demonstrate safety and/or efficacy in larger-scale or later clinical trials; our reliance on collaborative partners and other third-parties for development and commercialization of our product candidates; and the other risk factors discussed under the heading "Risk Factors" contained in our Current Report on Form 8-K filed with the Securities and Exchange Commission on October 31, 2013, as well as any updates to those risk factors filed from time to time in our periodic and current reports filed with the Securities and Exchange Commission. All statements contained in this press release are made only as of the date of this press release, and we do not intend to update this information unless required by law.

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