International Stem Cell Corporation Announces Successful Completion of Its Phase 1 Clinical Trial in Parkinson's Disease

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CARLSBAD, CA / ACCESSWIRE / June 30, 2021 / International Stem Cell Corporation (OTCQB:ISCO) (www.internationalstemcell.com) ("ISCO" or "the Company"), a California-based clinical stage biotechnology company developing stem cell-based therapies and biomedical products, announced today successful completion of its dose escalating phase 1 clinical trial (ClinicalTrials.gov Identifier: NCT02452723) evaluating the safety, tolerability and preliminary efficacy of its lead candidate, ISC-hpNSC® for the treatment of Parkinson's disease (PD).

Dr. Andrew Evans, M.D., Director of Movement Disorders at the Royal Melbourne Hospital, the study's principal investigator commented: "The safety evaluation is based on the initial 12 months of safety data from the first cohort (low dose), the second cohort (mid dose), and the third cohort (high dose). In all three cohorts there have been no serious adverse effects related to the transplanted ISC-hpNSC® cells. Based on all data collected in the clinical trial the therapy is considered safe."

In order to gain initial insight into what dose might show the greatest efficacy we have continued observations on a biannual basis of these patients. All the patients in the clinical trial have now completed at least 24 months of total post-operative observations. Patients treated with the mid dose (cohort 2) have been observed for at least 36 months and patients in the low dose group (Cohort 1) have been followed for 48 months post transplantation.

"We are excited about our phase 1 clinical trial results. Patients, followed for over two years after cell transplantation, have reported, on average, improvements in a Parkinson's Disease specific measures, when compared to baseline evaluations. In this context, the results are very encouraging that the ISC-hpNSC® transplanted cells are not only well tolerated, but also may be effective" commented Dr. Russell Kern, ISCO's Executive Vice President and Chief Scientific Officer.

In terms of preliminary efficacy, where scores are compared against baseline before transplantation, we observed a potential dose-dependent response, with an apparent peak effectiveness at our middle dose. The % OFF-Time, which is the time during the day when levodopa medication is not performing optimally and PD symptoms return, decreased an average 47% from the baseline at 12 months post transplantation in cohort 2. This trend continued through 24 months where the %OFF time in the second cohort dropped by 55% from the initial reading. The same was true for % ON-Time without dyskinesia, which is the time during the day when levodopa medication is performing optimally without dyskinesia. The % ON-Time increased an average of 42% above the initial evaluation at 12 months post-transplantation in the second cohort. The %ON result improved in the second cohort to 65% above the baseline in month 24. The quality of life of the patients as measured by the Parkinson's Disease Quality of Life Score-39 (PDQ-39) Summary Index, improved 43% for the second cohort at twelve months post-transplantation. This improved to a 45% better score in cohort 2 at 48 months.

About the clinical study

The Phase 1 clinical study is a dose escalation safety and preliminary efficacy study of ISC-hpNSC®, intracranially transplanted into patients with Parkinson's disease. The open-label, single center, uncontrolled clinical trial is evaluating three different dose regimens of 30 million to 70 million neural cells. A total of 12 participants with Parkinson's disease were treated. Following transplantation, the patients were monitored for 12 months at specified intervals to evaluate the safety and biologic activity of ISC-hpNSC®. A PET scan was performed at baseline, as part of the screening assessment, and at 6 and 12 months after surgical intervention. Clinical responses compared to baseline after the administration of ISC-hpNSC® will be evaluated using various neurological assessments such as Unified Parkinson Disease Rating Scale (UPDRS), Hoehn and Yahr as well as other rating scales. An extension phase of the study will evaluate patients every 6 months for 5 additional years.

About Parkinson's disease

Parkinson's disease is a degenerative disorder of the central nervous system mainly affecting the motor system. The motor symptoms of Parkinson's disease result from the death of dopamine-generating cells in the substantia nigra, a region of the midbrain. Early in the course of the disease, the most obvious symptoms are movement related. These symptoms include shaking, rigidity, slowness of movement and difficulty with walking and gait. Later, thinking and behavioral problems may arise, with dementia commonly occurring in the advanced stages of the disease. Depression is the most common psychiatric symptom. Parkinson's disease is more common in people over the age of 50. There are no approved treatments that restore the damaged dopaminergic neurons. Medications typically used in the treatment of Parkinson's disease, levodopa and dopamine agonists, improve the early symptoms of the disease. As the disease progresses and dopaminergic neurons continue to be lost, the drugs eventually become ineffective, while at the same time frequently producing a complication marked by involuntary writhing movements. There are over 10 million people afflicted with Parkinson's disease, worldwide. In 2013 Parkinson's disease resulted in about 103,000 deaths, globally. In 1990, the death toll recorded was 44,000.

About ISC-hpNSC®

International Stem Cell Corporation's proprietary ISC-hpNSC® consists of a highly pure population of neural stem cells derived from human parthenogenetic stem cells. ISC-hpNSC® is a suspension of clinical grade cells manufactured under cGMP conditions that have undergone stringent quality control measures and are free of any microbial and viral contaminants. Preclinical studies in rodents and non-human primates have shown improvement in Parkinson's disease symptoms and increase in brain dopamine levels following the intracranial administration of ISC-hpNSC®. ISC-hpNSC® provides neurotrophic support and neuroregeneration to the dying dopaminergic neurons of the recipient Parkinson's disease brain. Additionally, ISC-hpNSC® is safe, well tolerated and has shown not to cause adverse events such as dyskinesia, systemic toxicity or tumors in preclinical models. International Stem Cell Corporation believes that ISC-hpNSC® may have broad therapeutic applications for many neurological diseases affecting the brain, the spinal cord and the eye.

About International Stem Cell Corporation

International Stem Cell Corporation (ISCO) is focused on the therapeutic applications of human parthenogenetic stem cells (hpSCs) and the development and commercialization of cell-based research and cosmetic products. ISCO's core technology, parthenogenesis, results in the creation of pluripotent human stem cells from unfertilized oocytes (eggs). hpSCs avoid ethical issues associated with the use or destruction of viable human embryos. ISCO scientists have created the first parthenogenetic, homozygous stem cell line that can be a source of therapeutic cells for hundreds of millions of individuals of differing genders, ages and racial background with minimal immune rejection after transplantation. hpSCs offer the potential to create the first true stem cell bank, UniStemCell™. ISCO also produces and markets specialized cells and growth media for therapeutic research worldwide, through its subsidiary Lifeline Cell Technology (www.lifelinecelltech.com), and stem cell-based skin care products through its subsidiary Lifeline Skin Care (www.lifelineskincare.com). More information is available at www.internationalstemcell.com.

Safe harbor statement

Statements pertaining to anticipated developments, clinical studies expectations, potential additional applications for ISC-hpNSC®, and other opportunities for the Company and its subsidiaries, along with other statements about the future expectations, beliefs, goals, plans, or prospects expressed by management constitute forward-looking statements. Any statements that are not historical fact (including, but not limited to statements that contain words

such as "will," "believes," "plans," "anticipates," "expects," "estimates,") should also be considered to be forward-looking statements. Forward-looking statements involve risks and uncertainties, including, without limitation, risks inherent in the development and/or commercialization of potential products, regulatory approvals, need and ability to obtain future capital, application of capital resources among competing uses, and maintenance of intellectual property rights. Actual results may differ materially from the results anticipated in these forward-looking statements and as such should be evaluated together with the many uncertainties that affect the company's business, particularly those mentioned in the cautionary statements found in the Company's Securities and Exchange Commission filings. The Company disclaims any intent or obligation to update forward-looking statements.

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