September 12, 2016

Athersys Announces Acceptance by PMDA of Clinical Trial Notification in Japan for Treatment of Ischemic Stroke With MultiStem®

Study designed to provide confirmatory evidence of safety and effectiveness under Japan's accelerated regulatory pathway for qualified regenerative medicine therapies

CLEVELAND, Sept. 12, 2016 (GLOBE NEWSWIRE) -- Athersys, Inc. (Nasdaq:ATHX) announced today the successful completion of Japan's Pharmaceutical and Medical Devices Agency (PMDA) review of the Clinical Trial Notification (CTN), allowing the commencement by HEALIOS K.K. (Healios) of a confirmatory clinical trial evaluating the safety and efficacy of administration of MultiStem®, Athersys' novel cell therapy product, for the treatment of ischemic stroke in Japan (also designated by Healios as HLCM051 in Japan). In accordance with the regulatory system in Japan, a CTN is equivalent to an Investigational New Drug application, or IND, under the regulatory system used in the United States. This clinical trial to be conducted in Japan is part of a partnership and license agreement between Healios and Athersys, focused on the development and commercialization in Japan of novel cellular therapies, including MultiStem, for the treatment of ischemic stroke and potentially other indications. The study design was accepted as proposed to PMDA in the CTN.

"This announcement demonstrates exciting and important progress, achieved within less than eight months of launching the collaboration, and reflects the tremendous effort on the part of the teams at both organizations that are working together to advance this program in a highly focused and efficient manner," said Gil Van Bokkelen, Ph.D., Chairman and Chief Executive Officer of Athersys. "The development approach being taken by Healios and Athersys in Japan is consistent with the approach we plan to implement in a separate international study. Both studies are designed to provide the confirmatory evidence that we believe will put us in a strong position to obtain approval in one of the greatest areas of unmet clinical need in medicine today."

The planned study will be a randomized, double-blind, placebo-controlled clinical trial conducted at hospitals in Japan that have extensive experience at providing care for stroke victims. Based on the experience from the B01-02 study, subjects enrolled in the trial will receive either a single dose of MultiStem or placebo, administered within 18-36 hours of the occurrence of the stroke, in addition to standard of care. As previously disclosed, the study will evaluate patient recovery through approximately 90 days following initial treatment based on Excellent Outcome and other neurological, functional and clinical endpoints. Additional patient follow up will occur through one year, and other design elements of the Japan trial are consistent with a planned international Phase 3 study, and what has been described previously at the Stroke 2016 conference held in Sapporo earlier this year.

The trial in Japan follows a Phase 2 study completed by Athersys, referred to as the B01-02 trial, which was conducted at 33 clinical sites in the United States and United Kingdom. The study evaluated the safety and effectiveness of the intravenous administration of MultiStem cells within 24-48 hours after the occurrence of a moderate to severe stroke. The evaluable patient population comprised 126 subjects who were treated with MultiStem therapy or placebo.

As disclosed previously, intravenous administration of MultiStem following an ischemic stroke was well tolerated, consistent with observations from other clinical trials evaluating the safety of MultiStem treatment. Additionally, among all evaluable subjects in the B01-02 study, a greater percentage of subjects in the MultiStem group (15.4%) had an Excellent Outcome (mRS ≤1, NIHSS ≤1, and Barthel Index ≥95) at day 90 compared to the placebo group (6.6%) (p=0.10). At one year, a greater percentage of subjects in the MultiStem group had an Excellent Outcome compared to the placebo group (23.1% vs. 8.2%, p=0.02). For the MultiStem subjects treated within 36 hours of the stroke (i.e. the target population in the planned Japan study), the difference in Excellent Outcome was even greater (e.g., at one year, 29.0% vs. 8.2%, $p < 0.01$). Likewise, other measures of functional recovery, including the mRS distribution or "shift" analysis, biomarker data and clinical outcomes, including shorter hospitalization, less time in the Intensive Care Unit, and lower rates of mortality and life threatening adverse events, suggest that MultiStem treated patients experienced an improved recovery from post-stroke neurological complications.

"We are excited about reaching this point and about the degree of enthusiasm expressed for the study at the clinical

investigators meeting recently held in Tokyo. With the CTN now in effect, the next steps in the process prior to the initiation of enrollment include obtaining Institutional Review Board approval and finalizing agreements with each of the participating clinical sites. In the near term, Healios will be focusing on these activities, while Athersys provides support in other areas in preparation for the commencement of enrollment," concluded Dr. Van Bokkelen.

About Ischemic Stroke

Stroke represents an area where the clinical need is particularly significant, since it is a leading cause of death and serious disability worldwide, with a substantially impaired quality of life for many stroke victims. Currently, there are nearly 17 million people that suffer a stroke globally and more than two million stroke victims each year in the United States, Europe and Japan, combined. Ischemic strokes, which represent the most common form of stroke, are caused by a blockage of blood flow in the brain that cuts off the supply of oxygen and nutrients and can result in long-term or permanent disability due to neurological damage. Unfortunately, current therapeutic options for ischemic stroke victims are limited, since the only available therapies, administration of the clot dissolving agent tPA, or "thrombolytic," or surgical intervention using mechanical reperfusion to remove the clot, must be conducted within several hours of the occurrence of the stroke. As a consequence of this limited time window, only a small percentage of stroke victims are treated with the currently available therapy—most simply receive supportive or "palliative" care. The long-term costs of stroke are substantial, with many patients requiring extended hospitalization, extended physical therapy or rehabilitation (for those patients that are capable of entering such programs), and many require long-term institutional or family care.

About MultiStem

MultiStem cell therapy is a patented regenerative medicine product that has shown the ability to promote tissue repair and healing in a variety of ways, such as through the production of therapeutic factors produced in response to signals of inflammation and tissue damage. MultiStem therapy's potential for multidimensional therapeutic impact distinguishes it from traditional biopharmaceutical therapies focused on a single mechanism of benefit. The product represents a unique "off-the-shelf" stem cell product that can be manufactured in a scalable manner, may be stored for years in frozen form, and is administered without tissue matching or the need for immune suppression. Based upon its efficacy profile, its novel mechanisms of action, and a favorable and consistent safety profile demonstrated in both preclinical and clinical settings, MultiStem therapy could provide a meaningful benefit to patients, including those suffering from serious diseases and conditions with unmet medical need. Athersys has forged strategic partnerships and a broad network of collaborations to develop MultiStem cell therapy for a variety of indications, with an initial focus in the neurological, cardiovascular and inflammatory and immune disorder areas.

About Athersys

Athersys is an international biotechnology company engaged in the discovery and development of therapeutic product candidates designed to extend and enhance the quality of human life. The Company is developing its MultiStem® cell therapy product, a patented, adult-derived "off-the-shelf" stem cell product, initially for disease indications in the cardiovascular, neurological, inflammatory and immune disease areas, and has several ongoing clinical trials evaluating this potential regenerative medicine product. Athersys has forged strategic partnerships and collaborations with leading pharmaceutical and biotechnology companies, as well as world-renowned research institutions to further develop its platform and products. More information is available at www.athersys.com .

About HEALIOS K.K.

Healios is a biotechnology venture leading the field of developing iPS cell-based products for regenerative medicine. It was founded in 2011 and listed on the stock exchange (Tokyo Security Exchange Mothers: 4593) in 2015. In Japan, the company is developing a product for treatment of age-related macular degeneration (an intractable ocular disease) jointly with Sumitomo Dainippon Pharma Co., Ltd. In fields other than ophthalmology, the company has started R&D of products for regenerative medicine capable of creating functional human organs (three-dimensional organs) jointly with Yokohama City University. The company may be viewed as an enterprise providing products for regenerative medicine as a solution to the significant global issue "aging of the society." See the website (https://www.healios.co.jp/) for details.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 that involve risks and uncertainties. These forward-looking statements relate to, among other things, the expected timetable for development of our product candidates, our growth strategy, and our future financial performance, including our operations, economic performance, financial condition, prospects, and other future events. We have attempted to identify forward-looking statements by using such words as "anticipates," "believes," "can," "continue," "could," "estimates," "expects," "intends," "may," "plans," "potential," "should," "suggest," "will," or other similar expressions. These forward-looking statements are only predictions and are largely based on our current expectations. A number of known and

unknown risks, uncertainties, and other factors could affect the accuracy of these statements. Some of the more significant known risks that we face that could cause actual results to differ materially from those implied by forward-looking statements are the risks and uncertainties inherent in the process of discovering, developing, and commercializing products that are safe and effective for use as human therapeutics, such as the uncertainty regarding market acceptance of our product candidates and our ability to generate revenues, including MultiStem for the treatment of ischemic stroke, acute myocardial infarction, spinal cord injury and acute respiratory distress syndrome and other disease indications, including graft-versus-host disease. These risks may cause our actual results, levels of activity, performance, or achievements to differ materially from any future results, levels of activity, performance, or achievements expressed or implied by these forward-looking statements. Other important factors to consider in evaluating our forward-looking statements include: the success of our collaboration with Healios, our possible inability to realize commercially valuable discoveries in our collaborations with pharmaceutical and other biotechnology companies; the success of our collaborations, including our ability to reach milestones and receive milestone payments, including in connection with our collaboration with Healios, and whether any products are successfully developed and sold so that we earn royalty payments; our collaborators' ability to continue to fulfill their obligations under the terms of our collaboration agreements; the success of our efforts to enter into new strategic partnerships or collaborations and advance our programs; our ability to raise additional capital; results from our MultiStem clinical trials, including the clinical trial in Japan; the possibility of delays in, adverse results of, and excessive costs of the development process; our ability to successfully initiate and complete clinical trials; changes in external market factors; changes in our industry's overall performance; changes in our business strategy; our ability to protect our intellectual property portfolio; our possible inability to execute our strategy due to changes in our industry or the economy generally; changes in productivity and reliability of suppliers; and the success of our competitors and the emergence of new competitors. You should not place undue reliance on forward-looking statements contained in this press release, and we undertake no obligation to publicly update forward-looking statements, whether as a result of new information, future events or otherwise.

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Source: Athersys, Inc.