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HEALIOS Announces Enrollment of First Patient in Japan in TREASURE Study of MultiStem® Treatment for Ischemic Stroke

Off-the-shelf stem cell therapy has potential to extend treatment window and enhance recovery for stroke patients

CLEVELAND, Nov. 15, 2017 (GLOBE NEWSWIRE) -- Athersys, Inc. (NASDAQ:ATHX) announced today that its partner, HEALIOS K.K. ("Healios"), has enrolled the first patient in its study in Japan evaluating MultiStem® cell therapy treatment of patients who have suffered an ischemic stroke (the "TREASURE" study).

The TREASURE study is designed to enroll a total of 220 patients with moderate to severe strokes and will be randomized 1:1, with patients receiving a single intravenous infusion of MultiStem or placebo within 18 to 36 hours of the onset of the stroke. The primary efficacy outcome is the proportion of subjects achieving an Excellent Outcome at three months following treatment. Excellent Outcome is a commonly used measure of efficacy in stroke treatment, evaluated using three standard clinical rating scales of functional and neurological deficit and recovery following the stroke, including the NIH Stroke Scale, modified Rankin Scale and Barthel Index.

"We are pleased that the Healios TREASURE trial in Japan is now enrolling patients," stated Dr. Gil Van Bokkelen, Chairman and Chief Executive Officer of Athersys. "With a successful trial outcome, Japan's progressive regulatory framework for regenerative medicine products provides the potential for either conditional or full marketing approval. Additionally, the priority review designation obtained under the *Sakigake* framework is designed to speed the review by Japan's Pharmaceutical and Medical Devices Agency, allowing advancement of this program in a highly efficient manner."

"This is the largest stem cell trial initiated under the new regenerative medicine regulatory framework established in Japan, and it represents an important milestone," added Dr. Van Bokkelen. "We believe that MultiStem has the potential to meaningfully enhance recovery following a stroke and substantially extend the treatment window from several hours under current standard of care, which is only relevant to a small percentage of stroke patients, out to 36 hours, enabling treatment of a much larger number of people," concluded Dr. Van Bokkelen.

In parallel with the TREASURE trial, Athersys has been planning for its Phase 3 study entitled, *MultiStem Administration for Stroke Treatment and Enhanced Recovery Study-2* ("MASTERS-2"), to be conducted in North America and Europe. The MASTERS-2 study has received authorization from the U.S. Food and Drug Administration ("FDA") under a Special Protocol Assessment ("SPA") for the design and planned analysis of this pivotal clinical trial. The SPA provides formal agreement from the FDA that the protocol design, clinical endpoints, planned conduct and statistical analyses in this Phase 3 study are acceptable to support a regulatory submission for marketing approval of MultiStem cell therapy as a product for treating ischemic stroke patients. Additionally, the MultiStem stroke program has been awarded Fast Track designation by the FDA, meaning that the program is eligible for accelerated approval, priority review and rolling submission of the biologics license application, which facilitates a timely regulatory review.

Recently, in addition to the SPA and Fast Track designations from the FDA, the MASTERS-2 trial also obtained a Final Scientific Advice positive opinion from the European Medicines Agency, and received the Regenerative Medicine Advanced Therapy (RMAT) designation from the FDA, which was established this year under the 21st Century Cures Legislation. The RMAT designation may be obtained for eligible cell therapy and other regenerative medicine and advanced therapies when the FDA agrees that preliminary clinical evidence indicates that the therapy has demonstrated the potential to address unmet medical needs for a serious or life threatening disease or condition. The designation enables sponsors to discuss with the FDA multidisciplinary strategic development plans, including expediting manufacturing development plans for commercialization to support priority review and accelerated approval.

The MASTERS-2 clinical trial is planned to be a randomized, double-blind, placebo-controlled clinical trial designed to enroll 300 patients in North America and Europe who have suffered moderate to moderate-severe ischemic stroke. The enrolled

subjects will receive either a single intravenous dose of MultiStem cell therapy or placebo, administered within 18 to 36 hours of the occurrence of the stroke, in addition to the standard of care. The primary endpoint will evaluate disability using modified Rankin Scale (mRS) scores at three months, comparing the distribution, or the "shift," between the MultiStem treatment and placebo groups. The mRS shift analysis considers disability across the full spectrum, enabling recognition of large and small improvements in disability and differences in mortality and other serious outcomes, among strokes of different severities. The study will also assess Excellent Outcome (mRS ≤1, NIHSS ≤1, and Barthel Index ≥95) at three months and one year as key secondary endpoints. Additionally, the study will consider other measures of functional recovery, biomarker data and clinical outcomes, including hospitalization, mortality, life-threatening adverse events and post-stroke complications, such as infection.

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 who 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 treatments, administration of the clot dissolving agent tPA, or "thrombolytic," or surgical intervention 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. Follow Athersys on Twitter at www.twitter.com/athersys.

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 regulatory approval and 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 and others, including our ability to reach milestones and receive milestone payments, and whether any products are successfully developed and sold so that we earn royalty payments; our possible inability to realize commercially valuable discoveries in our collaborations with pharmaceutical and other biotechnology companies; 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 MASTERS-2 Phase 3 clinical trial and the TREASURE 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 within the expected time frame or at all; 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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