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One-Year Results From Phase 2 Stroke Study of MultiStem® Cell Therapy Demonstrate a Significantly Higher Rate of Complete or Nearly Full Recovery

Data Presented at 2016 International Stroke Conference Demonstrate Statistically Significant Improvement in Excellent Outcomes When Evaluating All Subjects and Key Subgroups

CLEVELAND, Feb. 17, 2016 (GLOBE NEWSWIRE) -- Athersys, Inc. (Nasdaq:ATHX) today announced positive results from the analysis of one-year follow-up data from its Phase 2 clinical study of the intravenous administration of MultiStem® cell therapy to treat patients who have suffered an ischemic stroke. Dr. David Hess, lead clinical investigator of this study and a stroke specialist and Chairman of the Department of Neurology at the Medical College of Georgia, Augusta University, presented the summary results today at the 2016 International Stroke Conference in Los Angeles. The one-year data demonstrates that MultiStem-treated subjects on average continued to improve through one year and had a significantly higher rate of "Excellent Outcome" (defined clinically as attaining mRS 0-1, NIHSS 0-1 and BI ≥95) compared to placebo subjects at one year when evaluating all subjects enrolled in the study (p=0.02), i.e., the intent-to-treat population. The relative improvement in Excellent Outcomes was even more pronounced in the patients who received MultiStem treatment within 36 hours of the stroke ($p < 0.01$).

"We are particularly excited by the one-year follow-up results because they show that MultiStem treatment can significantly increase the number of patients who have an Excellent Outcome, meaning complete or nearly full recovery, over the standard of care when considering all subjects in the trial," commented Dr. Gil Van Bokkelen, Chairman & CEO at Athersys. "The one-year data continues to confirm that MultiStem treatment is well tolerated and is associated with continued improvement of other measures of function through one year. As we saw in the 90-day interim analysis results announced last April, patients who received MultiStem treatment within 36 hours of the stroke did substantially better than placebo patients and later treatment MultiStem subjects. As a result, we will continue to focus our ongoing clinical development on treatment within 36 hours of the stroke."

Data highlights from the 365-day follow-up data analysis include:

- MultiStem treatment continued to be well tolerated through 365 days;
- Among all subjects who received MultiStem treatment (n=65), 23.1% of patients achieved an Excellent Outcome at 365 days, compared to 8.2% of patients who received placebo (n=61), and the 14.9% difference was statistically significant (p=0.02) and compared favorably to the 8.8% difference at 90 days;
- Among patients who received MultiStem treatment within 36 hours following the stroke, 29.0% achieved Excellent Outcomes (n=31), and compared to all placebo subjects (n=61), the 20.8% difference was significant ($p < 0.01$) and also greater than the 9.5% difference at 90 days;

Proportion of Subjects with Excellent Outcome at Day 90 and Over One Year

Subjects	Day 90	Day 365
All MultiStem (n=65)	15.4%	23.1%
All Placebo (n=61)	6.6%	8.2%
Difference with all placebo	8.8%	14.9%*
Early Treatment with MultiStem (n=31)	16.1%	29.0%
Difference with all placebo	9.5%	20.8%**

*p = 0.02, **p < 0.01

- Substantial improvements were also observed in the Barthel Index, which is the clinical scale used to assess the ability of patients to live independently. Among all subjects (65 MultiStem, 61 placebo), 61.5% of MultiStem patients had an excellent outcome in the Barthel Index (≥95), compared to 44.3% of placebo patients (p=0.05); furthermore,

67.7% of the subset of MultiStem patients who had treatment within 36 hours (n=31) achieved an excellent Barthel outcome, representing a 23.4% difference with the incidence for all placebo patients (p=0.03); and

- Among MultiStem patients who did not achieve an Excellent Outcome at 365 days, there appears to be meaningful benefit from the treatment relative to standard of care, with reductions in average initial hospitalization days, mortality, life threatening adverse events and infections. For example, comparing all such MultiStem and placebo subjects, MultiStem-treated patients had 1.6 fewer average hospitalization days, and an 11% lower proportion of patients with death or life threatening adverse events. In addition, when comparing subjects receiving early treatment with MultiStem against all placebo subjects, MultiStem patients had an average of 2.9 fewer hospitalization days, and an 11.4% lower incidence of death or life threatening adverse events. Further, such MultiStem patients appear to have better functional improvement than these placebo patients over one year, as evidenced by a higher proportion of excellent Barthel Index outcomes (≥95), 50% for MultiStem subjects (and 55% for early treatment MultiStem), compared to 39% for placebo subjects.

"Achievement of an Excellent Outcome is important because it means that a patient has substantially improved in each of the three clinical rating scales used to assess patient improvement and has regained the ability to live and function independently with a high quality of life," continued Van Bokkelen. "Furthermore, when evaluating patients that either received no reperfusion therapy, treatment with tPA alone, or mechanical reperfusion alone, we observed a greater than five-fold increase in the proportion of patients that achieved an Excellent Outcome at one year when comparing subjects that received MultiStem treatment within 36 hours versus placebo."

Phase 2 Clinical Study Design

The randomized, double-blind, placebo-controlled Phase 2 clinical trial was conducted at sites in the United States and the United Kingdom. The study was conducted in two parts - a small dose selection phase involving 16 patients in two cohorts, followed by larger efficacy phase of 118 patients. The evaluable patient population included 8 patients from cohort 2 and the cohort 3 patients, which all received a high dose of treatment or placebo.

The study enrolled subjects who received intravenously either MultiStem treatment or placebo one to two days following the stroke. Functional and neurological deficit and recovery following the ischemic stroke were evaluated using three standard methods: the modified Rankin Score (mRS), a scale from 0-6 directed to assessing disability; the NIH Stroke Scale (NIHSS), a scale from 0-42 for evaluating neurological deficit; and the Barthel Index, assessing performance related to activities of daily living on a 100 point scale. See www.strokecenter.org/professionals/stroke-diagnosis/stroke-assessment-scales/ for additional information on these assessment scales. Additionally, other clinical, safety and biomarker data was collected over the assessment period. Of the patients evaluated in the study, 65 patients were in the MultiStem treatment group and 61 patients were in the placebo group, and among the MultiStem subjects, 31 received MultiStem treatment within 36 hours following the stroke.

About the Disease Condition

Ischemic stroke is caused by a blockage of blood flow to the brain. A leading cause of death and disability globally, each year more than 15 million people are estimated to suffer a stroke, including more than two million people in the United States, Japan and European Union, combined. According to the American Heart Association, ischemic strokes comprise more than 85% of all strokes. Current standard of care for ischemic stroke involves the administration of a thrombolytic (clot dissolving) agent within three to four hours after a stroke has occurred, a narrow window that results in only a small percentage of patients receiving such treatment.

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.

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 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, 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; 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.