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Athersys Announces Results From Phase 2 Study of MultiStem(R) Cell Therapy for Treatment of Ischemic Stroke

CLEVELAND, April 17, 2015 (GLOBE NEWSWIRE) -- Athersys, Inc. (Nasdaq:ATHX) today announced interim results from its exploratory Phase 2 clinical study of the intravenous administration of MultiStem® cell therapy to treat patients who have suffered an ischemic stroke. The study results demonstrate favorable safety and tolerability for MultiStem, consistent with prior studies. With respect to the primary and secondary endpoints, the cell therapy did not show a difference at 90 days compared to placebo. However, MultiStem treatment was associated with lower rates of mortality and life threatening adverse events (AEs), infections and pulmonary events. Furthermore, post-hoc analysis shows that patients who received MultiStem treatment earlier in the treatment window had more robust recovery rates in comparison to placebo and relative to patients who received later MultiStem treatment.

Dr. David Hess, lead clinical investigator in the study, stroke specialist and Chairman of the Department of Neurology at the Medical College of Georgia at Georgia Regents University, will present the summary results at the European Stroke Organization conference on Sunday, April 19th at 8:50 AM in Glasgow, Scotland, United Kingdom.

Data highlights from the 90-day interim analysis include:

- MultiStem cell therapy demonstrated favorable tolerability and safety profile through the evaluation date, which was at least 90 days for all patients;
- Patients who received intravenous administration of MultiStem did not show a significant difference from placebo-treated patients for the primary endpoint (Global Stroke Recovery Assessment) and the related secondary endpoints - which were defined as the proportion of patients achieving a modified Rankin Scale (mRS) value of 0-2, improvement in the NIH Stroke Scale (NIHSS) by ≥ 75% and achieving Barthel Index (BI) ≥ 95 at day 90, however;
- Among all subjects who received MultiStem treatment, 15.4% of patients achieved an Excellent Outcome, defined clinically as attaining mRS 0-1, NIHSS 0-1 and BI ≥95, compared to 6.6% of patients that received placebo, (p=0.10);
- As described in the table below, patients who received MultiStem treatment earlier in the treatment window (24-36 hours post-stroke) exhibited more favorable recovery on the primary and key secondary endpoints than patients who received placebo or patients who received MultiStem treatment later (e.g. Excellent Outcome, p = 0.03), and this treatment effect was even more pronounced the earlier the MultiStem administration within the 24-36 hour timeframe;
- A higher proportion of patients who received treatment with MultiStem achieved a good clinical outcome by day 7 after treatment, defined as achieving mRS 0-2, which was 12.9% for patients receiving MultiStem, compared to 5.2% of patients who received placebo, and a similar pattern was seen for improvement in NIHSS ≥ 75%, which was achieved by 10.2% of MultiStem treated patients vs. 3.9% of patients who were treated with placebo;
- Mortality was lower among patients who received treatment with MultiStem in comparison with placebo. There were 9 subject deaths (14.8%) among those receiving treatment with placebo, and only 4 patient deaths (6.2%) among patients receiving treatment with MultiStem;
- The MultiStem treatment group had a lower rate of life threatening adverse events and death (p=0.04), and also exhibited lower rates of pulmonary events (p=0.08) and infections. The MultiStem treated group also had a significantly lower level of circulating CD-3^+ T-cells at two days following dosing ($p < 0.01$), suggesting a reduction in the inflammatory response post-stroke, consistent with the therapeutic hypothesis.

As noted above, post-hoc analyses show that earlier MultiStem administration appears to provide substantial benefit, as evident in the following table:

MultiStem Administered ≤36 Hours Compared to All Placebo*

Clinical parameter, at 90 days	MultiStem** n=27	Placebo n=52	Difference
Global Test Statistic	Odds Ratio =2.21, p=0.07		

Modified Rankin Scale ≤2	48.1%	32.7%	15.4%
NIHSS Improvement ≥75%	51.9%	30.8%	21.1%
Barthel Index ≥95	55.5%	38.5%	17.0%
Excellent Outcome	18.5%	1.9%	16.6%
(mRS ≤1, NIHSS ≤1, and BI ≥95%)			p=0.03

* Excludes confounding data from patients that received both tPA and mechanical reperfusion in addition to investigational product

** Results for patients getting MultiStem administration > 36 hours not materially different from placebo

"This exploratory Phase 2 trial represents our first clinical study in stroke and was designed to evaluate the safety and efficacy of a single dose of MultiStem 24-48 hours following the occurrence of the stroke. This treatment window extends well beyond the limits of current standard of care, treatment with tPA, which may only be administered within the first several hours after a stroke," commented Dr. Gil Van Bokkelen, Chairman & CEO at Athersys. "Going into this trial, based on extensive preclinical work we have conducted internally and with independent labs, we anticipated that administration of MultiStem could provide several mechanisms of benefit, including down regulation of key inflammatory pathways, up regulation of multiple reparative mechanisms, and could also extend the treatment window in a meaningful way."

"While the trial did not achieve the primary or component secondary endpoints, we believe the evidence indicating that patients who received MultiStem treatment early appeared to exhibit meaningfully better recovery is very important and promising," continued Dr. Van Bokkelen. "The results appear to confirm that our window of intervention with MultiStem therapy may extend well beyond the limits of current care. Additional key observations from the trial also appear consistent with key elements of our initial hypothesis. In particular, we are encouraged by the reduced mortality and lower incidence of infections, pulmonary events and life threatening adverse events among MultiStem-treated patients, as well as the limited biomarker data we have seen so far. We anticipate additional data and information from the study and will conduct further analyses to generate more insight about the potential for MultiStem treatment in this area."

"We also continue to advance other clinical programs in acute myocardial infarction (AMI) and acute respiratory distress syndrome (ARDS). In AMI, we have previously published promising data from our Phase 1 study, testing localized delivery of MultiStem shortly following a heart attack, and look forward to completing this Phase 2 study as soon as possible. With respect to ARDS, we are encouraged by the apparent impact in this study on mortality, life threatening adverse events, infection and pulmonary events, which together with our non-clinical study results in pulmonary injury models, suggests the potential for benefit in this patient population. We have a good balance sheet to support this ongoing work and remain confident that our MultiStem therapy and other technologies will have an important impact," concluded Dr. Van Bokkelen.

Phase 2 Clinical Study Design

The randomized, double-blind, placebo-controlled Phase 2 clinical trial is being 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 either MultiStem treatment or placebo one to two days following the stroke. The primary endpoints for the study include safety over the first seven days following treatment and global stroke recovery at day 90, which assesses disability (modified Rankin Score ≤ 2), neurological deficit (NIH stroke scale, delta ≥75%) and activities of daily living (Barthel Index ≥ 95%). Additionally, there are secondary and exploratory endpoints evaluating elements of recovery and dysfunction, including biomarkers associated with subject condition and recovery, and safety variables over the study period.

Of the patients evaluated in the study, 65 patients were in the MultiStem treatment group and 61 patients were in the placebo group. Among the enrolled patients, the groups were generally evenly balanced in terms of baseline stroke characteristics.

Characteristic	MultiStem	Placebo
Age, mean (at time of admission)	61.6	62.5
% of male Subjects	52.2%	54.8%
% of female Subjects	47.8%	45.2%
NIHSS at baseline, mean	13.3	13.4
% of patients that received tPA	43.3%	48.4%

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.

The Athersys, Inc. logo is available at: http://www.globenewswire.com/newsroom/prs/?pkgid=4548

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. 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 Chugai, 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 ability to raise additional capital; final 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 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 and advance our programs; 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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