Athens Announces Positive Results From Its Exploratory Clinical Study of MultiStem® Cell Therapy for Treatment of Acute Respiratory Distress Syndrome (ARDS)

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Results Confirm Safety Profile and Demonstrate Meaningful Potential Benefits in Mortality, Ventilator-free Days and ICU-free Days

CLEVELAND, Jan. 23, 2019 (GLOBE NEWSWIRE) -- Athersys, Inc. (NASDAQ: ATHX) announced today summary results from its exploratory clinical study of the intravenous administration of MultiStem® cell therapy to treat patients who are suffering from acute respiratory distress syndrome (ARDS). The study results provide further confirmation of tolerability and a favorable safety profile associated with MultiStem treatment. Importantly, MultiStem treatment was associated with lower mortality and a greater number of ventilator-free and intensive care unit (ICU) free days in the first month following diagnosis relative to patients receiving placebo. Furthermore, analysis of initial biomarker data reflects lower levels of inflammatory markers/cytokines following MultiStem treatment, an expected mechanism of action in this patient population.

Patients in the exploratory study were evaluated through 28 days for the primary clinical assessment and will be further assessed through a one-year follow-up period. Data highlights from the initial evaluation include the following results from the double-blind, randomized, placebo-controlled portion of the study:

- Lower mortality of 25% in the MultiStem treatment group vs. 40% in the placebo group;
- 40.2% higher ventilator-free (VF) days, (12.9 VF days in the MultiStem treatment group vs. 9.2 VF days for the placebo group);
- 27.2% higher ICU-free days, (10.3 days in MultiStem subjects vs. 8.1 days for subjects receiving placebo);
- In more severe ARDS patients (as evident in a prospectively defined analysis), the difference between MultiStem treatment and placebo was greater – 25% mortality in MultiStem group vs. 50% in placebo group, 14.6 VF days in MultiStem group vs. 8.0 VF days in placebo group, and 11.4 ICU-free days in MultiStem group versus 5.9 ICU-free days in placebo group; and
- MultiStem treatment was well tolerated in this very sick ARDS patient population, with no serious adverse events related to administration.

The study was designed to evaluate the impact of MultiStem treatment in subjects with acute onset of moderate to severe ARDS and was conducted at sites in the United States and United Kingdom. The study included two parts – a small initial dose confirmation phase, followed by the larger double-blinded, placebo-controlled and randomized phase (Phase 2a portion). Treatment was required to begin within four days of ARDS diagnosis with an average treatment time of approximately two days from the diagnosis. Six subjects were treated with MultiStem in the initial portion of the study, and in the Phase 2a portion of the study, 20 subjects were treated with an intravenous (IV) administration of 900 million MultiStem cells and 10 subjects received IV placebo. As disclosed previously, the study was not powered for the efficacy outcomes.

Athersys will continue to evaluate the data as the one-year follow-up period is completed for all patients in the trial. Athersys and the study investigators plan to present more detailed and comprehensive results at a medical science conference after additional analyses.

“We believe this exploratory study met all of its key objectives and view it as an important indication of the potential relevance of MultiStem in another critical care area where there is substantial unmet medical need,” commented Dr. Gil Van Bokkelen, Chairman & CEO at Athersys. “The consistent pattern of results suggesting a beneficial effect on mortality and key clinical parameters provides us with confidence that this is another condition where administration of MultiStem can have a meaningful therapeutic impact.

“We also believe these results provide strong support for the ARDS trial that our partner, HEALIOS K.K. (Healios), is authorized to conduct in Japan under the recently implemented regenerative medicine regulatory framework designed to expedite development. Healios is actively working towards initiation of this study, and we continue to support their efforts, as well as advance our other key programs,” concluded Dr. Van Bokkelen.

In addition to this ARDS study, Athersys is conducting ongoing studies in ischemic stroke (MASTERS-2 Phase 3 study) and acute myocardial infarction, and is planning for a study to treat severe trauma patients. Athersys is also supporting studies in Japan being conducted by Healios, targeting ischemic stroke (TREASURE study) and ARDS. Athersys continues to make progress in building its capabilities and capacity for completing development, gaining approval and supporting potential commercial activities, and ended the year with approximately $51 million in cash and cash equivalents to support these activities.

About ARDS

Acute Respiratory Distress Syndrome (ARDS) is a serious immunological and inflammatory condition characterized by widespread inflammation in the lungs. ARDS can be triggered by pneumonia, sepsis, trauma or other events, and represents a major cause of morbidity and mortality in the critical care setting. It has significant implications, as it prolongs intensive care unit (ICU) and hospital stays and requires convalescence in the hospital and
rehabilitation. There are limited interventions and no effective drug treatments for ARDS, making it an area of high unmet clinical need with high treatment costs. Given ARDS high treatment costs, a successful cell therapy could be expected to generate significant savings for the healthcare system by reducing days on a ventilator and in the ICU and importantly, could reduce mortality and improve quality of life for those suffering from the condition. The medical need for a safe and effective treatment of ARDS is significant due to its high mortality rate, and it affects annually approximately 400,000 - 500,000 patients in Europe, the United States and Japan.

MultiStem cell therapy has demonstrated the capacity to reduce inflammation, support tissue regeneration and promote homeostasis in acute immunological and injury settings. Preclinical data suggests that MultiStem cells may have a protective effect by shifting the physiological response from pro-inflammatory to anti-inflammatory, and through the promotion of key reparative mechanisms. In animal models, MultiStem cells have demonstrated an ability to reduce the severity of pulmonary distress, reduce alveolar edema and return lung endothelial permeability to normal. Intravenous MultiStem treatment early following the onset of the condition may ameliorate the initial hyper-inflammation and reduce the fibrotic activity that follows, thereby speeding the return to and improving the likelihood of more normal lung function and helping patient recovery.

About MultiStem®

MultiStem® cell therapy is a patented regenerative medicine product in clinical development 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 therapy 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 clinical studies, MultiStem therapy could provide a meaningful benefit to patients, including those suffering from serious diseases and conditions with unmet medical need.

About Athersys

Athersys is an international biotechnology company engaged in the development of therapeutic products 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 focused on disease indications in the neurological, cardiovascular, inflammatory and immune, certain pulmonary and other areas where the current standard of care is limited or inadequate for many patients, and has several ongoing clinical trials evaluating this potential regenerative medicine product. Athersys has forged strategic partnerships and a broad network of collaborations to further advance MultiStem cell therapy toward commercialization. More information is available at www.athersys.com. Follow Athersys on Twitter at www.twitter.com/athersys.

This clinical trial included subjects from the United Kingdom and the United States. The United Kingdom arm of the trial was supported by £2M funding from Innovate UK. Cell and Gene Therapy Catapult, working alongside Athersys, provided the United Kingdom clinical operations support for the successful enrollment of the trial. This research was also supported by the National Institute On Alcohol Abuse And Alcoholism, as part of the U.S. National Institutes of Health under Award Number R42AA024003. The information provided here is solely the responsibility of the authors and does not necessarily represent the official views of the National Institutes of Health.

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,” “suggests,” “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 therapeutics, including 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: our ability to raise capital to fund our operations; the timing and nature of results from our MultiStem clinical trials, including the MASTERS-2 Phase 3 clinical trial and the Healios’ TREASURE 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 of our product candidates; the possibility of delays, work stoppages or interruptions in manufacturing by third parties to us, such as due to material supply constraints, contaminations, or regulatory issues, which could negatively impact our trials and the trials of our collaborators; uncertainty regarding market acceptance of our product candidates and our ability to generate revenues, including MultiStem cell therapy for the treatment of stroke, acute respiratory distress syndrome, acute myocardial infarction and trauma, and the prevention of graft-versus-host disease and other disease indications; changes in external market factors; changes in our industry’s overall performance; changes in our business strategy; our ability to protect and defend our intellectual property and related business operations, including the successful prosecution of our patent applications and enforcement of our patent rights, and operate our business in an environment of rapid technology and intellectual property development; our possible inability to realize commercially valuable discoveries in our collaborations with pharmaceutical and other biotechnology companies; our ability to work with Healios to reach an agreement for an option in China; our ability to meet milestones and earn royalties under our collaboration agreements, including the success of our collaboration with Healios; our collaborators’ ability to continue to fulfill their obligations under the terms of our collaboration agreements and generate sales related to our technologies; the success of our efforts to enter into new strategic partnerships and advance our programs, including without limitation, in North America, Europe and Japan; 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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