



NEWS RELEASE

Athersys Presents Data From Its Acute Respiratory Distress Syndrome Clinical Trial at American Thoracic Society International Conference

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The MultiStem® treatment group had more ventilator-free and ICU-free days, and greater reduction in acute inflammatory biomarkers compared to the placebo

CLEVELAND, May 20, 2019 (GLOBE NEWSWIRE) -- Athersys, Inc. (Nasdaq: ATHX) announced additional favorable data from its exploratory Phase 1/2 acute respiratory distress syndrome (ARDS) clinical trial. Dr. Geoff Bellingan, Medical Director at University College London Hospitals, described the data from the clinical trial, referred to as the MUST-ARDS trial, during his presentation today at the American Thoracic Society International Conference in Dallas, Texas, the longest running, large-scale conference in the world offering groundbreaking research in pulmonary, critical care and sleep medicine. His talk was titled, "Primary Analysis of a Phase 1/2 Study to Assess MultiStem® Cell Therapy, a Regenerative Advanced Therapy Medicinal Product (ATMP), in Acute Respiratory Distress Syndrome (MUST-ARDS)".

As previously reported, subjects 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. The MultiStem treatment group achieved the primary endpoint of safety with the MultiStem treatment being well-tolerated and with no serious adverse events related to administration.

Day 28 Results

	MultiStem	Placebo
Intent to Treat Population	n=20	n=10
Day-28 Mortality	5 (25%)	4 (40%)
Ventilator-free (VF) days	12.9 (10.7)	9.2 (9.6)
	18.5 [0, 22]	6.5 [0, 18.3]
Intensive care unit (ICU)-free days	10.3 (8.9)	8.1 (8.9)
	12.5 [0, 18.5]	4.5 [0, 16.8]

Data are: n (%), mean (SD) or median [IQR]

“ARDS is more common than people realize, and there is no cure for it because it is a heterogenous condition,” commented Dr. Bellingan. “The data from the MUST-ARDS trial is very encouraging, and I’m looking forward about the potential of having a therapy to offer ARDS patients.”

In a prospectively-defined analysis examining the effects on subjects with poorer lung function as determined by a ratio of partial pressure arterial oxygen and fraction of inspired oxygen (PaO₂/FIO₂) of <150, the difference between MultiStem treatment and placebo was greater than that observed in the intent to treat population. There was 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. The median values of the data set were 18.5 VF days for the MultiStem treated patients compared to 3.5 VF days for the placebo group and 12.5 ICU-free days for MultiStem patients compared to 1 ICU-free day for the placebo group.

Dr. Bellingan discussed potential mechanisms by which MultiStem may be providing benefit to ARDS patients, such as through restored endothelial integrity of the lung, reduced lung edema, increased alveolar fluid clearance, reduced immune cell infiltrate (including neutrophils, macrophages and eosinophils), and the ability to shift immune cells from a pro-inflammatory to anti-inflammatory phenotype.

A preliminary analysis of the biomarkers reveals that, similar to results from the Company’s MultiStem MASTERS-1 study for acute ischemic stroke, there was an overall reduction in pro-inflammatory cytokines in the MultiStem treatment group compared to placebo group. Specifically, certain acute inflammatory cytokines were lower in the ARDS patients that received MultiStem. The same inflammatory cytokines were also downregulated in the stroke patients that were in the MultiStem treatment group in the MASTERS-1 study, suggesting that MultiStem may work in similar way for both ARDS and stroke.

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. Treatment was required to begin within four days of ARDS diagnosis with an average treatment time of approximately two days from the diagnosis. Initially, three subjects received 300 million MultiStem cells and, after a safety review, an additional three subjects received 900 million MultiStem cells. This was followed by the larger double-blinded, placebo-controlled and randomized study of twenty subjects treated with an intravenous (IV) administration of 900 million MultiStem cells and ten subjects receiving IV placebo.

Additionally, last week, Athersys announced that its clinical program evaluating MultiStem cell therapy for the treatment of ARDS received Fast Track designation from the United States Food and Drug Administration. This important designation is given to qualified investigational therapies that show promise in providing benefit to patients in areas of significant unmet medical need. Fast Track designation allows for an expedited regulatory review process after the clinical data is submitted to help speed development of promising therapies to the market in order to help patients in areas where current standard of care is limited.

In April 2019, the Company's collaborative partner in Japan, HEALIOS K.K. (Healios), announced the enrollment of the first patient into its MultiStem ARDS trial, referred to as the ONE-BRIDGE study. The study is intended to investigate the efficacy and safety of MultiStem therapy for patients with pneumonia-induced ARDS in Japan, and its primary endpoint will be the number of VF days in the first 28 days following treatment.

The Company will consider Healios' progress in its ONE-BRIDGE study as it further develops its plans to move the ARDS program through clinical development.

About ARDS

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 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 approximately 400,000 - 500,000 patients in Europe, the United States and Japan annually.

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 inflammation, reduce fluid retention in the lungs and return lung function to normal. Intravenous MultiStem treatment early following the onset of ARDS may ameliorate the initial 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 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 neurological, cardiovascular, and inflammatory and immune disease areas, 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 the MultiStem cell therapy toward commercialization. More information is available at www.athersys.com.

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 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 Healios’ TREASURE and ONE-BRIDGE clinical trials 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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