FDA Authorizes Athersys to Initiate a Pivotal Clinical Trial Evaluating MultiStem® Cell Therapy in Patients With COVID-19 Induced Acute Respiratory Distress Syndrome

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In a Phase 1/2 study of MultiStem therapy for the treatment of ARDS, favorable tolerability data and meaningful potential benefits in mortality, ventilator-free days and ICU-free days observed

Company plans to initiate a Phase 2/3 pivotal study in patients with COVID-19 induced ARDS this quarter

Advancement of key Athersys program with FDA Fast Track designation for the treatment of ARDS

CLEVELAND--(BUSINESS WIRE)-- Athersys, Inc. (NASDAQ: ATHX) announced today that the U.S. Food and Drug Administration (FDA) has authorized the Company to initiate a Phase 2/3 pivotal study to assess the safety and efficacy of MultiStem® therapy in subjects with moderate to severe acute respiratory distress syndrome (ARDS) induced by the novel coronavirus disease (COVID-19). This program falls under the current Investigational New Drug (IND) application for the Company's completed MUST-ARDS study and, therefore, a new IND does not need to be filed. The Company plans to open the first clinical sites for recruitment of this MACOVIA (MultiStem Administration for COVID-19 Induced ARDS) study this quarter.
According to the World Health Organization (WHO) and other recent clinical and epidemiological data, ARDS is the leading cause of death among COVID-19 infected patients. There are no FDA-approved medicines for the treatment of ARDS. Athersys recently completed a Phase 1/2 study evaluating administration of MultiStem to patients with ARDS and based on the promising data from the study, the program was recently granted Fast Track designation by the FDA.

This trial will be a multicenter study featuring an open-label lead-in followed by a double-blinded, randomized, placebo-controlled Phase 2/3 portion. The primary objectives of the MACOVIA study are to evaluate the safety and efficacy of MultiStem therapy as a treatment for subjects with moderate to severe ARDS due to COVID-19. The primary efficacy endpoint will be number of ventilator-free days through day 28 as compared to placebo, a well-established endpoint for ARDS trials that evaluates an intervention’s combined impact on survival and liberation from invasive mechanical ventilation. The secondary objectives of this study are to evaluate pulmonary function, all-cause mortality, tolerability and quality of life (QoL) among survivors associated with MultiStem therapy as a treatment for subjects with moderate to severe ARDS due to COVID-19. The study is designed to enroll approximately 400 subjects and will be conducted at leading pulmonary critical care centers throughout the United States. The first cohort of the study will be open-label, with a single active treatment arm to evaluate the safety of the MultiStem product candidate at two dose levels. The second cohort will be a double-blind, randomized, placebo-controlled run-in phase to evaluate the efficacy of MultiStem. The design of the third planned cohort will be based on analysis of the results of the second cohort. The intent-to-treat population will include all randomized subjects (i.e., subjects from the second and third cohorts).

“We are grateful for the FDA’s timely review and feedback during our design of this pivotal Phase 2/3 study,” commented Dr. Eric Jenkins, MD, Senior Medical Director and Head of Clinical Operations at Athersys. “With encouraging non-clinical and clinical data, affirmed by the FDA’s Fast Track designation for ARDS, Athersys and its collaborating clinical investigators are highly motivated by the FDA’s authorization that we may proceed with enrollment of the first open-label cohort to evaluate safety. We are in communication with FDA regarding the enrollment of further cohorts to conduct a scientifically rigorous evaluation of MultiStem’s safety and efficacy in the treatment of ARDS due to COVID-19. We believe that MultiStem treatment, by modulating patients’ hyperinflammatory response to highly pathogenic respiratory viruses, including SARS-CoV-2 which causes COVID-19, represents a very promising approach to improving outcomes in patients who suffer the most severe manifestations of these illnesses.”

With the spread of COVID-19 and the resulting ARDS cases, there is an immediate need for therapies for the treatment of ARDS. The data from the Company’s Phase 1/2 MUST-ARDS study met its primary endpoint of tolerability and participants were evaluated through 28 days on key clinical parameters as well as improvement in pulmonary function. As previously reported, study subjects receiving MultiStem experienced less mortality, more...
ventilator-free days, and more intensive care unit (ICU)-free days during the 28-day clinical evaluation period than the subjects who received placebo. These differences were observed to be greatest among the prospectively defined patients with more severe ARDS, defined as patients with PaO2/FiO2 above or below 150mmHg. Importantly, among all randomized subjects in the study, 45% of MultiStem treated subjects were off the ventilator within seven days or less, compared to only 20% of placebo recipients. Biomarker analysis confirmed a reduction in inflammatory biomarkers among MultiStem treated subjects. In addition, results from the one-year follow up were consistent with the positive day-28 clinical results and an evaluation of the subjects’ QoL, as measured by patient-reported EQ-5D-3L self-care responses and visual analogue scale (VAS) pain scores, more positive among patients who received MultiStem. The data from this study were presented at the American Thoracic Society Meeting in May 2019.

MultiStem therapy’s potential for multidimensional therapeutic impact may distinguish it from traditional biopharmaceutical therapies focused on a single mechanism of benefit. Since MultiStem is not virus- or pathogen-specific, we believe it has the potential to treat ARDS that develops from a variety of causes, including COVID-19, as well as other pathogen-induced or non-infectious causes of severe lung inflammation leading to ARDS. The Company is in discussions with the Biomedical Advanced Research and Development Authority (BARDA) to expedite the advancement of MultiStem to treat patients with ARDS resulting from the COVID-19 epidemic and other potential pandemic outbreaks. For more detailed information on the Company’s ARDS program, please visit the ARDS page on the Athersys website.

About ARDS

ARDS is a serious respiratory 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. ARDS is associated with a high mortality rate and significant long-term complications and disability among survivors. Among survivors, the condition prolongs ICU and hospital stays and often requires extended convalescence in the hospital and rehabilitation care settings. There are limited interventions and no effective drug treatments for ARDS. There is a large unmet need for a safe treatment that can reduced mortality and improve QoL for those surviving ARDS. Additionally, given the high healthcare resource burden associated with treatment of ARDS patients, a successful therapy could be expected to generate significant savings for the healthcare system by reducing days on a ventilator and in the ICU, or in the setting of a widespread high pathogenicity respiratory virus pandemic, make those resources more rapidly available to other patients.

About COVID-19

COVID-19 is the infectious disease caused by the most recently discovered human coronavirus, SARS-CoV-2. This
new disease was unknown before the outbreak was first discovered in Wuhan, China, in December 2019. Older people and those with underlying medical problems such as high blood pressure, heart problems or diabetes, are more likely to develop serious illness, but even young, previously healthy people can suffer severe disease and complications such as ARDS. Data are still emerging but published case series available today suggest mortality rates among COVID-19 patients who develop ARDS may be as high as 50% to 70%.

About MultiStem ®

MultiStem® cell therapy is a patented regenerative medicine product candidate 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 in response to signals of inflammation and tissue damage. MultiStem therapy's potential for multidimensional therapeutic impact may distinguish it from traditional biopharmaceutical therapies focused on a single mechanism of benefit. MultiStem represents a unique "off-the-shelf" stem cell product candidate 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 favorable efficacy data, its novel mechanisms of action, and favorable and consistent tolerability data in clinical studies, we believe that 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 a 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, inflammatory and immune, cardiovascular and other critical care indications 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. 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 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: the success of our MACOVIA study; our ability to raise capital to fund our operations; our ability to successfully finalize and implement an alliance with BARDA, and the terms of any such alliance, including the amount, if any, of funding that we might receive; the timing and nature of results from MultiStem clinical trials, including our MASTERS-2 Phase 3 clinical trial and the HEALIOS K.K. (Healios) TREASURE and ONE-BRIDGE clinical trials in Japan; the impact on our business, results of operations and financial condition from the ongoing global COVID-19 pandemic, or any other pandemic, epidemic or outbreak of infectious disease in the United States; 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 or us, such as due to material supply constraints 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 ischemic stroke, ARDS, 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 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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