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Athersys Reports Alignment of Key Regulators for Pivotal Registration Study in Stroke

MultiStem® stem cell technology receives Positive Opinion from European Medicines Agency

Adds to Special Protocol Assessment and Fast Track designation in U.S., and Priority Review under Sakigake in Japan, creating an expedited path forward to commercialization

CLEVELAND, Aug. 07, 2017 (GLOBE NEWSWIRE) -- Athersys, Inc. (Nasdaq:ATHX) today announced that the design of its Phase 3 clinical study treating ischemic stroke with its proprietary MultiStem® cell therapy product (the "MASTERS-2" study) has received a Final Scientific Advice positive opinion from the European Medicines Agency ("EMA"). This represents EMA's opinion that, upon success, the study should be sufficient to warrant approval for commercialization, which is in line with the U.S. Special Protocol Assessment and Fast Track Designation. In Japan, Athersys' partner, HEALIOS K.K. ("Healios") is conducting the TREASURE study, evaluating MultiStem therapy for ischemic stroke, and has received a priority review designation under Sakigake from Japan's Pharmaceutical and Medical Devices Agency. The results from the TREASURE study, if needed, are acceptable for supporting the applications for registration, along with the results from the MASTERS-2 study, in both the U.S. and Europe.

This means that, in the three major pharmaceutical markets, Athersys and its existing and future partners have clarity with respect to the development path to commercialization for MultiStem therapy for moderate to severe ischemic stroke, facilitating planning and operations and removing regulatory uncertainty from the development program. Additionally, the stroke program is positioned for priority review and accelerated approval, shortening the time from successful study results to commercialization. As a result, with successful study outcome and registration, Athersys' MultiStem cell therapy could represent the first major advancement in decades for the treatment of acute stroke, outside of limited current treatment options.

"We are excited about receiving the positive opinion on our pivotal, registrational stroke study, MASTERS-2, and we thank the EMA for its continued support of our efforts to develop an important new treatment option for patients in this area of significant unmet medical need," commented Dr. Gil Van Bokkelen, Chairman & CEO at Athersys. "Recent publications and presentations reinforce years of promising work and continue to illustrate how we might be able to improve outcomes and substantially extend the treatment window to 36 hours for stroke patients with MultiStem, beyond the narrow treatment window for tPA and mechanical reperfusion that exist today. The publications also provide further evidence of the important biological mechanisms that MultiStem cell therapy may convey to help patients recover from a debilitating stroke event. Stroke remains a leading cause of serious disability and represents a substantial burden on patients, families and healthcare systems around the world, and we are committed to transforming medical care in this important area."

About EMA Scientific Advice

Scientific Advice is a procedure offered by EMA to stakeholders for clarification of questions arising during development of medicinal products. The scope of Scientific Advice spans scientific issues that may be related to quality, non-clinical, and/or clinical aspects of the concerned medicinal product under development. The advice is designed to facilitate the development and availability of high-quality, effective and acceptably safe medicines, for the benefit of patients and helps to ensure that developers perform the appropriate tests and studies, so that no major objections regarding the design of the tests are likely to be raised during evaluation of the marketing-authorization application. Such major objections can significantly delay the marketing of a product, and, in certain cases, may result in refusal of the marketing authorization. Following the Agency's advice increases the probability of a positive outcome. For more information on the Scientific Advice process, please visit:
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000049.jsp

About Special Protocol Assessments

The Special Protocol Assessment process is a procedure by which the FDA provides official evaluation and written guidance

on the design and planned analysis of proposed study that are intended to form the basis of efficacy claims for a new drug application or biologic license application. Final marketing approval depends on the results of the efficacy, the adverse event profile and an evaluation of the benefit/risk of treatment demonstrated in the Phase 3 clinical program. The S.P.A. agreement may only be modified through a written agreement between the sponsor and the FDA, or if the FDA becomes aware of a substantial scientific issue essential to product efficacy or safety. For more information on the S.P.A. process, please visit:
http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm080571.pdf

About Fast Track Designation

Fast Track is a process designed by the FDA to facilitate the development and expedite the review of drugs to treat serious conditions and fill unmet medical needs. The purpose is to get important new drugs to the patient earlier. Fast Track addresses a broad range of serious conditions. For more information on the Fast Track process, please visit:
https://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm

About the SAKIGAKE Designation

The Strategy of the Pharmaceutical and Medical Devices Agency's SAKIGAKE designation consists of the following two measurements and its coverage ranges from basic research to clinical research/trials, approval reviews, safety measures, insurance coverage, improvement of infrastructure and the environment for corporate activities, and global expansion.

- SAKIGAKE Designation System: promoting research and development in Japan aiming at early practical application for innovative pharmaceutical products, medical devices and regenerative medicines.
- Scheme for Rapid Authorization of Unapproved Drugs: accelerating the practical application of unapproved/off-label use of drugs for serious and life-threatening diseases by expanding the scope of the Council on Unapproved Drugs/Off-label Use to include unapproved drugs in the western countries if certain conditions are satisfied, and by improving the environment for companies to undertake development of such drugs.

For more information on the SAKIGAKE process, please visit:
http://www.pmda.go.jp/english/review-services/reviews/advanced-efforts/0001.html

About Ischemic Stroke

Stroke represents an area where the clinical need is particularly significant, since it is a leading cause of death and serious disability worldwide, with a substantially impaired quality of life for many stroke victims. Currently, there are nearly 17 million people that suffer a stroke globally and, on average, someone in the United States has a stroke every 40 seconds. Ischemic strokes, which represent the most common form of stroke, are caused by a blockage of blood flow in the brain that cuts off the supply of oxygen and nutrients and can result in long-term or permanent disability due to neurological damage. Unfortunately, current therapeutic options for ischemic stroke victims are limited, since the only available therapies, administration of the clot dissolving agent tPA, or "thrombolytic," or surgical intervention using mechanical reperfusion to remove the clot, must be conducted within several hours of the occurrence of the stroke. As a consequence of this limited time window, only a small percentage of stroke victims are treated with the currently available therapy—most simply receive supportive or "palliative" care. The long-term costs of stroke are substantial, with many patients requiring extended hospitalization, extended physical therapy or rehabilitation (for those patients that are capable of entering such programs), and many require long-term institutional or family care.

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 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 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.

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 regulatory approvals and 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 and others, 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 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 or collaborations and advance our programs; our ability to raise additional capital; results from our MultiStem ongoing and planned 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 within the expected time frame or at all; 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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