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Athersys Announces Results From Phase 2 Study of MultiStem(R) Cell Therapy for Ulcerative Colitis

MultiStem Demonstrates Good Tolerability and Safety Profile But Fails to Show Efficacy Over 8 Weeks in Patients With Chronic, Advanced Ulcerative Colitis

CLEVELAND, April 28, 2014 (GLOBE NEWSWIRE) -- Athersys, Inc. (Nasdaq:ATHX) today announced interim results from the Phase 2 clinical study of the administration of Athersys' MultiStem® cell therapy to treatment refractory ulcerative colitis (UC) patients being conducted by Pfizer, Inc. The study results demonstrate favorable safety and tolerability for MultiStem through 8 weeks following treatment. However, the cell therapy failed to show meaningful benefit, following a single administration, in patients suffering from chronic, moderate-to-severe UC who have failed other therapies. These results reflect patient data 8 weeks following cell therapy or placebo administration and include the primary efficacy endpoints for the study. Additional 16-week results, including data about the impact from a second round of dosing for a subset of patients, longer term secondary clinical endpoints, and biomarker evaluation will be available after additional analysis has been completed.

Data highlights from the 8-week interim analysis include:

- MultiStem cell therapy demonstrated favorable tolerability and safety profile through 8 weeks following treatment;
- In this chronic, advanced UC patient population, a single IV administration of MultiStem did not show a statistically significant improvement compared to placebo in the primary efficacy endpoints - change in endoscopic score from baseline as measured by modified Baron score at 8 weeks and change in Mayo rectal bleeding subscore from baseline at 4 and 8 weeks; and
- At 4 weeks, the proportion of responders on MultiStem was significantly greater than placebo but the benefit was offset by declines in a minority of MultiStem-treated patients such that overall benefit at all time points measured was not significant. Multiple additional secondary endpoints at week 4 and week 8 were also explored and there were no significant differences observed between the two groups including clinical remission and clinical response.

"These results confirm the consistent safety profile Athersys has seen in previous clinical studies involving MultiStem," commented Dr. Gil Van Bokkelen, Chairman & CEO at Athersys. "This study was focused on a challenging patient population with chronic disease - ulcerative colitis patients who have become resistant, intolerant or unresponsive to other therapies. These interim results tell us a single administration of MultiStem in this patient group, while safe, was not sufficient to have a meaningful clinical effect. Obviously, we are disappointed by the efficacy results. We anticipate additional data from the study over time from patients who have received further treatment, which may provide more insight into the factors at work and the potential relevance for MultiStem in this area."

"In the meantime, we continue to advance our other clinical and preclinical programs. We have a strong balance sheet, and are pursuing multiple exciting opportunities where we remain confident MultiStem and our 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 by Pfizer under a collaboration and license agreement with Athersys at sites in the United States, Canada and Europe. The study was conducted in two parts - a small dose selection phase involving 18 patients, followed by larger efficacy phase of 88 enrolled patients powered for the primary endpoints. Eligible patients had moderate-to-severe active UC with a Mayo score of 6 to 12 points and endoscopic score of at least 2 (as measured by modified Baron score) despite prior treatment with corticosteroids, immunosuppressants, or anti-TNF agents.

Subjects enrolled in the efficacy stage of the study received either MultiStem treatment or placebo initially, followed by a second round of treatment with MultiStem therapy or placebo at eight weeks. The primary endpoints for the study include incidence and severity of adverse events over 16 weeks, change in endoscopic score (as measured by modified Baron score) at week eight, and changes in the Mayo rectal bleeding sub-score at weeks four and eight. Additionally, there are multiple secondary and

exploratory endpoints evaluating disease indicators and markers over 16 weeks and through the entire study period.

Of the 88 patients enrolled in the larger cohort, 48 patients were enrolled in to the MultiStem treatment group and 40 patients were enrolled in the placebo group. The mean disease duration for patients enrolled in the efficacy stage of the study was 10 years and the other baseline characteristics confirm a patient population with advanced ulcerative colitis.

Characteristic	Placebo	MultiStem
Male, %	75%	60%
Age, y, mean	41.2	41.0
Mayo score, mean	8.3	8.6
Prior anti-TNF therapy, %	60%	67%
Concommitant medication, %		
Steroids	48%	52%
Immunosuppressants	23%	23%

Enrollment was completed in North America and Europe, with 50 patients in the U.S. and Canada, and 38 patients in Germany, Sweden, Hungary, Slovakia and Italy.

About the Disease Condition

Ulcerative colitis (UC) is an inflammatory bowel disease (IBD). IBD is a group of chronic, relapsing inflammatory and autoimmune conditions. The most common IBD conditions include ulcerative colitis and Crohn's disease, which are estimated to affect more than four million people in the United States, European Union and Japan.

In UC, the sub-mucosa of the colon becomes progressively dominated by infiltration of lymphocytes causing further damage. UC patients most commonly present with diarrhea, urgency, rectal bleeding, and abdominal pain. Patients may also experience fatigue, fevers, weight loss, and dehydration, and the symptoms can be incapacitating. The disease is characterized by periods of increased disease activity, or flares, separated by periods of disease remission, and by progression in disease severity over time. The complications of UC may require surgery to remove the affected region of the colon, and may also require temporary or permanent colostomy.

First-line therapies for mild to moderately active UC are usually 5-Aminosalicylate acids (5-ASA) agents. Corticosteroids are often used as the second-line therapy, but these medications are associated with side effects and many patients develop steroid dependency. Alternative therapies include immunosuppressants, such as azathioprine (AZA) or 6-mercaptopurine (6-MP), or in severe cases, cyclosporine. For moderately to severely active UC, biologics such as anti-TNF therapies may be used to induce and maintain remission, but these therapies are not effective in many patients and may be associated with serious side effects.

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 multiple therapeutic factors produced in response to signals of inflammation and tissue damage. MultiStem has demonstrated therapeutic potential for the treatment of inflammatory and immune disorders, neurological conditions, and cardiovascular disease, as well as other areas. It 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. The product is extensively characterized for safety, consistency and potency. Athersys has a partnership with Pfizer Inc. which has rights to develop MultiStem for treating inflammatory bowel disease. Athersys is independently evaluating MultiStem therapy as a novel therapeutic approach for indications in the neurological, cardiovascular and transplant support areas, as well as other potential opportunities.

About Athersys

Athersys is a clinical stage 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 platform for disease indications in the cardiovascular, neurological, inflammatory and immune disease areas. The Company currently has several clinical stage programs involving MultiStem, including for treating inflammatory bowel disease, ischemic stroke, damage caused by myocardial infarction, and for the prevention of graft-versus-host disease. Athersys has also developed a diverse portfolio that includes other technologies and product development opportunities, and has forged strategic partnerships and collaborations with leading pharmaceutical

and biotechnology companies, as well as world-renowned research institutions in the United States and Europe 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

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, including MultiStem for the treatment of inflammatory bowel disease, acute myocardial infarction, stroke and other disease indications, including lysosomal storage disorders, and the prevention of 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: our ability to raise additional capital; the timing of and 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 ability to meet milestones under our collaboration agreements; 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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