



January 8, 2016

## **Healios and Athersys Enter Into Regenerative Medicine Partnership for Treatment of Stroke Using MultiStem(R) Therapy**

Â

### **Alliance to Focus on Development and Commercialization of Regenerative Medicine Products for Stroke and Other Potential Indications Using New Regulatory Framework in Japan**

CLEVELAND and TOKYO, Jan. 08, 2016 (GLOBE NEWSWIRE) -- Healios K.K. ("Healios") (Tokyo Stock Exchange:4593) and Athersys, Inc. ("Athersys") (NASDAQ:ATHX) have announced a partnership and license agreement that will focus on the development and commercialization of novel cell therapy treatments, including MultiStem® for the treatment of ischemic stroke and potentially other indications, in Japan. The partnership involves MultiStem, a proprietary, patented off-the-shelf stem cell therapy being developed by Athersys, with an initial focus on treating ischemic stroke.Â

Under the terms of the agreement, Healios will gain exclusive rights for the development of MultiStem for treating ischemic stroke in Japan. Healios will develop and commercialize the product in Japan, and Athersys will provide the manufactured product and support to Healios, while retaining all rights outside of Japan. In addition, Healios will obtain an exclusive option for development of two additional MultiStem clinical indications in Japan, including the treatment of Acute Respiratory Distress Syndrome ("ARDS"), which is currently in clinical development by Athersys in the United States ("U.S.") and the United Kingdom ("U.K."), and another indication in the orthopaedic area. Healios will also obtain an exclusive license to incorporate Athersys technology in the development and commercialization of its proprietary Healios "organ bud" technology, initially for transplantation to treat liver disease or dysfunction, which may be expanded upon exercise of the option.

"Stroke represents a major problem, both in Japan and globally. Currently available treatments such as tPA and mechanical thrombectomy must be administered within the first several hours after the stroke occurs, limiting treatment to a small percentage of patients, and such interventions may also pose certain risks," observed Dr. Kiyohiro Houkin, Chairman and Professor of Neurosurgery of Hokkaido University Medical School, and President of Hokkaido University Hospital. "The recently conducted international clinical study by Athersys in the U.S. and the U.K. suggests that intravenous administration of MultiStem within 36 hours of the occurrence of a stroke is safe, well-tolerated, and is a beneficial and effective treatment. The off-the-shelf administration of the product could be a simple and universal approach for treating acute stroke patients. Furthermore, it could be administered to patients that do not arrive at the hospital in time to receive current standard of care, or alternatively could be administered in addition to tPA or mechanical reperfusion, potentially enhancing outcomes for patients that have suffered significant strokes. If efficacy is confirmed in additional studies, it is possible that this therapy could become the new standard of care for treating stroke," concluded Dr. Houkin.

As part of the license, Athersys will receive an initial license fee of \$15 million, as well as have the opportunity to earn milestone and royalty payments upon the successful accomplishment of specific development and commercialization objectives, including the achievement of certain sales milestones.Â Development and approval milestones for stroke could total \$30 million, in addition to sales milestones that could reach \$185 million based on successful commercialization and the achievement of substantial sales of an approved product for treating stroke in Japan. Athersys will also receive tiered, double-digit royalties increasing into the high teens on product sales and will be responsible for providing manufactured product to Healios, subject to receiving reimbursement under a manufacturing supply arrangement. Furthermore, if Healios elects to expand the partnership following the successful completion of Athersys' ongoing clinical trial in ARDS, Athersys will receive a license expansion fee of \$10 million for the exclusive rights to two additional indications in Japan, with the corresponding potential for further milestones based on successful achievement of specific development and commercialization objectives. As part of the expanded alliance, Healios will also have the right to incorporate Athersys technology in other organ bud indications.Â Â Â

Japan currently represents the second largest national market for prescription biopharmaceuticals in the world. The country is also experiencing a significant demographic shift that is resulting in a rapid and unprecedented expansion of the elderly

population in Japan, which threatens to pose significant challenges for the national healthcare system over the next several decades. In order to help address the challenges posed by the potential increased demand for healthcare resources as a result of this expanding population, over the past two years policy-makers in Japan have revised the regulatory framework to focus on promoting the development of innovative new therapies that are demonstrated to be safe and that also provide promising signs of effectiveness. These recently implemented regulatory reforms, which are specifically designed to promote development of novel regenerative medicine therapies, are intended to speed the development of promising new medicines for patients where there is substantial unmet medical need.Â Â

Athersys' proprietary cell therapy product, MultiStem, has been evaluated in a Phase 2 clinical study for ischemic stroke in the U.S. and U.K. and is also in clinical development in several other indication areas, including the treatment of ARDS, myocardial infarction, and for transplant support, including prevention of Graft versus Host Disease and liver transplant support. Athersys has begun preparations for clinical development in Japan, including engagement with the Pharmaceuticals and Medical Devices Agency in Japan ("PMDA"). Healios and Athersys have already met jointly with PMDA and plan to complete additional preparations for initiation of a clinical study in Japan for stroke, with commencement of the study expected in the second half of 2016.Â

"I am very pleased that an exclusive licensing contract has been concluded with Athersys over development and distribution in Japan of MultiStem, a candidate pharmaceutical product for innovative cytotherapy having undergone phase 2 clinical trials in U.S. and U.K. There seems to be a large market need for MultiStem that can expand the period allowed before the start of treatment for ischemic stroke. Successful development of this product under the new regenerative medicine framework in Japan could accelerate Healios' ability to help patients, achieve profitable operations and deliver substantial value to our shareholders," commented Dr. Tadahisa "Hardy" Kagimoto , President of Healios. "Beyond stroke, the technology by Athersys is expected to improve the efficiency of production also when we prepare the organ as a platform for clinical transplantation programs. In essence, Healios has acquired a catalyst that can accelerate commercialization in the field of iPSC-based regenerative medicine products." Â Â Â Â Â

Healios is recognized as a leading regenerative medicine company in Japan, with a technology portfolio that includes the development of retinal pigmented epithelia cells produced from induced pluripotent stem cells for the treatment of age related macular degeneration ("AMD").Â Healios obtained a license to the technology from RIKEN to develop a novel treatment for AMD in 2013. Healios has also licensed additional technology from Yokohama City University related to the development of organ buds for transplantation indications. Healios is partnered with Sumitomo Dainippon Pharma Co., Ltd. and SHIBUYA CORPORATION (a leading robotics company in Japan), as well as NIKON CORPORATION and Osaka University who are focused on the joint development of advanced manufacturing capabilities for regenerative medicine therapies.

"Athersys is excited to be working with Healios for the development of MultiStem in Japan with an initial focus on treating patients that have suffered an ischemic stroke.Â Healios has an experienced and accomplished leadership team that recognizes the importance and transformational potential of the regenerative medicine field.Â They also have a compelling vision for our collaboration that includes the potential to work together across several important indication areas in a highly focused and efficient manner, which is why we selected them as our new partner," said Dr. Gil Van Bokkelen, Chairman and CEO at Athersys. "They have a strong balance sheet, an excellent network of institutional partners and collaborators, and they are committed to rapid and efficient development under the new regulatory framework in Japan, all of which are key factors for success."

## **About Ischemic Stroke**

Stroke represents an area where the clinical need is particularly significant, since it represents a leading cause of death and significantly lowers quality of life for many stroke victims. Currently, there are more than 15 million people that suffer a stroke globally and more than two million stroke victims each year in the United States, Europe and Japan, combined. 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 tissue loss and neurological damage, as well as long-term or permanent disability. 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.Â 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, Inc.**

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 cardiovascular, neurological, 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](http://www.athersys.com).

### **About Healios K.K.**

Healios is a biotechnology venture leading the field of developing iPS cell-based products for regenerative medicine. It was founded in 2011 and listed on the stock exchange (Tokyo Security Exchange Mothers:4593) in 2015. In Japan, the company is developing a product for treatment of age-related macular degeneration (an intractable ocular disease) jointly with Suimitomo Dainippon Pharma Co., Ltd., under the plan of obtaining approval of its manufacture/distribution in 2020. In fields other than ophthalmology, the company has started R&D of products for regenerative medicine capable of creating functional human organs (three-dimensional organs) jointly with Yokohama City University. The company may be viewed as an enterprise providing products for regenerative medicine as a solution to the significant global issue "aging of the society." See the website (<https://www.healios.co.jp/>) for details.

### **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 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 stroke, acute respiratory distress syndrome and other disease indications. 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, including our ability to reach milestones and receive milestone payments, and whether Healios elects to expand the partnership or whether any products are successfully developed and sold so that we earn royalty payments; our ability to raise additional capital; 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 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.*

Contact :

Athersys, Inc.

William (B.J.) Lehmann, J.D.

President and Chief Operating Officer

Tel: (216) 431-9900

Fax: (216) 432-2461

[bjlehmann@athersys.com](mailto:bjlehmann@athersys.com)

Healios K.K.

Corporate Communication Group

Tel: 81-(0) 3-5777-8308

Fax: 81-(0) 3-3434-7231

[pr@healios.jp](mailto:pr@healios.jp)

Â  
Â

---

*This announcement is distributed by NASDAQ OMX Corporate Solutions on behalf of NASDAQ OMX Corporate Solutions clients.*

*The issuer of this announcement warrants that they are solely responsible for the content, accuracy and originality of the information contained therein.*

*Source: Athersys, Inc. via Globenewswire*

*HUG#1977537*

News Provided by Acquire Media