Celsion Announces Enrollment Completion for Pivotal Phase III OPTIMA Study of ThermoDox® in Primary Liver Cancer

September 5, 2018

550 Patient Study Designed to Determine if ThermoDox® Can Significantly Prolong the Lives of Patients with Liver Cancer

OPTIMA Study's Hypothesis Independently Confirmed by the National Institutes of Health

LAWRENCEVILLE, NJ, Sept. 05, 2018 (GLOBE NEWSWIRE) -- Celsion Corporation (NASDAQ: CLSN), an oncology drug development company, today announced that it has reached its enrollment objective of 550 patients in the Company's pivotal, Phase III OPTIMA Study, a multinational, randomized, double-blind, placebo-controlled clinical trial of ThermoDox® in combination with radiofrequency ablation (RFA) for the treatment of patients with hepatocellular carcinoma (HCC), also known as primary liver cancer. The primary endpoint for the study is overall survival (OS). The OPTIMA Study's design and statistical plan incorporates two pre-planned interim efficacy analyses by the study's Data Monitoring Committee (DMC), and the first interim analysis is expected to occur in the first half of 2019. The DMC's interim analyses will evaluate safety, efficacy and futility to determine if there is significant evidence of clinical benefit.

"Completion of enrollment in the OPTIMA Study is yet another important milestone for Celsion and marks the conclusion of the execution phase in this multi-year study," said Michael H. Tardugno, Celsion's chairman, president and chief executive officer. "We believe ThermoDox® has enormous potential in combination with heat-based treatment modalities that are an increasingly important subject of research for controlling malignancy. With over 850,000 new cases of HCC each year worldwide, we believe the addressable market opportunity for ThermoDox® is conservatively over 200,000 patients with intermediate-stage HCC. On a global basis, the incidence of HCC is growing at 5% annually. For the United States, HCC is a particular problem. Recent reports from the Center for Disease Control and Prevention indicate that rates of new liver cancer cases rose 38% from 2003 to 2012, and the death rate from liver cancer has increased 56% since 2003. We believe that ThermoDox® has the potential to be a meaningful new treatment option in HCC, with the potential to change these grim trajectories. We look forward to the study's outcome, and to continuing our efforts -- in anticipation of potential success -- to advance our regulatory, commercial, clinical and manufacturing strategies."

An analysis of a large study evaluating ThermoDox® in 700 patients with HCC (the HEAT Study) indicated that a controlled administration of RFA heating time with ThermoDox® may dramatically improve patient survival. The findings were confirmed by an independent analysis by the National Institutes of Health. These specific lessons were incorporated into the design and execution of the OPTIMA Study.

ThermoDox® has received Fast Track Designation from the U.S. Food and Drug Administration (FDA), which provides for, among other things, priority review. ThermoDox® has also been granted orphan drug designation for primary liver cancer in both the U.S. and Europe. The European Medicines Agency (EMA) has confirmed that the OPTIMA Study would be acceptable as a basis for submission of a marketing authorization application (MAA). In China, the CNDA (former CFDA) has indicated that ThermoDox® may qualify for approval timelines of six months or less due to this important unmet medical need. In addition to meeting the Chinese FDA, U.S. FDA and European EMA enrollment objectives, the OPTIMA Study has also enrolled a sufficient number of patients to support registrational filings in South Korea, Taiwan, the Philippines, Thailand, Malaysia, and Vietnam, all major markets for ThermoDox®.

About Primary Liver Cancer

Primary liver cancer is one of the deadliest forms of cancer and ranks as the fourth most common solid-tumor cancer. The incidence of primary liver cancer today is approximately 30,000 cases per year in the United States, approximately 40,000 cases per year in Europe, and is rapidly growing worldwide to approximately 850,000 cases per year, with more than 50 percent occurring in China. The World Health Organization estimates that primary liver cancer may become the number one cancer worldwide, surpassing lung cancer, by 2020.

The standard first-line treatment for liver cancer is surgical resection of the tumor; however, over 80% of patients are ineligible for surgery. RFA has increasingly become the standard of care for non-resectable liver tumors, but the treatment becomes less effective for larger tumors. There are few non-surgical therapeutic treatment options available, as radiation therapy and chemotherapy are largely ineffective in the treatment of primary liver cancer.

About ThermoDox® and the Phase III OPTIMA Study

The Phase III OPTIMA Study has enrolled 550 patients in over 65 clinical sites in the United States, Europe, China and Asia Pacific, and will evaluate ThermoDox® in combination with optimized RFA, which was standardized to a minimum of 45 minutes across all investigators and clinical sites for treating lesions three to seven centimeters, versus optimized RFA alone. The primary endpoint for the trial is overall survival. The OPTIMA Study is supplemented by post-hoc analyses of data from the Company's 701-patient HEAT Study in which optimized RFA demonstrated the potential to significantly improve survival when combined with ThermoDox®. The OPTIMA Study's statistical plan calls for two interim efficacy analyses by an independent Data Monitoring Committee.

About Celsion Corporation

Celsion is a fully-integrated oncology company focused on developing a portfolio of innovative cancer treatments, including directed chemotherapies, immunotherapies and RNA- or DNA-based therapies. The Company's lead program is ThermoDox®, a proprietary heat-activated liposomal encapsulation of doxorubicin, currently in Phase III development for the treatment of primary liver cancer. The Company's pipeline also includes GEN-1, a DNA-based immunotherapy for the localized treatment of ovarian and brain cancers. Celsion has two platform technologies for the development of novel nucleic acid-based immunotherapies and other anti-cancer DNA or RNA therapies. For more information on Celsion, visit our website: http://www.celsion.com. (CLSN-LTSL/ThermoDox® CLSN-Optima Study/HCC)

Celsion wishes to inform readers that forward-looking statements in this press release are made pursuant to the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Readers are cautioned that such forward-looking statements involve risks and uncertainties including, without limitation, unforeseen changes in the course of research and development activities and in clinical trials; the uncertainties of, and difficulties in analyzing, interim clinical data, particularly in small subgroups that are not statistically significant; U.S. Food and Drug Administration and regulatory uncertainties and risks; the significant expense, time, and risk of failure of conducting clinical trials; the need for Celsion to evaluate its future development plans; possible acquisitions or licenses of other technologies, assets or businesses; possible actions by customers, suppliers, competitors, regulatory authorities; and other risks detailed from time to time in Celsion's periodic reports and prospectuses filed with the Securities and Exchange Commission. Celsion assumes no obligation to update or supplement forward-looking statements that become untrue because of subsequent events, new information or otherwise.

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