



Celladon's MYDICAR® and Small Molecule SERCA2a Studies in Acute and Chronic Heart Failure Selected as One of Windhover's Top 10 Cardiovascular Programs to Watch

SAN DIEGO--([BUSINESS WIRE](#))-- Celladon Corporation today announced that its SERCA2a programs for the treatment of patients with acute and chronic heart failure have been chosen by Windhover and an independent expert panel as one of the top 10 most interesting cardiovascular projects available for partnering. Projects available for partnering include MYDICAR®, a genetically targeted enzyme-replacement therapy intended to restore levels of SERCA2a and in phase 2 development; and CDN small molecule compounds that activate SERCA2a, as intravenous or oral drugs, for the treatment of acute and chronic heart failure. SERCA2a is a protein that is vital in the proper functioning of the heart.

"We are honored to have been chosen as having one of the most attractive cardiovascular opportunities the industry has to offer. Especially given the rigorous selection criteria, which includes strong science, unmet medical need, market potential, diversity of indications, multi-level partnering opportunities, potential for new opportunities beyond initial indications, and corporate stability," said Krisztina M. Zsebo, Ph.D., president and chief executive officer of Celladon Corporation. "We believe our extensive expertise and investigation of gene-based therapeutics for heart failure, and in particular, our encouraging data demonstrating improvements in cardiac function and overall condition of patients, sets us apart in the cardiovascular field."

Zsebo adds, "We have recently completed phase 2 enrollment of the double-blind portion of the Calcium Up-regulation by Percutaneous Administration of Gene Therapy in Cardiac Disease (CUPID) trial utilizing MYDICAR and expect Interim data results to be available mid-2010. We are also developing specific small molecule allosteric modulators of the SERCA2a enzyme for both acute and chronic heart failure."

The complete list of Windhover projects to date is available at www.windhover.com/taprojects. Each project has been hand-selected by Windhover's elite selection panel to ensure a high-quality slate of presenters.

CUPID Phase 2 Clinical Trial

The randomized, double-blind, placebo-controlled CUPID Phase 2 study is designed to examine the effect of MYDICAR® (AAV1/SERCA2a) in the treatment of severe heart failure. MYDICAR® is an enzyme-replacement therapy intended to restore levels of SERCA2a, a protein that is vital in the proper functioning of the heart. This CUPID trial enrolled 37 patients with severe forms of ischemic and dilated cardiomyopathies who had New York Heart Association Class III or IV heart failure, significantly impaired heart

pumping function, and less than half the normal ability to transport and utilize oxygen during cardiopulmonary exercise testing. Patients were treated with 1 of 3 doses of MYDICAR® or placebo via a single intracoronary infusion and will continue to be followed for 12 months after administration. Effects of treatment will be assessed by changes in how the heart contracts, a blood test of an important marker of heart failure called NT-proBNP, symptoms of heart failure and ability to exercise.

CUPID Phase 1 Clinical Trials

The phase 1 open-label, sequential dose escalation, multi-center phase of the trial was designed to investigate safety and biological effects of restoring SERCA2a enzyme activity in heart muscle cells. The phase 1 dose-escalation data demonstrated that MYDICAR® had an acceptable safety profile in 12 patients and 4 increasing doses. Safety was determined by study investigators and an independent safety monitoring committee. In addition, improvements from baseline to 6 months were observed across a number of key efficacy parameters important in assessing heart failure status. Efficacy was defined as the mean improvement in at least 2 of 5 endpoints without any worsening in the remaining endpoints, including a functional six-minute walk test, oxygen consumption, quality of life questionnaire, biomarker activity and left ventricular size and function.

Small Molecule Development of SERCA2a

The CDN small molecule trial is designed to activate SERCA2a, intravenously or orally, for the treatment of acute and chronic heart failure. These allosteric modulators of SERCA2a bind to the enzyme resulting in increased the activity, and enhance contractility of cardiomyocytes from normal and heart failure animals.

About Heart Failure

Chronic heart failure is an increasingly important health problem. It is the leading medical cause of hospitalization and is expected to result in an estimated direct and indirect cost to the healthcare system in 2009 of \$37.2 billion. About 5 million people in the United States have heart failure, and another 550,000 new cases are diagnosed each year. Heart failure contributes to or causes about 280,000 deaths annually. The most common symptoms of heart failure are shortness of breath, feeling tired, and swelling in the ankles, feet, legs, and sometimes the abdomen. There is no cure for heart failure.

About Celladon Corporation

Celladon Corporation, based in La Jolla, California, was launched in October 2004 as a privately held biotechnology company founded with the goal of becoming the leader in developing molecular therapies for the treatment of heart failure. The company's products target the key enzyme deficiency in advanced heart failure, SERCA2a, which regulates calcium cycling and contractility in heart muscle cells. Celladon's first product

candidate, MYDICAR®, delivers the gene for the SERCA2a enzyme. MYDICAR® is currently being tested in Phase 1 and 2 clinical trials. Celladon is also developing traditional small molecule activators of SERCA2a for the treatment of heart failure. To learn more about Celladon, visit Celladon's website at www.celladon.net.

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