



Celladon Receives Breakthrough Therapy Designation from FDA for MYDICAR[®], Novel, First-in-Class Therapy in Development to Treat Heart Failure

-- MYDICAR is First Gene Therapy Reported to Receive Breakthrough Designation from FDA's Center for Biologics Evaluation and Research (CBER) –

SAN DIEGO, April 10, 2014 – Celladon Corporation (NASDAQ: CLDN), a clinical-stage biotechnology company developing novel therapies for patients with heart failure and other diseases characterized by SERCA enzyme deficiencies, today announced that its lead product candidate, MYDICAR[®], has been granted breakthrough therapy designation by the U.S. Food and Drug Administration (FDA) for reducing hospitalizations for heart failure in NYHA class III or IV chronic heart failure patients who are NAb negative. This designation is intended to expedite the development and review of drugs for serious or life-threatening conditions and where preliminary clinical evidence suggests it provides a substantial improvement over existing therapies. Celladon is developing MYDICAR as a novel, first-in-class therapy for patients with chronic heart failure due to systolic dysfunction. MYDICAR uses genetic enzyme replacement therapy to correct the deficiency in the enzyme SERCA2a, which is an enzyme that becomes deficient in heart failure patients and results in inadequate pumping of the heart. Celladon has developed a companion diagnostic to identify the patients who are AAV1 NAb negative and therefore eligible for MYDICAR treatment.

“We are looking forward to working with the senior staff at the FDA to determine the most expeditious path to bring MYDICAR to patients with advanced heart failure. This breakthrough therapy designation validates MYDICAR's unique characteristics and clinical data to date and underscores the urgent need for new treatments for heart failure,” said Krisztina Zsebo, Ph.D., president and CEO of Celladon. “MYDICAR has the potential to provide transformative disease-modifying effects with long-term benefits in heart failure patients with a single administration. Our goal is to bring MYDICAR to market as quickly as possible in the United States, where we estimate approximately 350,000 heart failure patients with currently limited remaining treatment options could be eligible for therapy.”

Celladon is currently evaluating MYDICAR in the Phase 2b CUPID 2 trial to determine its efficacy in reducing the frequency of and/or delaying heart failure-related hospitalizations. This randomized, double-blind, placebo-controlled, multinational trial is evaluating a single intracoronary infusion of MYDICAR versus placebo added to a maximal, optimized heart failure regimen in patients with NYHA class III or IV symptoms of chronic heart failure due to systolic dysfunction. Patient enrollment has been completed and 250 patients have been randomized in this trial. The Company expects to report results in April 2015.

About Breakthrough Therapy Designation

The breakthrough therapy designation was enacted as part of the FDA Safety and Innovation Act of 2012 (FDASIA). The breakthrough therapy program is intended to streamline drug development and review of innovative new medicines that address certain unmet medical needs for serious or life-threatening diseases or conditions. The criteria for breakthrough therapy

designation require preliminary clinical evidence indicating that the drug may demonstrate a substantial improvement over existing therapies on at least one clinically significant endpoint. A breakthrough therapy designation conveys all of the fast track program features, as well as a commitment that FDA will work closely with the drug sponsor on an efficient drug development program. The statute calls for reducing exposure of patients to a potentially less-effective active control drug. However, FDA intends to expedite the development and review of a breakthrough therapy by intensively involving senior managers and experienced review staff in a proactive, collaborative, cross-disciplinary review.

About Heart Failure

Heart failure is the inability of the heart to pump blood efficiently due to weakening and enlargement of the ventricles. Nearly 6 million individuals are currently diagnosed with heart failure in the United States according to the American Heart Association (AHA). Despite optimal guideline-directed therapies employing a wide range of pharmacologic, device and surgical options, many heart failure patients deteriorate over time. The long-term prognosis associated with heart failure is worse than that associated with the majority of cancers, with a mortality rate of approximately 50 percent at five years following initial diagnosis. In the United States, over one million primary heart failure-related hospitalizations and over 280,000 heart failure-related deaths occur annually. The one- and six-month readmission rates after heart failure-related hospitalization are close to 25 and 50 percent, respectively, and there is growing pressure on hospitals to reduce readmissions for heart failure. According to a recently published review article in *Journal Clinical Cardiology*, the estimated direct cost of heart failure in the United States in 2012 was greater than \$60 billion, over half of which was related to repeated hospitalizations.

In patients with heart failure, SERCA2a, an enzyme critical to the contraction of the cardiac muscle cell, becomes deficient. Numerous human studies have established a clear association between depleted SERCA2a enzyme in cardiac cells and the progression of end-stage heart failure.

About MYDICAR

MYDICAR uses gene therapy to selectively target and restore SERCA2a enzyme levels by transferring the SERCA2a gene directly into cardiac muscle cells, which improves the heart's ability to pump blood. MYDICAR utilizes a non-pathogenic recombinant adeno-associated virus (AAV) and is delivered directly to the heart in a routine outpatient procedure, similar to an angiogram, in a cardiac catheterization laboratory.

Results of the 39-patient Phase 2a CUPID 1 clinical trial of a single intracoronary infusion of high-dose MYDICAR in patients with advanced heart failure due to a systolic dysfunction showed the therapy was safe and well tolerated in the study. In CUPID 1, MYDICAR reduced heart failure-related hospitalizations and improved patients' symptoms, quality of life and key markers of cardiac function predictive of survival, such as elevated levels of natriuretic peptides and left ventricular end systolic volume. Long-term follow-up results from CUPID 1 showed that in the additional two-year follow-up period, the durability of reduced cardiovascular and terminal events previously observed in the MYDICAR high-dose cohort at 12 months was maintained. The risk of recurrent cardiovascular events in the presence of terminal events over three years of follow up was reduced by 82 percent in the high-dose group compared with the placebo group ($p=0.048$). No safety concerns were noted during the three-year follow-up period for patients who received MYDICAR.

About Celladon Corporation

Celladon, a clinical-stage biotechnology company, is a leader in the field of calcium dysregulation. The Company is targeting SERCA enzymes to develop novel therapies for diseases with significant unmet medical needs, such as heart failure. Its therapeutic portfolio for diseases characterized by SERCA enzyme deficiency includes both gene therapies and small molecule compounds. MYDICAR[®], its most advanced product candidate, uses gene therapy to target SERCA2a, an enzyme that is deficient in heart failure patients. Its small molecule platform of SERCA2b modulators includes a number of potential first-in-class compounds that address novel targets in diabetes and neurodegenerative diseases. For more information, visit www.celladon.com.

Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements regarding the extent of the role MYDICAR may have in improving the clinical course of heart failure patients, Celladon's goal to bring MYDICAR to market as quickly as possible, the estimated number of patients who could be eligible for MYDICAR therapy if approved, the anticipated timing for reporting results from CUPID 2, estimates regarding the size of the heart failure market, as well as the extent to which the breakthrough therapy designation may streamline and expedite development of MYDICAR. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. These forward-looking statements are based upon Celladon's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks and uncertainties associated with the process of conducting product development activities and clinical trials and obtaining regulatory approval to commercialize product candidates, Celladon's reliance on third parties, the need to raise additional funding when needed in order to conduct Celladon's business, and the degree of market acceptance of MYDICAR by physicians, patients, third-party payors and others in the medical community. These and other risks and uncertainties are described more fully in Celladon's filings with the Securities and Exchange Commission, including without limitation its Annual Report on Form 10-K. All forward-looking statements contained in this press release speak only as of the date on which they were made. Celladon undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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