Celladon Corporation Announces Receipt of EMA Scientific Advice for MYDICAR® and Initiation of AGENT-HF Trial

--Ongoing CUPID 2 Study Potential to Serve as Primary Basis of Efficacy and Safety Required for MMA Submission in European Union--

San Diego, California, December 11, 2013/PRNewswire/ -- Celladon Corporation, a clinical-stage biotechnology company focused on developing novel therapies by applying its leadership position in the field of SERCA enzymes, today announced that the Company has received requested scientific advice from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) relating to its product candidate MYDICAR®. The CHMP confirmed that if MYDICAR demonstrates a substantial and a highly significant treatment effect in the advanced heart failure population, and assuming that no untoward effects are attributed to MYDICAR, a safety database of approximately 205-230 subjects may be sufficient for a safety assessment to allow for acceptance of a Marketing Authorization Application (MAA) for review. The CHMP also recommended long-term follow-up of 5 years for patients in the CUPID 2 study, in accordance with EMA's guideline for gene therapy medicinal products.

"We are very pleased with the feedback from the CHMP. Assuming the results from our ongoing CUPID 2 trial are favorable, based on this feedback we believe our CUPID 2 trial can serve as the primary basis for marketing approval of MYDICAR by the EMA, in which case no further phase 3 studies would be required prior to an MAA submission," said Krisztina Zsebo, Ph.D., President and Chief Executive Officer of Celladon Corporation.

Separately, Celladon also announced that investigators have initiated the AGENT-HF Trial (AAV1-CMV-SERCA2a Gene Therapy Trial in Heart Failure). This trial is an investigator-initiated clinical trial and is partially funded by the French government and sponsored by Assistance Publique – Hôpitaux de Paris. This trial is not required by any regulatory authorities for systolic heart failure indications, but the patients enrolled in this trial will be included in the overall safety database submitted in an MAA submission.

The primary objective of the AGENT-HF Trial is to determine whether treatment with MYDICAR leads to reverse remodeling of the heart. In patients with heart failure, the size, shape, structure and physiology of their heart changes over time, and these changes that lead to a progressive decline in left ventricular (LV) function are referred to as remodeling. In reverse remodeling, there would be changes back to the more normal, healthier state of the heart along with an improvement in the functioning of the heart. This trial will enroll approximately 44 heart failure patients in France with half receiving MYDICAR and the other half placebo. The primary endpoint at six months will be change, compared to baseline, in LV end systolic volume as measured by cardiac computed tomography.

About the CUPID Phase 2b Trial

The CUPID Phase 2b trial was initiated in August 2012 and will enroll up to 250 patients in approximately 60 sites worldwide. Patients will first be prescreened for the presence of AAV neutralizing antibodies. Those patients with a negative titer will undergo further screening tests

and procedures to determine eligibility prior to randomization and enrollment into the study. All patients will be randomized in parallel to MYDICAR (1×10^{13} Dnase resistant particles AAV1/SERCA2a) or placebo in a ratio of 1:1. The primary objective is to determine the efficacy of MYDICAR added to an optimal heart failure regimen in patients with ischemic or dilated cardiomyopathy and moderate to advanced symptoms of heart failure by reducing the frequency and/or delaying heart failure-related hospitalizations and episodes of ambulatory worsening heart failure compared to placebo-treated patients. The primary efficacy endpoint is time-to-recurrent heart failure-related hospitalizations in the presence of terminal events (all-cause death, heart transplant, left-ventricular assist device (LVAD) implantation). The secondary efficacy endpoint is the time-to first terminal event (all-cause death, heart transplant, LVAD implantation). Exploratory endpoints include changes from baseline in New York Heart Association class, 6 minute walk test distance, NT-proBNP and quality of life (KCCQ) score. Safety will be evaluated by determining the incidence and severity of adverse events and changes in laboratory parameters in MYDICAR versus placebo-treated subjects. Safety evaluations include all adverse events (including procedure-related), concomitant medications, vital signs, electrocardiography parameters, physical exams, implantable cardioverter defibrillator (ICD) interrogations, laboratory evaluations and the time-to cardiovascular-related death.

About Celladon

Celladon is a clinical-stage biotechnology company applying its leadership position in the field of calcium dysregulation by targeting SERCA enzymes to develop novel therapies for diseases with tremendous unmet medical needs. Sarco/endoplasmic reticulum Ca^{2+}-ATPase, or SERCA, enzymes are a family of enzymes that play an integral part in the regulation of intra-cellular calcium in all human cells. Calcium dysregulation is implicated in a number of important and complex medical conditions and diseases, such as heart failure, diabetes and neurodegenerative diseases. Celladon's therapeutic portfolio for diseases characterized by SERCA enzyme deficiency includes both gene therapies and small molecule compounds. MYDICAR, Celladon's most advanced product candidate, uses gene therapy to target SERCA2a, which is an enzyme that becomes deficient in patients with heart failure. In a 39-patient randomized, double-blind, placebo-controlled phase 2a trial in patients with systolic heart failure, which is referred to as CUPID 1, MYDICAR was found to be safe and well-tolerated, reduced heart failure-related hospitalizations, improved patients' symptoms, quality of life and serum biomarkers, and improved key markers of cardiac function predictive of survival, such as end systolic volume in high dose MYDICAR-treated versus placebo patients. Based on these results, as well as Celladon's previous preclinical studies and clinical trials, Celladon has advanced MYDICAR to a 250-patient randomized, double-blind, placebo-controlled international phase 2b trial in patients with systolic heart failure, which is referred to as CUPID 2. Celladon expects to complete enrollment of CUPID 2 in the first half of 2014 and announce results from this trial in mid-2015.

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For further information, please contact:

Fredrik Wiklund
Vice President, Corporate Development and Investor Relations
+1- 858-432-7215
fwiklund@celladon.net