

Celladon Corporation Announces MYDICAR® Enzyme Replacement Therapy for Advanced Heart Failure Provides Sustained Reduction in Clinical Events in Patients for 18 Months Compared with Placebo

CUPID Trial Data Presented Today at American Society for Cell and Gene Therapy Conference

SEATTLE, Wash., May 23, 2011 /PRNewswire/ -- Celladon Corp., a biopharmaceutical company focused on the discovery and development of innovative treatments for cardiovascular diseases, today announced that 18-month data from its Phase 2 CUPID clinical trial of MYDICAR® demonstrated continued improvements in clinical outcomes in advanced heart failure patients treated with the genetically-targeted enzyme replacement therapy.

"We are pleased to present data from the long-term follow-up portion of the CUPID trial with MYDICAR demonstrating reduced hospitalizations and other cardiovascular events at 18 months after treatment," said Krisztina Zsebo, Ph.D., CEO and President of Celladon, who presented the study's long-term follow-up results during the American Society of Cell and Gene Therapy 2011.

The study of 39 patients met its primary safety and efficacy endpoints at 6 months for high dose MYDICAR versus placebo. Additionally, after 12 months of receiving a single infusion of MYDICAR, patients treated with the highest dose versus placebo had an 88 percent risk reduction (Hazard Ratio = 0.12, $P=0.003$), of major cardiovascular events such as:

- Death
- Need for left ventricular assist device (LVAD) or cardiac transplant
- Episodes of worsening of heart failure

Additionally, the 18-month CUPID data from long-term follow-up demonstrate a durable benefit in preventing major cardiovascular events.

The 12 month data presented in 2010 showed that heart failure, which is a progressive disease, became stabilized in high dose MYDICAR-treated patients: heart failure symptoms, exercise tolerance, serum biomarkers and cardiac function essentially improved or remained stable while these parameters deteriorated substantially in patients treated with placebo and concurrent optimal drug and device therapy.

We believe the efficacy sustained in patients over a 18-month period strongly support the continued development of MYDICAR for a Phase 3 study and commercialization," said Roger Hajjar, M.D., Director, Cardiovascular Research Center, Mount Sinai School of Medicine, NY, and co-founder of Celladon. "The clinical deterioration seen in the placebo patients receiving ongoing optimal standard therapy emphasizes the tremendous unmet medical need in people with advanced heart failure. Based on these results we are encouraged that MYDICAR can fill this need."

The CUPID Trial

The CUPID trial (Calcium Up-regulation by Percutaneous administration of gene therapy In cardiac Disease) is a randomized, double-blind, placebo-controlled study to assess the efficacy and safety of MYDICAR®, a genetically targeted enzyme replacement therapy for advanced heart failure. Enrolled patients had severe forms of the disease defined by New York Heart Association Class III or IV heart failure, significantly impaired pumping function of their hearts (ejection fraction ≤ 35 percent), and less

than half the normal ability to transport and utilize oxygen during exercise testing ($VO_{2max} \leq 20$ mL/kg/min). The CUPID trial ClinicalTrials.gov Identifier is NCT00454818.

Primary outcome measures included safety, worsening of heart failure leading to hospitalization, frequency of and time to cardiac transplantation or LVAD implantation, changes in patients' ability to exercise, echocardiographic assessments, a blood test for NT-proBNP, and symptoms of heart failure.

About MYDICAR®

MYDICAR® is a genetically targeted enzyme replacement therapy intended to restore levels of SERCA2a, a regulator of calcium cycling and contractility. SERCA2a levels decline in all forms of late-stage heart failure resulting in deficient heart function. With MYDICAR®, the SERCA2a gene is delivered using a recombinant adeno-associated virus (AAV) as the vector. AAV is a naturally occurring virus not associated with any disease in humans. MYDICAR® is delivered in a single dose directly to the heart muscle during a routine outpatient cardiac catheterization procedure, similar to an angiogram. MYDICAR® is synergistic and additive across current heart failure treatments such as ACE inhibitors, beta-blockers, spironolactone/diuretics, and biventricular pacing devices. No treatment substitution decision is required by the treating physician.

About Heart Failure

Chronic heart failure is a leading cause of hospitalization and is expected to result in direct and indirect costs of \$39.2 billion to the U.S. healthcare system in 2010. Nearly 6 million people in the U.S. have heart failure, and at least 670,000 new cases will be diagnosed this year. Heart failure leads to 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.

About Celladon

Celladon Corp., based in La Jolla, Calif., was launched in October 2004 as a privately held biotechnology company with the goal of becoming the leader in developing molecular therapies for the treatment of heart failure. The company's products target calcium cycling and contractility deficit in heart muscle cells. In addition to MYDICAR®, Celladon is developing traditional small molecule activators of SERCA2a for the treatment of heart failure. To learn more about Celladon, visit Celladon's Web site at www.celladon.net.

CONTACT: Krisztina Zsebo, +1-858-366-4288, for Celladon Corporation