



Celladon Corporation Announces MYDICAR® Enzyme Replacement Therapy for Advanced Heart Failure Provides Sustained Clinical Improvements in Patients for 12 Months Compared with Placebo

CUPID Trial Data Presented Today at American Heart Association Conference

Chicago, November 15, 2010 – Celladon Corp., a biopharmaceutical company focused on the discovery and development of innovative treatments for cardiovascular diseases, today announced that 12-month data from its Phase 2 CUPID clinical trial of MYDICAR® demonstrated significant improvements in clinical outcomes and key disease markers in advanced heart failure patients treated with the genetically-targeted enzyme replacement therapy.

“Physicians and patients have long believed that a failing heart cannot be repaired, but the CUPID trial with MYDICAR adds support to other recent studies suggesting that the deterioration of heart function can indeed be slowed down, resulting in reduced hospitalizations and other cardiovascular events,” said Donna Mancini, M.D., medical director of cardiac transplantation at New York-Presbyterian Hospital/Columbia University Medical Center and professor of medicine at Columbia University College of Physicians and Surgeons, who presented the study’s one-year results during the American Heart Association Scientific Sessions 2010. “I am very encouraged that our approach of restoring a critical enzyme that is depleted in such advanced patients will enhance our understanding of the human heart.”

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
- Number of heart failure-related hospitalizations

The mean duration of hospitalization in the MYDICAR high dose group during the 12-month period was 0.4 days per patient compared with 4.5 days per patient in the placebo group. This finding is especially noteworthy because heart failure is the leading cause of hospitalization in Americans 65 and older.

Additionally, the 12-month CUPID data show 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 the same while these parameters deteriorated substantially in patients treated with placebo and concurrent optimal drug and device therapy.

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 heart failure 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 ($VO_2\text{max} \leq 20$ 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 recombinant adeno-associated viral vector (AAV), a naturally occurring virus that is not associated with any disease in humans. MYDICAR® is delivered in a single dose directly to the heart muscle during a short outpatient procedure that is performed in a cardiac catheterization laboratory. MYDICAR® is synergistic and additive across current heart failure treatments such as ACE inhibitors, b-blockers, spironolactone/diuretics, and biventricular pacing devices. No treatment substitution decision is therefore 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.