

MAF D21CA-042 - Developing Gene-Editing Tools for Heart Disease

UNIVERSITY OF FLORIDA

Projected Duration: 3 Years **Study Cost:** \$80,316 **DCAF Grant \$12,000** **1/2022**

SUMMARY: Researchers will use novel gene-editing tools to correct damaging mutations in heart cells to help advance gene therapies for dilated cardiomyopathy, a common canine heart disease.

DESCRIPTION: Dilated cardiomyopathy (DCM) is the third most frequent type of heart disease diagnosed in dogs, with some breeds at higher risk. In Doberman pinschers, DCM prevalence is between 45% and 63%, suggesting a strong genetic component to the disease in this breed. Using stem cells from Doberman pinschers and differentiating them into heart cells, researchers will test the potential of a new gene-editing strategy to correct defects in genes important for heart function in dogs. Specifically, the team will edit a known mutation in two proteins associated with DCM and measure its impact on heart cell function. Findings will help inform future studies toward the development of gene therapies for dogs with DCM and other heart diseases.