



# ECD GLOBAL ALLIANCE

Supporting those affected by  
Erdheim-Chester Disease worldwide.

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## FOR IMMEDIATE RELEASE

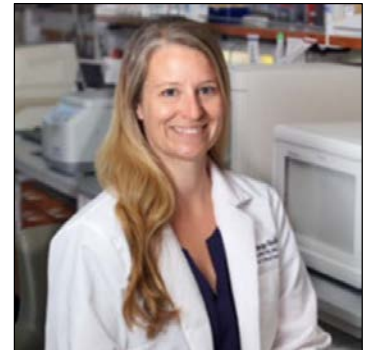
### San Diego Researcher Awarded 2019 ECDGA Research Grant

*Erdheim-Chester Disease Global Alliance awards research grant to the University of California-San Diego to improve the understanding of ECD and LCH*

DeRidder, LA – March 30, 2020 – The Erdheim-Chester Disease Global Alliance (ECDGA) awarded a new research grant to University of California, in San Diego, CA, not only to improve the understanding of Erdheim-Chester Disease (ECD) and Langerhans cell histiocytosis (LCH) associated with neurodegeneration, but to generate hypotheses and models that will allow for novel drug screening standards. The goal is to ultimately improve the treatment of the devastating neurological form of this disease. Primary Investigator, Nicole G. Coufal, M.D., Ph.D., will lead the study.

The aim of this study is to generate stem cell models for LCH/ECD associated neurodegeneration. This will be accomplished by reprogramming blood samples from patients with *BRAF* mosaicism to stem cells and differentiating those stem cells to microglia, the inflammatory cells of the brain.

*“Thank you so much for the ECD grant, this funding will support an exciting early stage project to make individualized stem cells from ECD patients in order to try to understand why some ECD patients have neurodegenerative symptoms. The hope is by being able to make a novel model for ECD in a dish we will not only improve our understanding of why neurodegeneration occurs in ECD but open avenues to screen for new treatments. These studies would not be possible without support from the ECDGA.” – Dr. Nicole Coufal*



The ECDGA is excited to be funding this groundbreaking work and believes it will result in a brighter future for ECD patients and a better understanding of other blood cancers.

Research is a mission-critical goal for the ECDGA. The organization has previously awarded nine (9) medical research grants, devoting over \$650K to ECD medical research. Funding for grants comes from generous, private donations. ECD research efforts, backed in part by the non-profit, have recently led to an FDA approval of a drug called vemurafenib, originally developed to treat other types of cancers, such as melanoma skin cancer, to treat ECD patients with the *BRAFV600E* mutation.

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Erdheim-Chester Disease is an ultra-rare condition with no known cause and is often misdiagnosed. It is considered a histiocytic neoplasm (type of blood cancer). The illness is characterized by the accumulation of histiocytes, cells that normally fight infections, in tissue and organs. The tissue and organs become dense and fibrotic due to the infiltration of the histiocytes and can lead to organ failure unless a successful treatment is found

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