

## SMALL-TOWN NON-PROFIT GRANTS BIG FUNDS FOR LEADING RESEARCH

### *INNOVATIVE RESEARCH MADE POSSIBLE THROUGH THE ERDHEIM-CHESTER DISEASE GLOBAL ALLIANCE*

**DERIDDER, LA, November 11, 2015** – The Erdheim-Chester Disease Global Alliance (ECDGA) awards two (2) grants in 2015 for top Erdheim-Chester Disease (ECD) research projects from leading research institutions. Two teams of investigators benefited from the endowments. Collaborating on “Understanding and Targeting Novel Molecular Alternations in Erdheim-Chester Disease without the BRAFV600E Mutation” are Filip Janku, MD, Ph.D. from The University of Texas MD Anderson Cancer Center and Omar Abdel-Wahab, MD from Memorial Sloan-Kettering Cancer Center. The second winning grant awarded investigators, Eli L. Diamond, MD and Vaios Hatzoglou, MD, both from Memorial Sloan-Kettering Cancer Center. Their project is “A Clinical, Structural, and Functional Neuroimaging Study of Cognition in Erdheim-Chester Disease”. Both studies will begin in 2016 and are to take place in each investigator’s research institution.

The Janku & Abdel-Wahab study focuses on understanding the genetic alterations in ECD patients. Currently about 50% of ECD patients test positive for a BRAFV600E mutation. For these patients, promising treatments are currently being studied. The funded study will research to find other genetic alterations that may be present in ECD patients. The team hopes, as Janku writes in his proposal abstract, “to provide novel therapeutic approaches for *BRAFV600-wildtype* ECD patients.” The ECD community is hopeful that this will translate to targeted therapies being available for most ECD patients in the near future.

MSKCC medical researchers, Diamond and Hatzoglou will attempt to understand and characterize neurological abnormalities in ECD patients. According to the Diamond and Hatzoglou study abstract, even ECD patients without neurological ECD tumors “suffer from neurocognitive, psychological, and behavioral dysfunction.” Dr. Diamond goes on to write, “This remains an unexplored and poorly understood phenomenon, although in our experience it is of paramount importance to ECD patients and their families as it affects familial relationships and employment.” This study will try to compile information to help find better treatment for patients with these issues.

Both of the funded research projects bring hope that findings will positively influence the lives of ECD Patients. Research is a mission critical goal for the ECDGA. The organization has awarded four (4) medical research grants in the short six-year period since they incorporated, representing over \$400K devoted to medical research. Money for these grants comes from generous private donations. ECD Research efforts, backed in part by the non-profit, have recently led to the BRAFV600E discovery that allows physicians to treat ECD using drugs that were originally developed to treat other cancers originating in other parts of the body.

Erdheim-Chester Disease is an ultra-rare condition with no known cause and is very often misdiagnosed. It is considered a non-Langerhans histiocytosis, cancer-like (neoplastic) disease. The illness 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. Currently,

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Erdheim-Chester Disease Global Alliance  
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there is no cure for ECD but breakthrough ECD research is at the forefront of science and “precision medicine,” illustrating that therapies targeting a specific genetic mutation can be effective, regardless of where cancer originates.

Learn more about Erdheim-Chester Disease or these trials please visit [www.erdheim-chester.org](http://www.erdheim-chester.org).

About the Erdheim-Chester Disease Global Alliance:

The ECD Global Alliance, founded in 2009, is a 501(c) 3 non-profit organization dedicated to awareness, support, education, and research related to Erdheim-Chester Disease. To donate, please send a check to ECD Global Alliance | P.O. Box 775|DeRidder, LA 70634 or go online to <http://www.razoo.com/story/Ecd-Global-Alliance> Thank you for your support!

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