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Erdheim-Chester Disease Global Alliance
337-404-6033
www.erdheim-chester.org

ITALIAN SCIENTISTS WIN GRANT FROM GRASSROOTS LOUISIANA NON-PROFIT

THE ECD GLOBAL ALLIANCE AWARDS ERDHEIM-CHESTER DISEASE RESEARCH GRANT FOR NEW TREATMENT POSSIBILITIES USING THE RCCS™ BIOREACTOR

DERIDDER, LA, January 11, 2016 – The Erdheim-Chester Disease Global Alliance (ECDGA) awards a new research grant for scientists in Milan, Italy studying Erdheim-Chester Disease (ECD). Primary investigators, Marina Ferrarini, MD and Lorenzo Dagna, MD from San Raffaele Scientific Institute benefited from the endowment. Collaborating on "Tailoring Treatment for Erdheim-Chester Disease", Ferrarini and Dagna anticipate the results of this study will provide a greater understanding of the molecular mechanisms responsible for the ultra-rare condition. Using this knowledge, they seek to discover better-targeted treatments for ECD patients. The study is set to begin in 2016 and to take place at the investigators' research institution using a state-of-the-art cell culture system, the RCCS™ bioreactor.

The Ferrarini and Dagna study focuses on understanding how malignant cells in ECD patients accumulate and interact with neighboring normal cells to fuel the disease, causing lesions and tumors to form. With this understanding, the discovery of a treatment strategy that can interrupt the processes, stopping the disease from progressing, may be possible.

No treatments for Erdheim-Chester Disease are FDA or EMA (European Medicine Agency) approved. With this investigation, scientists expect to find therapies that are more promising and thus become one-step closer to someday having FDA/EMA approved ECD treatments.

Research findings suggest that about 50% of ECD patients test positive for the BRAFV600E mutation. For many of these patients, current studies offer promising treatments. However, for those who test negative for the BRAF mutation, or have tested positive but have had suboptimal results with currently available therapies, the endowed project attempts to bridge the gap in treatment. The investigation is fundamental to helping all ECD patients regardless of genetic mutation type. The team will compare the effectiveness of currently available off-label drugs to find those that are best suited for successful treatment of ECD. Findings from this project have the potential to improve current treatments as well as design combination therapies for more effective disease-fighting results.

In addition to revolutionizing treatment for ECD patients, the study uses recently developed tissue culture technology, the RCCS™ bioreactor, to optimize results. The RCCS™ bioreactor, originally developed by NASA, preserves three-dimensional organization and viability of tissue samples, thus providing the unique possibility to analyze the impact and efficacy of a given drug on ECD tissues. The research team, using the cultivated tissue, will compare the effectiveness of drugs currently available that are used to fight other diseases, to find those medicines that offer the best potential as successful treatments of ECD.

Research is a mission critical goal for the ECDGA. The organization has previously awarded six (6) medical research grants in the short six-year period since it has existed, representing over $500K 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 use of drugs, originally developed to treat other types of cancers, such as melanoma skin cancer, to treat ECD patients with the BRAFV600E mutation. This breakthrough ECD research is at the forefront of science and "precision medicine," illustrating that therapies targeting a particular genetic mutation can be effective, regardless of where a cancer originates.

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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 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.

Learn more about ECD and other studies by visiting 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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