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FOR IMMEDIATE RELEASE

DRUG TRIAL FOR ERDHEIM-CHESTER DISEASE PATIENTS NOW RECRUITING

MD Anderson in Houston, Texas takes lead on dabrafenib and trametinib drug trial.

DERIDDER, LA, January 25, 2019 – The Erdheim-Chester Disease Global Alliance (ECDGA) announces the launch of the *Phase II Therapeutic Trial of the use of Dabrafenib and Trametinib in patients with BRAF V600 Mutations positive lesions in Erdheim Chester Disease*, conducted by the National Cancer Institute (NCI) and M.D. Anderson Cancer Center. Filip Janku, MD, PhD, is the Principal Investigator leading the research team at MD Anderson Cancer Center (MDA) to collect patient data in order to better understand the impact of dabrafenib and trametinib on Erdheim-Chester disease.

This phase II trial studies the side effects and how well dabrafenib and trametinib work in treating patients with Erdheim-Chester disease that have BRAF V600 gene mutations. Dabrafenib and trametinib may stop the growth of tumor cells by blocking some of the enzymes needed for cell growth.

The primary objective of this trial is to study the efficacy and safety of dabrafenib and trametinib as combination therapy in patients with BRAF V600E positive Erdheim-Chester disease. As well as determine the clinical response rate to dabrafenib and trametinib combination therapy in patients with BRAF V600E positive Erdheim Chester disease.

This study holds promise as this combination treatment may come with fewer negative side-effects for patients. In addition, it may be that the positive effects of treatment will continue longer than a mono-treatment which can cease to be as effective when treatment resistance occurs.

Drug trials such as this help accomplish FDA-approved treatments that are lifesaving. Rare disease research is essential for medical science discoveries, offering targeted perspectives on cancer in general. Repurposing existing drugs eliminates a large amount of funding and allows a timelier solution for the 30 million Americans fighting rare disease. A trial of this nature brings hope to the small ECD community. Having scientifically-proven effective treatments means access to these treatments becomes easier for patients.

Choosing to participate in a study is an important personal decision. Families interested in joining a clinical trial are encouraged to consult with their doctor and family members or friends about deciding to join a study.

To learn more about the *Phase II Therapeutic Trial of the use of Dabrafenib and Trametinib in patients with BRAF V600 Mutations* and how to contact them, visit ClinicalTrials.gov website: [Erdheim-Chester Disease Clinical Trial](#)



Filip Janku MD, PhD, Principal Investigator, MDA

How to learn more:

ECDGA website: www.erdheim-chester.org

ClinicalTrials.gov website: [Erdheim-Chester Disease Clinical Trial](#)

Principal Investigator Contact: Janku, Filip, email: FJanku@mdanderson.org

Find other trials available to ECD patients here: <http://erdheim-chester.org/studies-trials/>

[Learn About Clinical Studies](#)

[Erdheim-Chester Disease](#) is an ultra-rare condition that is believed to be under diagnosed. 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.

The [ECD Global Alliance](#) is a 501(c)(3) non-profit organization dedicated to awareness, support, education, and research related to Erdheim-Chester Disease.

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