Erdheim-Chester Disease Global Alliance

2018 Trials and Studies

Patient Guide to Active ECD Studies & Trials

The following trials and studies are recruiting ECD patients as of August 2017. More details about these trials can also be found on our website at the address http://erdheim-chester.org/studies-trials/.

Benefits of Clinical Trials to You & the ECD Community

- The drugs are usually provided free of charge to the patient. In some cases testing and travel may also be provided as part of the trial. Patients should always ask what costs are included.
- Follow-up appointments are very thorough and side effects are closely monitored and treated.
- Trials can lead to FDA/EMA approval for the treatment of ECD. With FDA/EMA approval, payers will be more likely to approve payment for the treatment.
- With a limited number of ECD patients, it is very important that data on the treatment be captured centrally to aid in the understanding of things such as: (a) how long should patients be kept on the treatment, (b) what, if any, indicators exist for possible treatment issues that might arise, etc.

Here are helpful resources to help decide if a drug trial or study participation is right for you.

- Demystifying the Clinical Trial – by Mary Elizabeth Davis, RN, MSN, AOCNS and Allison Hyde, RN, BSN, Memorial Sloan Kettering Cancer Center
- ECD and Clinical Trials – by Dr. Filip Janku, MD Anderson Cancer Center
- Learn about Clinical Studies – National Institutes of Health
- The ECD Global Alliance list of trials are also located here: http://erdheim-chester.org/studies-trials/.

Thank you for considering participation in this option for your care and treatment. Please contact the organization for more assistance.

	Principal Investigator(s)/ Institution	Study	Objective	Patient Involvement	Contact Information
1.	Allen, Carl McClain, Kenneth Texas Children's Hospital, Houston, TX USA	Spit for A Cure: New Study to Define the Role of Inheritance in Histiocytic Diseases	Analyze and compare the genetic information of a patient and the parents from several hundred families to identify and uncover the inherited risk factors in Histiocytic Diseases. The goal is to enroll 500 patients and their parents. All patients with a history of any histiocytic disorder and their parents are welcome to participate in this study.	ECD patients with both natural parents still living, are encouraged to enroll. Saliva samples will be collected from the patient and both parents.	(Allen) 832-822-4212 email: ceallen@txch.org; (McClain) 832-822-4208 email: klmcclai@txch.org
2.	Allen, Carl McClain, Kenneth MD Anderson Cancer Center, Houston, TX, USA	Developing a Comprehensive Genomic Database – Histiocytosis Research Laboratory	Understand the basic causes of histiocytosis which hopefully will lead to more effective treatments.	ECD patients' surgical biopsies, blood samples, and/or cerebrospinal fluid, along with medical information, is being sought.	(Allen) 832-822-4212 email: ceallen@txch.org (McClain) 832-822-4208 email: klmcclai@txch.org
3.	Aouba, Achille Department of Internal Medicine and Clinical Immunology, CHU Côte de Nacre-Caen, France	Observational Multi-center Study on Erdheim-Chester Disease treated with Anakinra	To establish the objective efficacy and safety results recorded with Kineret® (Anakinra) for ECD patients and its effect on various symptoms and organs. This study is opened to ECD patients who are or have been previously treated with Kineret® (Anakinra).	Consent to have your doctor supply the principal investigator with information about your case. Your identity will not be disclosed.	Tel: 00 33 2 31 06 45 79; Fax: 00 33 2 31 06 49 54 email: achille.aouba@gmail.com or aouba-a@chu-caen.fr;

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4.	Braiteh, Fadi Comprehensive Cancer Centers of Nevada	An Open Label Phase 2 Multicohort Trial of Nivolumab in Advanced or Metastatic Malignancies clinicaltrails.gov ID NCT02832167	The purpose of this study is to determine whether Nivolumab is an effective treatment for advanced or metastatic cancer. The study will evaluate the clinical benefit rate of Nivolumab in subjects at 16 weeks from enrollment. Various advanced or metastatic tumor types are eligible for enrollment. Subjects must have received prior standard of care treatment for their cancer before enrollment.	ECD patients 18 Years and older	Fadi Braiteh, 1-702-952-3400, fadi.braiteh@usoncology.com
5.	Diamond, Eli Memorial Sloan-Kettering, New York City, NY USA	Phase 2 Trial of Single-Agent Cobimetinib for Adults with Systemic Histiocytosis clinicaltrails.gov ID NCT02649972	This is a clinical trial of a new medication to treat patients with Erdheim-Chester disease whose ECD tissue (1) does NOT have the BRAF mutation or (2) whose tissue DOES have the BRAF mutation, but they do not have access to a BRAF inhibitor or they have taken a BRAF inhibitor and cannot tolerate the medication.	Any patient with ECD who is BRAF-negative or who is BRAF positive and cannot acquire or tolerate a BRAF inhibitor. The study requires monthly visits to MSKCC although after one year on the trial these can be every two months.	(212) 610-0243 email: diamone1@mskcc.org
	Additional Information about this study (5): Drug Administered: Cobimetinib Tests that may be ordered: Scans and laboratory tests Travel requirements: The study requires monthly visits to MSKCC for the first 6 months of the study, then every 8 weeks thereafter. Travel costs covered: No Length of time patient in study: 12 months or longer International Participants accepted: Yes Associated costs: Study drug is provided and the remainder of costs are billed to insurance.				
6.	Diamond, Eli Memorial Sloan-Kettering, New York City, NY USA	A Clinical, Structural, and Functional Neuroimaging Study of Cognition in Erdheim-Chester Disease clinicaltrails.gov ID NCT03127709	Patients with ECD often experience difficulty with thinking and memory and this can affect one's ability to work, function in daily life, and personal relationships. This is a study of ECD patients who do not have lesions in the brain to examine the effects of ECD on the brain. Participants will undergo a brain MRI and 45-60 minutes of cognitive testing. The goal of this study is to understand and characterize the ways that ECD affects the brain even without tumors.	Patients 18 years or older with ECD (1) without lesions in the brain and (2) who have not received intravenous chemotherapy	Eli L. Diamond, MD, 212-610-0243, diamone1@mskcc.org

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	Additional Information about this study (6): Tests that may be ordered: Participants will undergo a brain MRI and 45-60 minutes of cognitive testing. Length of time patient is in study: One year International participants accepted? Yes, fluency in English required. Number of participants included to date: 5 Number of remaining patients to be accepted: 25 Associated costs (Will patient be required to pay for tests? For associated hospital stays? For doctor visits?): None				
7.	Ronald Go and Gaurav Goyal	Defining Genomic Alterations and Discovering Druggable Targets in Histiocytic Neoplasms	This is a study at Mayo Clinic, Rochester that will enroll patients diagnosed with any histiocytic cancer, including Erdheim-Chester disease, and perform comprehensive gene testing on the biopsy tissue without any additional cost to the patient. Our goal is to find mutations in the cancer that can help understand the disease better and help with treatment using specific drugs.	Currently enrolling patients with all histiocytic cancers (Erdheim-Chester disease, Langerhans Cell histiocytosis, Rosai-Dorfman disease)	Ronald S. Go, (507) 284-2511, email: Go.Ronald@mayo.edu; or Gaurav Goyal, (507) 284-2511, email: Goyal.Gaurav@mayo.edu
8.	Thomas E. Witzig, MD, James E. Cerhan, MD, Ronald S. Go, MD and Gaurav Goyal, MBBS	Histiocytic neoplasms biobank and database-Lymphoma Specialized Programs of Research Excellence (SPORE) (09/2017-07/2022) and Lymphoma Epidemiology of Outcomes (LEO) Cohort Study (LEO)	This is an ongoing biobank and clinical database at Mayo Clinic and the University of Iowa, Rochester that will enroll patients diagnosed with any histiocytic cancer, including Erdheim-Chester disease, and collect and store tissue/blood samples for current and future studies. Our goal is to study these rare cancers in detail by means of various tests (genetic, inflammatory markers, other lab tests) that can improve our understanding and help identify new treatments.	Currently enrolling patients with all histiocytic cancers (Erdheim-Chester disease, Langerhans Cell histiocytosis, Rosai-Dorfman disease)	Ronald S. Go, (507) 284-2511, email: Go.Ronald@mayo.edu; or Gaurav Goyal, (507) 284-2511, email: Goyal.Gaurav@mayo.edu

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9.	Haroche, Julien Hôpital Pitié-Salpêtrière Paris, France	Long-term Outcome After Vemurafenib / BRAF Inhibitors Interruption in Erdheim-chester Disease (LOVE) clinicaltrails.gov ID NCT02089724	ECD patients with vemurafenib treatment seem to have a stable disease. This study evaluates the possibility of treatment interruption. Other BRAF inhibitors, such as dabrafenib, have recently been proposed for treating BRAF mutated histiocytoses. Other BRAF inhibitor interruption treatment should also be prospectively evaluated.	Patients and treating doctors are encouraged to contact Dr. Julien Haroche for more information.	+33 1 42 17 80 37 email: julien.haroche@psl.aphp.fr
10.	Jacobsen, Eric Dana-Farber Cancer Institute, Boston, MA, USA	A Study of Lenalidomide for Adult Histiocyte Disorders clinicaltrails.gov ID NCT02523040	This research study is a Phase II clinical trial studying a chemotherapy drug Lenalidomide as a possible treatment for one of three histiocyte disorders: Langerhans cell histiocytosis (LCH), Erdheim-Chester disease (ECD), or histiocytic sarcoma (HS).	Patients diagnosed with ECD 18 years and older.	617-632-6633, email: edjacobsen@partners.org
11.	Janku, Filip Sakamuri, Divya MD Anderson Cancer Center, Houston, TX, USA	A Safety, Tolerability and PK Study of DCC-2618 in Patients with Advanced Malignancies clinicaltrails.gov ID NCT02571036	This is a Phase 1 trial to investigate the safety and efficacy of the investigational drug, DCC-2618, administered orally (PO), in adult patients with advanced malignancies.	ECD patients 18 years.	(Sakamuri) 1-713-745-3296; dsakamuri@mdanderson.org (Janku) 713-563-1930; FJanku@mdanderson.org

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12.	Janku, Filip Holley, Veronica MD Anderson Cancer Center, Houston, TX, USA	Anakinra or Denosumab and Everolimus in Advanced Cancer clinicaltrails.gov ID NCT01624766	The goal of this Phase I clinical research study is to determine the tolerable dose of the combination of Afinitor (everolimus) either with Kineret (anakinra) or Xgeva (denosumab) for patients with advanced cancer. Everolimus is designed to stop cells from dividing. Anakinra is designated to block a protein that is involved in tumor development, new blood vessels growing, and spread of cancer. Denosumab is designed to block the activity of a protein, which may prevent bone complications in cancer that has spread to the bone.	ECD patients at least 18 years of age. Patients may be BRAF positive or negative.	(Holley) VRHolley@mdanderson.org (Janku) 713-563-1930; FJanku@mdanderson.org
13.	Janku, Filip Sakamuri, Divya MD Anderson Cancer Center, Houston, TX, USA	A Study of PLX8394 as a Single Agent in Patients with Advanced, Unresectable Solid Tumors clinicaltrails.gov ID NCT02428712	The goal of this clinical research study is to learn how PLX8394 may affect cancer cells with mutations in the gene BRAF. PLX8394 is designed to block mutations in BRAF. These mutations can cause cancer and cancer cell growth. By blocking these mutations, the drug may kill the cancer cells with the mutation and/or stop the tumor from growing.	ECD patients greater than 18 years of age.	(Sakamuri) 1-713-745-3296; dsakamuri@mdanderson.org (Janku) 713-563-1930; FJanku@mdanderson.org
14.	Janku, Filip Sakamuri, Divya MD Anderson Cancer Center, Houston, TX, USA	Phase I Dose-Escalation, Safety, Pharmacokinetic and Pharmacodynamic Study of BVD-523 in Patients with Advanced Malignancies clinicaltrails.gov ID NCT01781429	This study is a Phase I clinical trial to determine the recommended dose of BVD-523 for the treatment of BRAF and/or MEK positive disease.	CD BRAF or MEK positive patients ages 18 years or older. Patients may not have been previously treated with BRAF and/or MEK inhibitors.	(Sakamuri) 1-713-745-3296; dsakamuri@mdanderson.org (Janku) 713-563-1930; FJanku@mdanderson.org

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15.	Janku, Filip MD Anderson Cancer Center, Houston, TX, USA	Longitudinal monitoring of BRAF V600E mutation in urinary cell-free DNA of patients with metastatic cancers and Erdheim Chester Disease Study	An open-label, multi-center study is to assess the detection and monitoring of BRAF V600E mutational tumor load. The study is open to ECD patients with BRAF V600 mutation positive lesions.	ECD patients being treated at MD Anderson are encouraged to enroll by having their treating physician contact Dr. Janku.	713-563-2632 email: fjanku@mdanderson.org
16.	Janku, Filip MD Anderson Cancer Center, Houston, TX, USA	Retrospective Review of Patients With ECD Treated with Interferon-alpha or imatinib mesylate	Understand clinical characteristics, underlying biology, response to therapy and outcome of patients with Erdheim-Chester disease.	ECD patients being treated at MD Anderson are encouraged to enroll by having their treating physician contact Dr. Janku.	Phone: 713-563-2632
17.	Kurzrock, Razelle University of California, San Diego	Observational: Study of Personalized Cancer Therapy to Determine Response and Toxicity (UCSD_PREDICT) clinicaltrails.gov ID NCT02478931	PREDICT permits analysis of individualization of therapy to a patient's genomic results for ECD (and diverse cancers).	ECD patients of any age. Patients seen at University of California, San Diego Health System Diagnosed with a cancer or cancer-related condition	Lee Suzanna, MPH- (858) 534-1306 email: suzanna@ucsd.edu or Michaela Doering, BS (858) 822-5127, email: mdoering@ucsd.edu

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	Additional Information about this study (15): Tests that may be ordered: Molecular testing Travel requirements: Minimum of 1 visit; 1-2 week stay if genomics already done and results available Are travel costs covered? No Length of time patient is in study: Indefinitely or until the end of the research project. Patient can stop participation at any time. International participants accepted? Yes Associated costs Patient must pay for clinic/hospital visit(s) and test(s) ordered Number of participants included to date: 1,220 Number of remaining patients to be accepted: 780 Results of study to date: Reference: Schwaederle M, Parker BA, Schwab RB, Daniels GA, Piccioni DE, Kesari S, Helsten TL, Bazhenova LA, Romero J, Fanta PT, Lippman SM, Kurzrock R. Precision Oncology: The UC San Diego Moores Cancer Center PREDICT Experience. Mol Cancer Ther 2016; 15: 743-52.				
18.	O'Brien, Kevin J. National Institutes of Health, Bethesda, MD USA	Clinical and Basic Investigations into Erdheim-Chester Disease *The NIH will pay for all testing costs. Travel costs will be paid on a case-by-case basis. clinicaltrails.gov ID NCT01417520	Natural history study designed to understand the disease, identify its cause, and classify it according to presentation and progression. The study does not include any modifications to patient treatment plans although follow on studies may address this.	ECD Patients are encouraged to enroll for one week of testing at the NIH. ECD patients are also encouraged to contact Dr. Estrada-Veras prior to any medical procedure to determine if, and how, any tissue samples might be sent to the NIH as a result of the procedure.	Kevin J O'Brien, C.R.N.P. (301) 435-2824 ko85t@nih.gov