

THE TEXAS CHILDREN'S HOSPITAL HISTIOCYTOSIS PROGRAM AND ERDHEIM-CHESTER DISEASE

Clinical Program

The Texas Children's Cancer Center Histiocytosis Program is the largest Histiocytosis Clinic in the world. The clinic is associated with Baylor College of Medicine, one of the top medical schools in the country, and Texas Children's Hospital, the largest pediatric hospital in the United States.

Texas Children's Cancer Center has the unique capability to provide state of the art treatment for children with Histiocytic Disorders including Langerhans Cell Histiocytosis, Hemophagocytic Lymphohistiocytosis, Juvenile Xanthogranuloma, and Rosai-Dorfman Disease. Dr. Ken McClain and a team of internal medicine physicians are also available to evaluate **adults** with histiocytic diseases including **Erdheim-Chester Disease** and offer advice for appropriate treatment plans.

The Histiocytosis Team is a multidisciplinary group including physicians, nurse practitioners, nurses, social workers, and research coordinators with specific focus on children and adults with Histiocytic Disorders.

The purpose of the Histiocytosis Program at the Texas Children's Cancer Center is to provide the best care available today for children and adults, and ultimately to find cures. At the Texas Children's Cancer Center, histiocytic disorders are not rare. The Histiocytosis Clinic is the largest in the world, and the dramatic increase in the number of patients seen here, 450 new patients since 2006, now provides an opportunity for our program to develop and expand clinical trials of innovative therapies and make significant contributions to research of LCH, HLH, JXG, RDD, and **ECD**. The clinical experience we have developed, coupled with the basic research studies we design with rare biopsy and other tissues samples, provide a unique opportunity for us to establish clinical and biologic correlations that no other center in the world can. We built a program to discover the causes of these conditions so that we may expedite development of more effective and innovative therapies.

Research Program

Comprehensive Analysis of Cell-Specific Gene Expression in LCH Tumors:

This project is the core of our research program. It is based on the idea that we cannot improve treatment of patients with LCH until we understand the fundamental nature of the cells that comprise histiocytic lesions. We have developed a method to process, store, and analyze viable cells from histiocytic lesions/tumors, including **ECD**. From very few cells, we can isolate RNA, then amplify genetic material to determine the genetic signature of **ECD**. We are comparing the signatures of LCH, JXG, RDD and **ECD** to circulating dendritic cell populations from patients with histiocytic diseases as well as healthy patients to determine the crucial genes and pathways that cause these disorders and also to identify the cells of origin of the different conditions. These experiments will inform future clinical therapeutic trials.

Molecular Epidemiology:

While direct evidence for inheritance of most histiocytic diseases remains uncertain, genetic background may confer risks for developing the disease. We are working with the Cancer Epidemiology team at TXCH as well as University of Texas to analyze the genomes of parents and patients with histiocytic disorders, including **ECD**, to determine genes that may confer increased risk of developing histiocytic diseases. These studies will also be helpful to determine genes that are important in pathogenesis of the disease.

Clinical Trials:

The ultimate goal of all of our research is to find cures for all histiocytic diseases. In addition to our translational research efforts, we have built a team dedicated to design and oversight of clinical trials including local, multi-center, and cooperative group studies. Please contact us to learn what clinical trial opportunities are currently available.

What is needed for this research?

We collect biological samples from clinically-indicated procedures. Any of the following would be helpful for our research:

- **Surgical biopsies** The current research is focusing on an effort to isolate pure populations of cells fresh or frozen biopsy specimens to discover which specific genes are causing ECD.
Biopsy samples may be collected only from clinically-indicated surgical procedures using excess tissue not needed by your physicians.
- **Blood samples** White blood cells can be isolated and analyzed in order to determine which circulating cells and genes contribute to Histiocytic Diseases. The plasma fluid in blood can also be analyzed to determine which proteins are important in diagnosing and treating Histiocytic Diseases.
Blood samples (approximately 2 teaspoons) may be collected at a time when you are undergoing a clinically-indicated blood draw procedure.
- **Cerebrospinal fluid** CSF is the fluid that surrounds the brain and spinal cord. The Histiocytosis Team studying proteins that are involved in Histiocytosis-related nerve problems.
CSF may be collected from clinically-indicated lumbar puncture procedures from excess fluid not needed by your physicians.
- **Medical information** Medical details associated with the biology samples will help determine the clinical significance the genes, proteins and cells identified in the Histiocytosis research studies.

How can I participate in Histiocytosis Research?

If you are interested in participating in Histiocytosis Research, you are invited to contact the TXCH Histiocytosis Team so they can send you information and a consent form for his study that is approved by the Institutional Review Board for Baylor College of Medicine and Affiliated Institutions. Dr. McClain or his staff will then discuss the consent with you over the phone. If you are interested in participating, you can then sign the consent and return it to Dr. McClain by FAX, mail, e-mail or Fed-Ex. The Histiocytosis Team can provide you with a prepaid FEDEX airbill for the consent. Once all your questions are answered and you have signed consent, the Histiocytosis Team can discuss specific instructions for collection and shipping of your samples with you and your doctors. Please **contact us** if you are interested or if you and/or your physicians have other questions about Histiocytosis Research.

Carl Allen MD, PhD
832-824-4312

Kenneth McClain MD, PhD
832-822-4208

<http://www.txch.org/histiolab/>