What is Langerhans cell histiocytosis?

Langerhans cell histiocytosis (LCH) occurs in patients when the body accumulates too many immature Langerhans cells, a subset of the larger family of cells known as histiocytes. A Langerhans cell is a type of white blood cell that normally helps the body fight infection. In LCH, for some unknown reason, too many Langerhans cells are produced and then build up in certain parts of the body where they can form tumors or damage organs.

LCH is caused by somatic mutations in genes that control how histiocytes, or LCH cells, grow. Cells from LCH tumors (lesions) are found to have cancer-causing DNA changes (mutations); these include mutations of the BRAF, MAP2K1, and ARAF genes. These mutations may cause LCH cells to grow inappropriately in areas where they are not usually present, and damage tissues or form lesions (tumors). Previously thought to be an auto-immune disorder, LCH was classified as a blood cancer in 2008 by the World Health Organization.

LCH is believed to occur in fewer than 1 in 200,000 children, but any age group can be affected. It occurs most often between the ages of 1- 3 years. It is, however, believed to be under-diagnosed. Some patients may have no symptoms at all, while others have symptoms that are mistaken for injury or other conditions/diseases. The damage caused by LCH may affect a single area; however, it's possible that other areas can be involved such as skin, bone, lymph nodes, liver, lung, spleen, brain, pituitary and bone marrow.

Not all patients have all areas of involvement. LCH was first described in medical literature in the mid to late 1800's. Through the years it has been known by various names, such as histiocytosis-X, eosinophilic granuloma, Letterer-Siwe disease, Hashimoto-Pritzger disease and Hand-Schuller-Christian syndrome. In 1973, the name Langerhans cell histiocytosis was introduced. This name was agreed upon to recognize the central role of the Langerhans cell.

Will LCH patients recover?

Most patients with LCH will survive this disease. LCH in the skin, bones, lymph nodes or pituitary gland usually get better with treatment. Some patients have involvement in the spleen, liver, bone marrow and skeleton which may be more difficult to treat.

Some patients may develop a condition called diabetes insipidus, more recently known as Arginine Vasopressin-Deficiency (AVP-D). This condition is characterized by excessive thirst and urination.

Symptoms of LCH

General symptoms like fever, weakness and failure to gain weight may be present. All patients do not have all of the below involvement. It is difficult, if not impossible, for a physician to say with certainty how each patient will respond to therapy. Possible site involvement include:

- Skin (rash)
- Bone (single or multiple lesions)
- Lung, liver, spleen (dysfunction)
- Teeth and gums (loose/lost teeth, swollen gums)
- Ear (chronic infections or discharge)
- Central nervous system issues (problems balancing,depression, learning issues)
- Pituitary gland - causing diabetes insipidus *(characterized by excessive thirst and urination)*

How is LCH treated?

A diagnosis of LCH is usually made following a biopsy and microscopic examination of the affected tissue. To determine the extent of the disease and subsequent treatment plan, several other tests may be done. These can include blood work, ultrasound, CT and MRI scans.

Treatment of LCH depends upon the individual patient and is planned after thorough testing to determine the extent of the disease. It may include no treatment with the lesion being watched for signs of remission or minimal types of treatment with surgery or low-dose chemotherapy. In patients with more extensive disease, such as when multiple organ systems are involved, systemic chemotherapy is most often recommended. Targeted therapies can be used for severe LCH or LCH that does not respond to chemotherapy.

Hematologists/Oncologists treat children with Langerhans cell histiocytosis.

To learn more about the Association and its Scientific Initiatives, Outreach Efforts and Research Program visit www.histio.org.

HISTIO

The Histiocytosis Association is dedicated to raising awareness about histiocytic disorders, providing educational and emotional support, and funding research leading to better treatments and a cure.

A world free of histiocytic disorders.

www.histio.org

About the Association

A Rare Community

The Histiocytosis Association is a global nonprofit organization dedicated to addressing the unique needs of patients and families dealing with the effects of histiocytic disorders while leading the search for a cure. It is the only organization of its kind, connecting the patient and medical communities to:

- Grow and share knowledge of histiocytic disorders
- Provide critical emotional and educational support to patients and families
- Identify and fund key research initiatives that will lead to a world free of histiocytic disorders

Partnership for a Cure

The Histiocytosis Association works closely with the Histiocyte Society, an international organization of over 240 physicians and researchers, which is dedicated to studying the histiocytic disorders. Through this partnership, understanding of this disease has greatly increased, and survival rates and quality of life continue to improve.

Community Outreach

While the search for more effective treatments and a cure continues, the Histiocytosis Association is dedicated to supporting and empowering the patients and families who live with these diseases every day.

Funding the Association

Histiocytic disorders are considered "orphan diseases." An orphan disease is one that affects less than 200,000 individuals in the United States.* Subsequently, these disorders do not receive a high priority for government-funded research. The Association relies on contributions from corporations, foundations and individual donors to fund critical research, build awareness and conduct community outreach initiatives.

**Rare Disease Act of 2002*

You are not Alone

While the search for more effective treatments, and ultimately a cure continues, the Histiocytosis Association is dedicated to informing and empowering those who live with histiocytic diseases every day.

Whether newly diagnosed and learning to navigate this rare diagnosis or searching for strength and support while caring for a loved one, you can turn to the Histiocytosis Association to connect you with a community who understands what you are going through.

The Histiocytosis Association invites you to become a part of our rare community. We encourage you to explore our online resources by visiting our website:

www.histio.org

Making a Donation

All donations are tax deductible (Federal Tax ID # 22-2827069). Visit www.histio.org/donate to join us in the pursuit of a cure.

HISTIOCYTOSIS ASSOCIATION
A Rare Community

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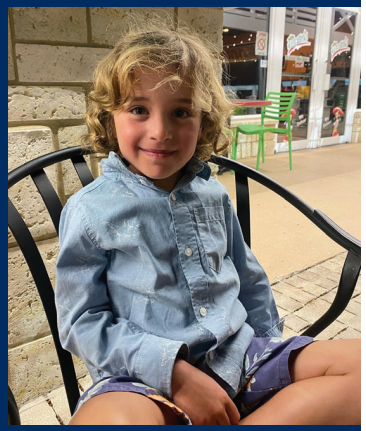
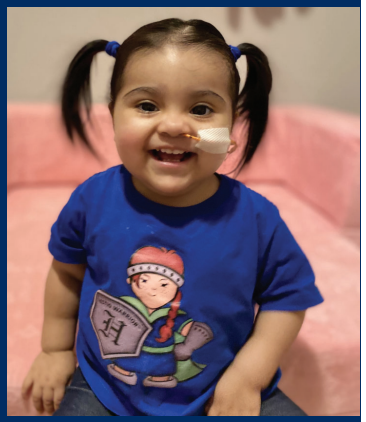
Published by the Histiocytosis Association
in partnership with the Histiocyte Society

revised 05/2023





LANGERHANS CELL HISTIOCYTOSIS IN CHILDREN

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