Hemophagocytic Lymphohistiocytosis (HLH)

Cincinnati Children's
changing the outcome together

20+

Number of years Cincinnati Children's has been conducting research in the field of HLH

200+

Number of bone marrow transplants for HLH patients since the program began in 1981

BEST CHILDREN'S HOSPITALS
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HONOR ROLL
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Cincinnati Children's is ranked #3 in the nation among Honor Roll hospitals.

HLH is a rare disease, occurring in about one in 1 million children. If not diagnosed early enough, it often leads to death. Cincinnati Children's is home to internationally recognized experts in HLH who are dedicated to bringing about earlier, accurate diagnosis and treatment of this devastating disease.

HOW WE'RE DIFFERENT

The HLH Center of Excellence at Cincinnati Children's brings together the most respected HLH physicians: specialists in immune deficiency and dysregulation disorders,cancer and blood diseases, rheumatologic disorders and bone marrow transplant. Their shared knowledge in the clinical setting results in the most effective treatments for patients. And their collaboration on research studies means novel treatment approaches are on the horizon.

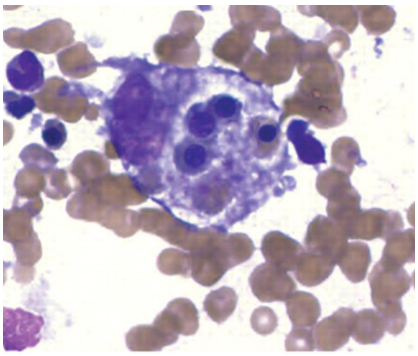
Our care team has treated children and teens with HLH for decades and sees patients from all over the world. We provide a single resource for comprehensive care of HLH patients, with these features as focal points of our program:

- The HLH clinical care team at Cincinnati Children's is led by Michael Jordan, MD, Rebecca Marsh, MD, and Ashish Kumar, MD, PhD, some of the world's leading authorities on HLH.
- The Diagnostic Immunology Lab (DIL) and Molecular Genetics Division provide diagnoses for all known genetic forms of HLH.
- We have additional studies in development to unravel the complex immunology of active HLH disease in order to identify targeted biologic agents with fewer side effects.
- Cincinnati Children's was the lead U.S. site for an international clinical trial of a 'breakthrough therapy' that led to the first-ever drug approval (Emapalumab) for HLH by the FDA.

SYMPTOMS AND DIAGNOSIS

HLH has both inherited and acquired forms. Primary/familial HLH is often caused by an inherited problem of the immune system. Secondary HLH can be related to a number of underlying conditions including infections, malignancy and autoimmune diseases.

One of the primary goals of the HLH Center of Excellence at Cincinnati Children's is to provide families with an accurate diagnosis. Often HLH is undiagnosed or misdiagnosed because it is so rare and has such varying symptoms.



In hemophagocytic lymphohistiocytosis (HLH), certain immune cells engulf other cell types, a condition called "hemophagocytosis" or "blood-eating," which damages the bone marrow and other organs. This image is from the bone marrow of a patient affected by HLH.

HLH almost always presents with fevers that don't go away. It can be triggered by common infections. Symptoms may include:

- Persistent fevers
- Rash
- Enlarged liver
- Enlarged spleen
- Anemia
- Low platelets
- Low white blood cells
- Jaundice
- Hepatitis
- Liver failure
- Respiratory issues (coughing, respiratory distress)
- Seizures
- Altered mental status

HLH can only be diagnosed with the proper blood tests, which we perform on site in our clinical labs. Genetic testing, also performed here at Cincinnati Children's, is critical for confirming a clinical diagnosis, predicting the risk of recurrence in affected patients, and defining the likelihood of HLH to develop in family members not yet showing symptoms of the disease.

TREATMENT APPROACH

Correct treatment is critical for patients with HLH, as the condition is life threatening. Our first course of treatment for HLH aims at immunosuppression to induce remission. The choice of treatment depends on how sick a patient is and the underlying cause of HLH. Treatments may include:

- Steroids
- Antibody therapy that targets interferon gamma (Emapalumab)
- Chemotherapy (etoposide / VP-16)
- Antibody therapy that destroys T cells (Alemtuzumab or ATG)
- Anti-cytokine therapies (such as JAK inhibitors)
- Other medications that suppress the immune system
- Medications that help treat any infections that are present or prevent new infections from occurring

Patients need to be evaluated for a variety of infections, cancers and rheumatologic diseases, which may need treatment. Some patients should be evaluated for genetic causes of HLH and other inborn errors of immunity.

Typically, patients with primary/familial HLH must also have their immune systems replaced with a bone marrow transplant.

The Bone Marrow Transplantation Program within the Division of Bone Marrow Transplantation and Immune Deficiency at Cincinnati Children's is an international leader, having performed more than 200 transplants for HLH patients throughout the program's history.

For urgent issues, or to speak with the specialist on call 24/7, call the Physician Priority Link® at 1-888-636-7997.

TREATMENT TEAM

Bone Marrow Transplant and Immune Deficiency

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