IPC & IPH
REGISTRY

UNC HEALTH®
Children's

BE THE CATALYST FOR A BETTER WORLD

BY CREATING AN IPH REGISTRY

IPH MUST BE STUDIED IN ORDER TO IMPROVE OUTCOMES

LANDON'S STORY

Landon's journey began in April 2023 when he developed a seemingly typical cold. However, his symptoms quickly escalated, leading to severe anemia and multiple hospital visits. Despite initial treatments, including blood transfusions and supplements, his condition worsened by May, causing extreme fatigue and paleness. By August, after an episode of vomiting blood and difficulty breathing, Landon was admitted to the ICU. A lung biopsy revealed bleeding in his lungs, leading to a diagnosis of Idiopathic Pulmonary Hemosiderosis (IPH), a rare lung disease, at Hasbro Children's Hospital. With no clear treatment path, Landon's family sought expertise at Boston Children's.

Luckily, at Boston Children's, Dr. Alicia Casey took on Landon's complex case, confirming the IPH diagnosis and starting a new treatment regimen. Despite the ongoing struggle with his condition, including frequent hospital visits due to complications like RSV and COVID-19, Landon has shown remarkable resilience. Monthly treatments, including steroid pulses and IVIG infusions, have stabilized his condition, allowing him to return to school with special precautions.

Thankfully, Landon was a lucky one who received treatment. **Not all children are as lucky.**

Landon wouldn't be feeling so much better today without the support of the community who backed him and his family. We need to extend this supportive community across the country through a registry so that many more children can have the same results.

IPH BACKGROUND AND CHALLENGES

IPH is a serious and often life-threatening condition that mostly affects children. The different ways the disease progresses and how patients respond to treatment make it difficult for doctors to decide the best course of action. We need to better understand the disease. By raising awareness, this project aims to improve time to diagnosis, reduce time to treatment, find the best treatments, understand current outcomes and how they can be improved, and create a community for affected families. A better understanding of the disease may also reduce side effects of current treatments, such as growth suppression and weakened immune systems.

THE REGISTRY

This patient registry for IPH will serve as a critical tool in addressing treatment challenges by systematically collecting and organizing detailed patient data. The registry will enable the aggregation of clinical data, treatment outcomes, and patient demographics on a scale previously unattainable. This comprehensive dataset will provide invaluable insights into the natural history of IPH, patterns of disease progression, and long-term outcomes of various treatment strategies.

ADVANCING TREATMENT

By compiling and analyzing this data, researchers can identify key prognostic indicators and potential biomarkers that predict disease course and treatment response. This knowledge is essential for the development of more targeted therapies, reducing reliance on high-dose steroids, and possibly mitigating their adverse effects. Additionally, the registry can facilitate the identification of patient subgroups who might benefit from novel therapeutic approaches, thereby fostering personalized medicine in the treatment of IPH.

IMPROVING PATIENT OUTCOMES

For clinicians, access to registry data will support evidence-based decision-making, enabling them to tailor treatment plans more precisely to the individual needs of their patients. This personalized approach not only aims to enhance the efficacy of treatments but also to reduce the time to diagnosis and treatment, which will improve patient outcomes. In the long run, the insights gained from the registry will contribute to establishing new standards of care that improve the prognosis and quality of life for children afflicted by IPH.

The establishment of an IPH patient registry is an urgent priority that promises to revolutionize our understanding and management of this challenging condition. It will pave the way for more effective treatments and quicker diagnosis, offering hope to affected children and their families.

THE GOAL

$650,000 is needed to register 30 patients with five years of follow-up.

Breakdown:

$5,000 per patient with five years follow-up. This includes data entry for initial analysis and each follow-up, collection and transport of lung tissue, lung biopsies, and blood sample collection.

$100,000 per year to maintain the Registry, including regulatory fees, a coordinator who can enroll patients from our site but also serve as a point person for other sites, database manager, statistician who can help analyze data from the Registry, and investigator effort to lead the Registry.

Total: $650,000

THANK YOU

We hope to partner with you to better serve our communities and provide resources that children and families could not have had otherwise, standing together to foster healthy lifestyles and improve the lives of children across the United States and beyond.

Should these opportunities not match your desired intent, we are happy to offer alternative partnership opportunities.

For questions, please reach out to:
Cameron Lafferty
Assistant Director of Development
UNC Health Foundation, Children's
cameron_lafferty@med.unc.edu |
919.966.7816