

#### What is LLS PedAL?

The Leukemia & Lymphoma Society (LLS) is leading an unprecedented effort to fundamentally change how children with pediatric acute leukemia (acute myeloid leukemia and other high-risk leukemias) are treated. LLS PedAL is a global precision medicine master clinical trial that will test multiple targeted therapies simultaneously at up to 200 clinical sites worldwide. We are setting the groundwork for LLS PedAL throughout 2019, with the goal of treating the first patient in the summer of 2020.

LLS PedAL is a key component of **The LLS Children's Initiative: Cures and Care for Children**. We are taking on children's cancer from every direction, disrupting the status quo with our bold vision for young patients — to not only survive their cancer, but thrive in their lives after treatment. Through The LLS Children's Initiative we are:

- More than doubling our investment in pediatric cancer research grants
- Expanding education and support services for children and families
- · Advocating for and driving policies to accelerate the development of new treatments and break down barriers to care
- · Launching LLS PedAL, a groundbreaking precision medicine clinical trial in acute pediatric leukemia

### Why LLS PedAL?

We urgently need to change the course of treatment for children with acute leukemia. Many children with acute myeloid leukemia (AML) will relapse from their treatment and most of these will not survive more than five years. What's more, many of those who do survive experience long-term complications. The survival rate for acute lymphoblastic leukemia (ALL), the most common type of pediatric blood cancer, is higher, but many of these children also suffer long-term effects from the harsh and outdated treatments. Over the past four decades, only four cancer treatments have been approved for first use in children. While we're finally seeing progress in AML for adults, the biological drivers of the disease are different in children and new approaches are needed.

## Why LLS?

LLS is a patient-focused nonprofit organization with a proven track record in precision medicine. Our Beat AML® Master Clinical Trial, launched in 2016, has shown we are uniquely positioned to convene the right team of researchers and physicians, regulatory agencies, pharmaceutical and biotech partners, and patient groups to bring the promise of precision medicine to patients.

#### How will LLS PedAL work?

LLS PedAL is a strategic initiative to overcome the obstacles to progress in treating children with acute leukemia.

LLS has brought together leaders in childhood leukemia to build the foundation for a collaborative master clinical trial for children with relapsed acute leukemia. The initiative is paving the way to:

- Centralize management of the trial to drive efficiency and leverage LLS's unique relationship with the U.S. Food and Drug Administration (FDA) and other stakeholders
- Establish a process to capture genetic sequencing and other biological markers for every child with high-risk acute leukemia to identify patterns and drivers of the cancer so patients can be matched with the most promising targeted therapies for their disease
- Identify and validate the right targets the underlying drivers of disease so the right therapies can be tested
- Consolidate pediatric cancer data from multiple institutions into a single data set, establish a common language to define and analyze the data, and make that data available to researchers worldwide
- Form a global collaborative to standardize and unite efforts in pediatric leukemia drug development, and create a
  more efficient means to secure early pharmaceutical company commitments to provide targeted drugs



# Who's Who of PedAL Leadership Team

The PedAL Master Clinical Trial is being conceived, developed and implemented under the direction of preeminent leaders in pediatric acute leukemia.



**Gwen Nichols, MD**The Leukemia & Lymphoma Society

Co-Chair, PedAL Initiative



**Kimberly Stegmaier, MD**Dana-Farber Cancer Institute

Preclinical Discovery Lead



**E. Anders Kolb, MD**Nemours Center for Cancer and Blood Disorders
Co-Chair, PedAL Initiative



**Samuel L. Volchenboum, MD, PhD** *University of Chicago* Bioinformatics Lead



**Soheil Meshinchi, MD, PhD**Fred Hutchinson Cancer
Research Center
Target and Biomarker Lead



**Laura Di Laurenzio, PhD** *The Leukemia & Lymphoma Society*Project Leader



**Todd Cooper, DO** Seattle Children's Hospital Clinical Trial Lead



**Julie Guillot**AML Parent and Partnership
Outreach Chair



**Todd Alonzo, PhD** *Children's Oncology Group*Group Statistician



