

## **No More Kids with Cancer Awards \$220,000 to Children's Hospital of Philadelphia**

### **The Phase I clinical trial investigates using a child's own immune system - CAR T-cells – to treat acute myeloid leukemia (AML)**

*Leverages breakthroughs in CAR T-cell therapy to find a less toxic and more effective treatments for children with cancer*

PHILADELPHIA — September 24, 2018 — [No More Kids with Cancer](#) is pleased to announce a \$220,000 award to Children's Hospital of Philadelphia (CHOP). The \$220,000 award will be directed to a Phase I clinical trial that will investigate the effectiveness of immune system cells called CAR T cells – to treat relapsed or refractory acute myeloid leukemia (AML) in children and young adults. The trial will test the safety and potential efficacy of a new CAR T-cell immunotherapy in children and young adults with AML - the second most common form of leukemia in children.

Based at the Philadelphia campus, the trial will be led by Dr. Richard Aplenc, MD, PhD, MSCE, a physician-scientist and Assistant Vice President and Chief Clinical Research Officer at the Children's Hospital of Philadelphia Research Institute, and Professor of Pediatrics at the University of Pennsylvania School of Medicine. He will be working with Dr. Sarah Tasian at CHOP as well as Dr. Nirali Shah at the Pediatric Oncology Branch in the Center for Cancer Research at the National Institutes for Health (NIH) and Dr. Terry Fry at the University of Colorado, Denver.

There have been successes in using CAR T-cell therapies for children with other types of leukemia and preclinical studies have demonstrated that CAR T-cell therapies could be used in AML treatments. During the Phase I clinical trial, the team will test appropriate dosage levels to be effective, determine the percentage of children who achieve remission, and identify side effects of the treatment. This trial is modeled upon current, successful Phase 1 trials for children with leukemia ongoing at CHOP and the National Institutes of Health.

"AML is the second most common form of leukemia in children, and there has been very little research into AML treatments specifically around relapsed/refractory AML," said Dr. Richard Aplenc. "New strategies for treatment are needed in order to treat relapsed and refractory AML in a safer, more effective way. With advances in research, science and technology, we are aiming to find treatments that will give children not only a cancer-free future, but a future without lasting effects from debilitating treatments."

"Children with cancer deserve better treatments than drugs developed 50 years ago," said Amy Summy, co-Founder and President of No More Kids with Cancer. "AML is one of the most challenging childhood cancers to treat. This innovative trial brings the latest advances in cancer research to kids. We want to thank CHOP and the NIH team for their continued research

innovation and dedication to children with cancer. We would also like to thank our donors for making this grant possible and believing in the power of research to cure kids. We hope this trial moves the ball forward for children and their families battling AML.”

### **CAR T cell Treatments and Acute Myeloid Leukemia**

Leukemia is the most common cancer in children and teens. There has been success in using CAR T cell therapies for children with other types of leukemia and preclinical studies have demonstrated that CAR T cell therapies could be used in AML treatments.

In this Phase I clinical trial, the team will be testing CAR T cell treatment. In this type of treatment, T cells are removed from a patient’s blood and then genetically modified to enable the cells to identify and specifically attack cancer cells.

This type of treatment is currently seeing success in adult AML clinical trials demonstrated by a clinical trial at the Moffitt Cancer Center and Research Institute. A new case study found that an acute myeloid leukemia adult patient has remained cancer free for 9 months following treatment with CAR T cells, CYAD-01, and a bone marrow transplant. The patient did not receive chemotherapy.

### **About Childhood Cancer and AML**

Cancer takes the lives of more children than any other disease in the United States. Childhood cancer research is severely underfunded, resulting in few new treatment options and drugs for children. Today, most children receive highly-toxic treatments developed as far back as the 1950s, and most survivors live with debilitating, life-threatening side effects.

AML is the second most common form of leukemia in children and is one of the most challenging childhood cancers to treat since it is a cancer of the blood and bone marrow.

AML is currently treated with intensive chemotherapy or Allogeneic donor stem cell transplantation. Only 60% of children with AML will remain in long-term remission even with these intensive therapies. While there has been decades of research into treatment for the most common form of childhood leukemia, there has been much less research into AML treatments and very little focus on relapsed/refractory AML. New strategies for treatment are needed to treat relapsed and refractory AML (cases where children reach remission but then have a return of leukemia cells in the body or cases where there are still residual traces of leukemia in the body after extensive treatment).

### **Make a Difference**

Childhood cancer research is severely underfunded. Please help No More Kids with Cancer fund more groundbreaking childhood cancer research. Make a [donation](#) today.

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### **About No More Kids with Cancer**

[No More Kids with Cancer](#) is a nonprofit research accelerator. We serve children with cancer — and the doctors and researchers working to save them — who urgently need alternatives to the 50s-era, inhumane, side-effect-laden standard treatments used today. We are a national organization focused exclusively on accelerating discovery of non-toxic, effective, modern treatments for children with cancer.

No More Kids with Cancer backs groundbreaking translational research — such as gene sequencing, pre-clinical and clinical trials — that leverages the latest understanding in cancer biology and advancements in technology to bring non-toxic, and more effective treatments to children. Collaborating with leading scientists — who share our belief that more research leads to more options — we're working toward our vision of No More Kids with Cancer. Inquiries from media and organizations interested in collaborating with No More Kids with Cancer should contact

**Inquiries from media and organizations interested in collaborating with No More Kids with Cancer should contact:**

Amy Summy  
+1.610.742.4500  
[amy@normorekidswithcancer.org](mailto:amy@normorekidswithcancer.org)

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