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For Immediate Release

## **Alliance for Cancer Gene Therapy Announces ACGT Wendy Walk Grant Recipient for Sarcoma**

*Crystal Mackall, MD, Stanford University, Receives ACGT's 50th research grant and 16th Clinical Translation Grant*

**STAMFORD**, Conn., April 15, 2016 -- **Alliance for Cancer Gene Therapy (ACGT)**, the nation's only nonprofit dedicated exclusively to cell and gene therapies for cancer, and **Wendy Walk**, a nonprofit with the aim to raise funds and awareness for sarcoma, a rare form of cancer, announced today a new Clinical Translation Grant recipient for its **Wendy Walk ACGT Sarcoma Grant**. **Dr. Crystal Mackall**, professor of Pediatrics and Internal Medicine at Stanford University, associate director of the Stanford Cancer Institute, and leader of the Cancer and Immunology and Immunotherapy Program, will receive a \$500,000 Clinical Translation Grant for her work studying osteosarcoma.

Osteosarcoma is the most common bone tumor cancer that represents about five percent of all pediatric tumors. The five year survival rate is approximately 50 percent, so there is clearly a need for improved therapeutics for this disease. Sarcoma in general is a rare cancer of the connective tissues, such as nerves, muscles, and bones. Sarcomas can arise anywhere in the body and are frequently hidden deep in the limbs. They are often misdiagnosed and assumed to be sports injuries or benign bumps, especially in teens.

Dr. Mackall's research is designed to improve upon earlier clinical studies in osteosarcoma, other sarcomas and neuroblastomas that express the tumor antigen GD2 (a mutant antibody) using engineered CAR T cells. The hypothesis of this proposal is that it will be possible to improve outcomes by (1) increasing the persistence of CAR-T cells and (2) differentiation of suppressor cells. If successful, this proposed trial could significantly improve outcomes for patients with this malignancy. The purpose of the grant is to fund original research that could translate into a clinical trial and possible pharmacological and toxicological studies using gene and immunotherapy.

ACGT has a 15 year history of funding innovative cancer research studies and this year marked several major accomplishments including surpassing \$26.4 million in funding these types of basic and clinical translation cell and gene therapy studies. Dr. Mackall is ACGT's 50th grant recipient and will be the 16th clinical translation grantee. This is particularly exciting given ACGT's commitment to contributing 100 percent of donations directly to research. Additional breakthroughs carried out by ACGT grantees, like Dr. Carl June at the University of Pennsylvania and his work [successfully treating leukemia](#) through cancer gene therapy, have been touted in recent national documentaries aired on [PBS](#) and [HBO](#) and are showing a 90 percent remission rate of children and adults with acute lymphoblastic leukemia that have gone into remission after participating in a personalized cellular therapy trial. ACGT-funded work is also attracting increasing attention from

the pharmaceutical industry, which is swiftly discovering the potential of cell and gene therapies.

Wendy Walk, which co-funds the ACGT Sarcoma Clinical Translation Grant, was founded in 2010 by Ali, Matt, and Jackie Landes to raise funds and awareness for all sarcomas, which are rare forms of cancer that their mother, Wendy, was diagnosed with. Despite her tragic passing in March of 2013 from liposarcoma, Wendy's legacy has been kept alive through the organization and continues to be an inspiration to others battling the disease. With a profoundly personal understanding of how cancer impacts the entire family system, Wendy Walk is committed to helping families cope with the unique challenges sarcoma brings. Wendy Walk works to provide courage, strength and hope to families and patients battling sarcoma through charitable social and athletic events held all over the country. The organization's most notable events are walks which take place each spring in New York City, Miami, Los Angeles, and Park City. Through the walks, supporters have contributed over \$2.25 million. The funds raised allows the Wendy Walk to continually fulfill its mission to find and develop new innovative treatments for sarcoma and serve as a valuable resource for those impacted by this rare disease.

"Wendy was very focused on finding research that would lead to a cure in real time," noted Ali Landes, Wendy's daughter and executive director of Wendy Walk. "She was always searching and investing in research that was outside of the box. She would be really happy that Wendy Walk is partnering with ACGT and matching funds to help accelerate the treatment options for sarcoma."

"I am honored that our work has been awarded the 2016 Wendy Walk ACGT Investigator's Award in Clinical Translation of Cell and Gene Therapy for Sarcoma," said Dr. Mackall. "There has been much recent success in cancer immunotherapy and we are hopeful that this work can bring similar success to sarcoma patients. The funds will be used to support a cutting edge clinical trial of engineered T cells for osteosarcoma. It builds upon previous clinical trials and experimental studies to provide what we believe to be a state-of-the-art immunotherapy for osteosarcoma. Thanks to the Alliance for Cancer Gene Therapy and the Wendy Walk Foundation for working together to improve outcomes for patients suffering from sarcoma."

"Through the rigor of its scientific selection process, ACGT has funded many of the leading cancer gene therapy and immunotherapy researchers in the United States these past 15 years," said John Walter, CEO and president of ACGT. "For example, ACGT's success include studies it funded in treating leukemia, melanoma and lung cancer. I'm a believer in the opportunity cancer gene therapy presents to help combat this dreadful disease and I'm thrilled to partner with Wendy Walk to bring this research to sarcoma."

Dr. Mackall holds a BS in Natural Sciences from the University of Akron, Akron, Ohio, and received her MD from Northeastern Ohio University College of Medicine. She completed her residency in internal medicine/pediatrics at Akron General Medical Center/Children's Hospital of Akron, her fellowship in pediatric hematology/oncology at the Pediatric Oncology Branch, National Cancer Institute in Bethesda, Maryland, and her postdoctoral fellowship in immunology at the National Cancer Institute.

#### **About Alliance for Cancer Gene Therapy (ACGT)**

Established in 2001, ACGT is the nation's only non-profit dedicated exclusively to cell and gene therapy treatments for all types of cancer. One hundred percent of contributions go directly to research. ACGT has funded 50 grants in the U.S. and Canada since its founding in 2001 by Barbara Netter and her late husband Edward, to conduct and accelerate critically needed innovative research. Since its inception, ACGT has awarded 34 grants to Young Investigators and 16 grants to Clinical Investigators, totaling more than \$26 million in funding. ACGT is located at 96 Cummings Point Road, Stamford, Connecticut 06902; 203-358-5055. To learn more, visit [acgtfoundation.org](http://acgtfoundation.org) or join the ACGT community on Facebook, Twitter and YouTube at @acgtfoundation.

**About Wendy Walk**

Since the first walk in 2010, [Wendy Walk](#) has raised more than \$2.25 million for sarcoma research. Donations are used to fund a unique and exciting international cooperative research studies. For more information, visit [wendywalk.org](http://wendywalk.org).

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