$1.9 Million NIH Grant Supports Research in the Most Common Soft Tissue Tumor in Children

(COLUMBUS, Ohio) — A nearly $2 million grant from the National Institutes of Health (NIH) will help investigators at Nationwide Children’s Hospital search for biomarkers that may be linked to the development and outcome of hemangiomas, the most common soft tissue tumor in children. Nationwide Children’s is home to the only Hemangioma and Vascular Malformations Clinic in the United States with an NIH-sponsored clinical study.

A hemangioma is an abnormal buildup of blood vessels in the skin or internal organs. Hemangiomas appear as a red to reddish-purple, raised lesion or as a massive, raised tumor.

“Hemangiomas can be extremely disfiguring and life-threatening, resulting in significant distress for the families of affected children,” said the grant’s principal investigator, Gayle Gordillo, MD, director of the Hemangioma and Vascular Malformations Clinic in Plastic and Reconstructive Surgery at Nationwide Children’s Hospital. “A critical barrier to improving the clinical outcomes for affected children is the lack of low-risk treatment options. The most effective treatment options all have life-threatening side effects.”

Dr. Gordillo’s laboratory research using a mouse-model has shown that the formation of these soft tissue tumors depends on nox-4. Nox-4 is an enzyme involved in the production of reactive oxygen species, a natural part of a healthy cellular environment. However, excessive production of reactive oxygen species can result in significant damage to the cell.

As part of the NIH-funded study, Dr. Gordillo and colleagues at both Nationwide Children’s Hospital and The Ohio State University Medical Center, will examine urine and blood samples from patients with hemangioma beginning from the time they enroll in the study until they are 2 years of age. They will also perform ultrasound on each patient to measure the size of the hemangioma and velocity of the blood flowing to the tumor. Recruitment for study participants will take place at Nationwide Children’s.

By comparing samples from these patients with samples from age-matched healthy children, Dr. Gordillo’s team will be able to investigate how nox-4 regulates growth of endothelial cells that develop into tumors. They will also search to determine whether biological products caused by nox-4 function can be used as biomarkers to determine patient outcome.
“This is the first prospective, longitudinal study aimed at identifying biomarkers in children with hemangiomas,” said Dr. Gordillo, also a faculty member at The Ohio State University College of Medicine. “Our goal is to further understand the role oxidant production has on hemangioma formation. Our findings could help identify potential new treatment targets and establish biomarkers that can be used to design clinical trials to test new therapeutics to treat these soft tissue tumors.”

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