UC San Diego
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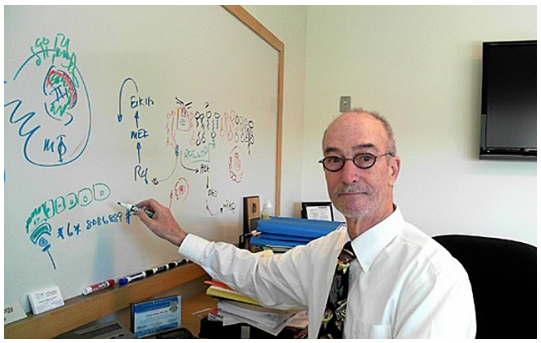
Novel Therapeutic Agent for Pediatric Cancer Developed at UC San Diego in Clinical Trials

Phase I trial to launch at 14 hospitals nationally, first-time used in children

Donald L. Durden, MD, PhD, pediatric researcher at University of California, San Diego School of Medicine and Moores Cancer Center has identified and developed a novel therapeutic target for neuroblastoma, the second most common solid-tumor childhood cancer. The agent, named SF1126, acts by inhibiting the part of the cancer cell engine that promotes tumor angiogenesis and growth.



Donald L. Durden, MD, PhD, UC San Diego School of Medicine

"This is the first time that a PI-3 kinase inhibitor has been used to treat a child with cancer," said Durden, professor and vice-chair of research, Department of Pediatrics, UC San Diego School of Medicine and pediatric oncologist at Rady Children's Hospital-San Diego. "This agent has been shown to be safe and effective in adults and is now being evaluated in pediatric patients. We are incredibly excited to see how this agent progresses in clinical trials."

In some children, neuroblastoma tumors spontaneously regress. In children with high grade disease, however, the cancer is metastatic and can become resistant to the best available standard therapy. Between 20 and50 percent of high-risk neuroblastoma cases do not respond adequately to high-dose chemotherapy. In 50 percent of patients, the cancer spreads to other parts of the body.

"We are hopeful that this discovery at Moores Cancer Center may lead to an effective therapy for this vexing pediatric cancer. More of these novel agents need to be developed to help this incredibly fragile population of children," said Scott Lippman, MD, director of Moores Cancer Center at UC San Diego Health.

Durden added that only one-third of children have the malfunctioning genes (MYCN, ALK) associated with the disease. The abnormal genes in the other two-thirds of these children have not been identified.

Participants who are eligible for the Phase I clinical trial must have a diagnosis of neuroblastoma that is verified by pathology or by demonstration of tumor cells in the bone marrow with increased catecholamines – hormones produced by the adrenal glands. Participants must have high-risk disease that is considered either recurrent, refractory or persistent. A special arm of the clinical trial will be opened to evaluate the drug target in especially aggressive cases with alterations in MYCN.

Symptoms of neuroblastoma may affect the abdomen or chest resulting in abdominal pain, bowel changes, compression of the trachea, leg swelling or changes to the eyes.

This Phase I clinical trial is funded by the National Institutes of Health and St. Baldrick's Foundation. The trial is also supported by the New Approaches to Neuroblastoma Therapy (NANT) Consortium. NANT brings together a multidisciplinary team of laboratory and clinical scientists from the U.S. and Canada with complementary expertise in genetics, biology, immunology, chemistry, pathology, biostatistics, clinical investigations and imaging all with a single focus of finding better treatments for children with high-risk neuroblastoma.

According to the American Cancer Society, neuroblastoma is the most common cancer in infants less than 1 year old. It accounts for about 6 percent of all cancers in children. There are approximately 700 new cases of neuroblastoma each year in the United States. The average age at the time of diagnosis is 1 to 2 years. In rare cases, neuroblastoma is detected by ultrasound before birth. In two of three cases, the disease has already spread to the lymph nodes or to other parts of the body when it is diagnosed.

Families who are interested in participating in this clinical trial may learn more at www.clinicaltrials.gov. Search by the term SF1126 to learn more.

Fellow research collaborators include Alok Singh, PhD, and Shweta Joshi, PhD from UC San Diego; and Joe Garlich, and Guillermo Morales from SignalRx.

The Durden lab at Moores Cancer Center studies the role of PTEN, a gene, and PI-3 kinase in cancer and human disease. SF1126 was developed in collaboration with SignalRx Pharmaceuticals. Durden is the scientific founder and serves as scientific advisor to the company.

MEDIA CONTACT

Jackie Carr, 858-249-0456, jcarr@ucsd.edu

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