

Innovations in Cancer & Blood Disorders



*Rady Children's - A comprehensive system
focused solely on children.*



PEOPLE

Bleeding disorders expert leads specialized center



Courtney Thornburg, M.D., M.S., is the director of the Hemophilia and Thrombosis Treatment Center (HTC) at Rady Children's Hospital-San Diego, which cares for approximately 400 patients with bleeding and clotting disorders and is actively involved in patient-centered research.

One of more than 130 federally recognized HTCs nationwide, the HTC provides diagnostic services and ongoing care to infants, children and young adults up to 21 years old. Among its current research, the center is collaborating with the American Thrombosis and Hemostasis Network and the Centers for Disease Control and Prevention to document the number of patients with bleeding disorders and patient outcomes; is participating in the *My Life, Our Future* project, which offers free genetic testing to individuals with hemophilia; is collaborating with the Indiana Hemophilia and Thrombosis Center to evaluate risk factors for inhibitor formation for individuals with hemophilia; and is participating in two industry-sponsored clinical trials. Dr. Thornburg is establishing investigator-initiated research focused on hemophilia as well.

Dr. Thornburg also leads the Venous Thromboembolism Prevention Task Force at Rady Children's, which works to develop protocols and clinical pathways throughout the Hospital to reduce hospital-acquired blood clots. The first protocol has been implemented in the pediatric intensive care unit, whereby compression devices are ordered for patients based on a nursing risk assessment. In addition, the HTC is participating in two clinical trials evaluating optimal treatment for children with blood clots.

[Learn more about the Hemophilia and Thrombosis Treatment Center.](#)



PROGRAMS

Proton therapy program draws patients nationwide

Nearly 80 children and adolescents have been referred to Rady Children's Proton Therapy Program since the program started in June 2014, including patients from nearby states, such as Arizona and Utah, and as far away as Minnesota.



innovation
belongs in every moment



Logan

Thirty-three patients have completed treatment to date. Logan, along with his parents and four siblings, traveled from the Phoenix area to San Diego for the therapy. He required radiation therapy after surgery to remove a large and aggressive brain tumor. The treatments were delivered successfully, and Logan and his family returned home.

Treatment is provided by Rady Children's Proton Therapy team, which includes [Andrew L. Chang, M.D.](#), a pediatric radiation oncologist and chief of pediatric proton beam therapy, certified pediatric oncology nurses, radiation therapists, medical dosimetrists, medical physicists and pediatric anesthesiologists.



Dr. Chang

Proton therapy is considered the ideal form of radiation for pediatric patients, as it provides a more precise approach to destroying certain tumors and cancers compared to conventional X-ray radiation. The precision spares healthy tissue, which is especially important for children. The pencil beam technology used to deliver the treatment makes the therapy even more accurate.

[Learn more about the Proton Therapy Program.](#)



INNOVATIONS

Novel therapeutic agent developed for neuroblastoma

[Donald L. Durden, M.D., Ph.D.](#), a pediatric oncologist at Rady Children's Hospital-San Diego and pediatric researcher at UC San Diego School of Medicine and the Moores Cancer Center, has identified and developed a novel therapeutic target for neuroblastoma, the second most common solid-tumor childhood cancer.

The new drug, a PI-3 kinase inhibitor named SF1126, is now being evaluated in clinical trials for pediatric patients. It has already been shown to be safe and effective in adults.



Dr. Durden

In some children, neuroblastoma tumors spontaneously regress, but for those with high-grade disease, the cancer is metastatic and can become resistant to the best available standard therapy. Between 20 and 50 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.



RESEARCH

Evaluating molecular-guided therapy for childhood cancers

A clinical trial conducted by the [Neuroblastoma & Medulloblastoma Translational](#)

[Research Consortium](#) (N MTRC), of which Rady Children's is a founding member, is conducting a prospective, open-label, multicenter study to evaluate the feasibility of molecular-guided therapy in patients who have relapsed or refractory childhood cancers.



Dr. Roberts

The study aims to determine the feasibility of using tumor samples to assess genomic sequencing using predictive modeling to make real-time treatment decisions via a molecular tumor board, composed of pediatric oncologists, scientists and a pediatric pharmacist. A total of 48 evaluable patients with childhood cancer who are refractory to or have relapsed on conventional therapy will be treated with molecular-guided therapy. Patients will be monitored for survival, disease response, progression and safety.

It is hoped that this personalized approach to cancer therapy will lead to more effective and better tolerated treatment plans for children. The study is the third molecular-guided clinical trial conducted by the NMTRC, which recently published the first study describing the feasibility of using molecular-guided therapy for the treatment of relapsed neuroblastoma. ([Click here for the abstract.](#))

[William Roberts, M.D.](#), director of the Peckham Center for Cancer and Blood Disorders and a clinical professor of pediatrics at UC San Diego School of Medicine, is Rady Children's principal investigator for this study. He has served as the Hospital's principal investigator for the NMTRC since the consortium's inception.

[Learn more about the NMTRC trial.](#)

Additionally, 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.

"We are hopeful that this discovery at Moores Cancer Center may lead to an effective therapy for this vexing pediatric cancer," Dr. Durden says.

The [Durden lab](#) at the 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.

[Read more about SF1126 and the pediatric clinical trial.](#)



Learn more at RCHSD.org