Desmoplastic Small Round Cell Tumor

A New Vision for Researchers, A New Hope for Families

Desmoplastic small round cell tumor (DSRCT), while rare, is the most common soft-tissue sarcoma. Yet fewer than 200 cases have been reported in the literature, meaning it has not been studied nearly as much as more common malignancies. Because of its rarity, there is no reliable funding source for DSRCT research. All we need are visionary philanthropists to step into the gap. Our patients deserve better treatments and improved survival rates, and we will not stop until we can cure each one.

Andrea Hayes-Jordan, M.D., the first female African-American pediatric surgeon in America, has a long line of achievements behind her. But her most audacious victories have been in the form of patients cared for and lives saved. After graduating from Dartmouth and Dartmouth Medical School, she came to The University of Texas MD Anderson Cancer Center with a goal to find new ways to heal children. Dr. Hayes-Jordan is particularly concerned about DSRCT, a serious malignancy with very poor survival rates. Below, we discuss three of her most important current projects.

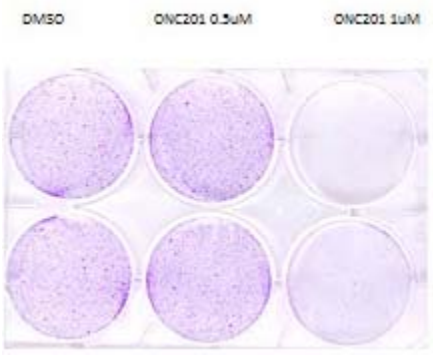
In the 10 years that Dr. Hayes-Jordan has been studying DSRCT, the five-year survival rate has surged from 15 percent to 60 percent.

Warm Chemotherapy: A New Paradigm

Hyperthermic intraperitoneal chemotherapy (HIPEC) offers the profound yet simple solution of using heat and chemotherapy synergistically to fight DSRCT. As the first doctor in North America to perform a continuous hyperthermic peritoneal perfusion (CHPP) operation on a child with abdominal tumors, Dr. Hayes-Jordan is already a trailblazer in hyperthermic chemotherapy in the pediatric setting.

In a Phase II trial involving patients with DSRCT and other sarcomas, we found that treatment with cytoreductive surgery and HIPEC using cisplatin seems to benefit DSRCT patients and should be studied in a larger trial. While our follow-up is still ongoing, our preliminary results have shown that HIPEC provides the longest disease-free survival. In a review of our first 50 pediatric, adolescent and young adult patients (ages 3 to 21), we found that DSRCT patients had a better response to HIPEC than those with other abdominal cancers.

A Promising Mouse Model and a Novel Drug



In the above experiment, ONC201 caused a more than 95 percent

decrease in cells ***as they could no longer replicate.***

It is very hard to study any disease without a mouse model. Until recently, we were very limited by the lack of any reliable murine models of DSRCT. Finally, just this year, we appear to have replicated DSRCT in a mouse with the same disease presentation that we see in people. This advance is groundbreaking and allows us to finally test new drugs in animals without risk to patients. In partnership with Joseph Ludwig, M.D., associate professor of Sarcoma Medical Oncology, is working with two exciting mouse models using patient-derived tumor samples. Dr. Ludwig will inject sarcoma cells from patients onto a 3D scaffold to mimic how the tumor grows, he will then transfer the tumor to mice to look at its different genetic expressions. This will help us find new biologic targets and identify new therapeutics. These mouse models will allow us to try out, refine and perfect promising new drugs specifically for DSRCT.

DNA Analysis as a Path to the Future

Andrew Futreal, Ph.D., chair ad interim of Genomic Medicine and a world-renowned genomicist, is partnering with the sarcoma team to perform whole-genome sequencing of DSRCT. Samples of blood and tumor tissue have been sent to Dr. Futreal for comparative genomic analysis, which will compare genes in the tumor to healthy genes found in the blood. This has never been done before in DSRCT and could pinpoint the mutations causing tumors to grow. This opens up exciting new avenues for potential treatments.

Your Generosity, Your Impact

With ever-growing competition for federal funding of cancer research, philanthropic contributions are more important than ever before. By enabling our pediatric experts like Dr. Hayes-Jordan and her team to discover and develop safer, more effective therapies for children, adolescents and young adults with cancer ----- providing treatment options they would not have otherwise ----- donors are helping to translate new ideas into lifesaving reality.

THE UNIVERSITY OF TEXAS
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Our mission is to eliminate cancer in Texas, the nation and the world through outstanding programs that integrate **patient care**, **research** and **prevention**, and through **education** for undergraduate and graduate students, trainees, professionals, employees and the public.

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