Finding New Solutions for Pediatric Sarcoma Patients

Sylvester researchers and clinicians team up with SebastianStrong to identify personalized treatments

From left, Oscar Ortiz with his son Sebastian Nicolas Ortiz

Oscar Ortiz vividly remembers the moment his son Sebastian’s oncologist told him there were no more treatment options. At 16, Sebastian was a cancer veteran, having spent 14 months fighting the rhabdomyosarcoma that would ultimately take his life.

“I wanted to believe at every turn: Okay, what’s next?” Ortiz said. “This didn't go the way we thought; what do we do now? There was always something … until they tell you there’s nothing more they can do.”
Rhabdomyosarcomas are soft tissue cancers that start in muscle. There are only about 400 to 500 cases in the U.S. each year, which makes it difficult to conduct clinical trials and develop new treatments. Sebastian’s only option was a 40-year-old protocol. Ortiz is still floored by that.

To honor his son, Ortiz started SebastianStrong, a foundation that raises money for pediatric cancer research. He had no idea how deeply this mission would resonate. The first year, the nonprofit raised $350K, far above expectations. The next year, it brought in $500K and the third year, $650K.

Raising this support was a great start. The next step was finding worthy research to fund.

Therapeutic Matchmaking

Cancers are clinically challenging because every patient’s disease is unique, and people react differently to the same treatment protocol. Success is measured in increments – with some responding and others moving to other options, if they exist.
Genomic sequencing often finds mutations that drive a patient’s cancer, but those variations can rarely be targeted therapeutically. Even with this information, pediatric cancers can be difficult to treat.

For several years, Ines Lohse, Ph.D., associate scientist in the Wahlestedt Lab at Sylvester Comprehensive Cancer Center, has been part of a multidisciplinary team developing drug sensitivity testing. A type of therapeutic matchmaking, the lab tests more than 200 cancer treatments against a patient’s own tumor sample to find one that kills the cancer cells.

“Initially, the platform was developed for liquid tumors, such as AML, but we moved it into solid tumors and pediatric cancers because that’s the greatest need,” said Dr. Lohse. “Only about 2% of cancer funding goes to pediatric cancers, but they are our most vulnerable population. We wanted to make sure they are on the front line of drug sensitivity testing.”

The project is co-led by pediatric hematologist/oncologist Warren Alperstein, M.D., who cares for children at alex’s place, Sylvester’s pediatric hematology-oncology clinic, where Sebastian was treated. Sometimes, he must deliver sad news to patients and families, and drug sensitivity testing is giving him new opportunities to avoid that.

“I’m a big fan of precision medicine and drug screens to find ways to focus our care for children,” Dr. Alperstein said. “There are a lot of side effects that we could eliminate, and drug screens can come up with out-of-the-box solutions. Maybe it’s a drug that’s not traditionally used for that type of cancer, but if it works in the patient sample, it may be worth a try.”
Scaling the Process

BioTek MultiFlo FX

The team is focusing on sarcomas, at present, and is receiving samples from patients all over the U.S. In addition, oncologists are approaching them to better understand their workflow and perhaps make this testing available for their patients. But everywhere the question is the same – are there enough resources to make this happen?

“I saw Dr. Lohse speak at a conference about this work, and afterward there was a line of parents trying to get their children into the program,” Ortiz said. “It’s tragic that they can’t take them all.”

To scale up the program and care for more children, SebastianStrong recently helped the lab purchase a BioTek MultiFlo FX, a robotic device that quickly and precisely distributes the patient’s tumor cells on the assay plates. With this instrument, the Sylvester team hopes to accelerate sample processing and provide better answers for more patients.

For Oscar Ortiz, these donations are like seed funding.
Relatively small gifts can help researchers get the data they need to apply for larger grants, which is exactly how this support is playing out for drug sensitivity testing. The research group recently received a grant to begin clinical trials. If the results are positive, this approach could become a mainstay at cancer hospitals worldwide.

“As lofty and crazy as it sounds, our goal is to save a life,” Ortiz said, “so at least one family doesn't have to hear there are no more treatment options for their child. There's no better way for me to honor my son.”