MEDICAL BREAKTHROUGHS

RESEARCH SUMMARY

TOPIC: **THE RIGHT TEAM, TIMING AND TREATMENT SAVE MICHELLE**

REPORT: **MB #4715**

BACKGROUND: Childhood soft tissue sarcoma occurs when cancer cells form in soft tissues of the body. Though it occurs in adults as well, having certain diseases and inherited disorders can increase the risk of it occurring in childhood. The most common sign of childhood soft tissue sarcoma is a painless lump or swelling in soft tissue of the body. If tests show there may be a soft tissue sarcoma, a biopsy is done. There are many classifications such as fat tissue tumor, skeletal muscle tumor, nerve sheath tumor, etc. Certain factors affect available treatment options and the patients' chance of recovery. Diagnostic tests include physical exam, X-rays, MRI or magnetic resonance imaging, CAT scan, and/or ultrasound exam. Treatment options will vary and may cause side effects. Standard treatment includes but is not limited to drug therapy, surgery, chemotherapy, targeted therapy, immunotherapy, and observation.

(Source: https://www.cancer.gov/types/soft-tissue-sarcoma/patient/child-soft-tissue-treatment-pdq)

"In children the tumors can arise in different parts of the body and they're diagnosed based on what the cells look like and what the genetic alterations are. That's true in adults as well, but the primary diagnosis for adults are based on the anatomic location - so breast cancer, prostate cancer, lung cancer, colon cancer. Children don't typically get those cancers, those are rare, and those are what we call epithelial tumors. The changes that you see in those tumors as well as the drugs that you use to treat them are different. Children are not just little adults; they actually have different types of cancer. For example, they can withstand a lot more chemotherapy than adults can. They respond better to bone marrow transplant, they recover better, and so you have to take everything into account." (Jaclyn A Biegel, PhD, FACMG)

ONCOKIDS: OncoKids is a targeted, next generation sequencing panel that includes both RNA and DNA content. It is used to diagnose children with all types of cancer, from leukemia to brain tumors and solid tumors. The test helps determine prognosis and potentially identify therapies for the patient. It is more comprehensive than the previous adult-focused panel, including content using these DNA and RNA targets for pediatric cancer, which develops differently from adult cancers. (Jaclyn A Biegel, PhD, FACMG)

LAROTRECTINIB: Larotrectinib is the second drug after Merck's Keytruda to be approved as a "tissue-agnostic" drug by the FDA. This means it is not directed at cancers in specific organs, but rather at the cancers caused by particular genetic mutations (ideal for childhood soft tissue sarcomas.) Patients selected for consideration of this treatment are based on the presence of NTRK gene fusion in the tumor specimens. In clinical trials, identifying this positive gene fusion status was determined in local labs using NGS or fluorescence testing. The recommended dose is orally twice daily with food, dose size varying to the patient.

Clinical trials evaluated both adults and pediatric patients with these tumors, and overall response rates were positive.

(Sources: https://www.acs.org/content/acs/en/molecule-of-the-week/archive/l/larotrectinib.html

http://www.ahdbonline.com/issues/2019/march-2019-vol-12-tenth-annual-payers-guide/2748-vitrakvi-larotrectinib-first-trk-inhibitor-approved-by-the-fda-for-solid-tumors-based-on-a-genetic-mutation)

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If this story or any other Ivanhoe story has impacted your life or prompted you or someone you know to seek or change treatments, please let us know by contacting Marjorie Bekaert Thomas at mthomas@ivanhoe.com