Percutaneous Cryoablation with and without VEGF-Tyrosine Kinase Inhibitor Therapy for Pediatric and Adult Patients with Desmoid Tumors

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Medical therapies for desmoid tumors (DT) such as VEGF-tyrosine kinase inhibitors (TKIs) generally require a prolonged treatment course to achieve optimal effect. We propose that the duration of DT medical therapies in pediatric and young adult patients may be abbreviated with consolidative percutaneous cryoablation (CRYO), an increasingly recognized therapy for DT. Interestingly, there exists potential immunogenic synergy between VEGF-TKIs and CRYO. However, CRYO has not been well studied in the pediatric setting or in combination with VEGF-TKIs. To further evaluate these questions we undertook a retrospective analysis of the initial CRYO experience, both as an isolated treatment modality and in combination with VEGF-TKIs, as delivered at Cincinnati Children's Hospital Medical Center.

From April 2021 to May 2023, 11 pediatric and adult sporadic extra-abdominal DT patients, ranging from 11 to 41 years of age, were treated with CRYO. This experience included 13 measurable tumors treated with 16 CRYO procedures. Seven patients were heavily pretreated with complex DT medical/surgical histories. In order to minimize known CRYO toxicities such as rhabdomyolysis and neuropathy, a strategy targeting subtotal treatment volumes was utilized with 15 to 90% of the tumor frozen in a single setting. Two patients underwent sequential subtotal CRYO to the same tumor. One patient had 3 discrete segments of a recurrent DT treated individually. Four patients received consolidative CRYO following VEGF-TKI therapy.

One patient was lost to follow-up, and two others have not yet undergone follow-up assessments. With a median follow-up of 9.5 months in 8 patients (10 tumors or discrete segments of tumor and 13 CRYO treatments), one patient demonstrated a dramatic PR measured by RECIST at 6 months, and another patient experienced a PR in one of three tumor segments at 21 months. A third patient demonstrated a suspected abscopal response in an adjacent untreated DT. Importantly, only two patients have required re-institution of medical therapy for worsening pain, 10 and 23 months post-CRYO, respectively.

While arrangements were made for all patients to be hospitalized post-operatively, only 5 of 11 patients were admitted for acute pain control and/or monitoring (two patients required three night stays for parenteral analgesia). CRYO was found to be safe and tolerable as a single treatment modality or in combination with VEGF-TKI with no evidence of rhabdomyolysis, infection, bleeding, or persistent pain requiring re-hospitalization. Two CRYO-only patients experienced persistent grade 1 neuralgia (CTCAE).

We conclude that CRYO can be safely and effectively delivered in the pediatric/young adult setting. Based on our preliminary experience, sequential subtotal therapy is effective with minimal toxicity. CRYO following VEGF-TKIs appears to be safe and tolerable and may be a consolidative strategy for curtailing the duration of DT medical therapy.