Abstract (lay version) of project

Mrinal Gounder, MD, Memorial Sloan Kettering Cancer Center

Development of a patient reported outcome (PRO) tool in desmoid tumors

Desmoid tumor (DT/DF) is a sarcoma of fibroblastic origin that afflicts children, adolescents and adults and has a median age of onset of 30 years. Desmoid tumors can occur in any anatomic location and threatens vital organs and limbs which often results in significant morbidity from organ failure, mutilating surgeries (and amputations), loss of limb function, pain and death. Surgery is associated with high rates of recurrences (~40%). In advanced disease, systemic therapies range from anti-estrogens to cytotoxic chemotherapies with variable response rates however no systemic therapies has been approved since pivotal trials evaluating survival or quality of life has never been conducted. In the last few years, deeper biological understanding of this disease has led to promising drugs such as sorafenib, mTOR and Notch inhibitors that are currently in clinical trials. These and other drugs cause both tumor reduction and improvement in quality of life. Tumor reduction is measured by standard images and by established criteria (RECIST or WHO). However, the improvement in quality of life described by patients is lost as there are no validated tools to measure this drug effect.

The classical regulatory (FDA) pathway to approving new drugs in cancer is demonstration of improvement in overall survival. Surrogates of overall survival include progression free survival and durable tumor responses. An emerging endpoint for regulatory approval is patient reported outcomes (PRO) tools. More recently, the FDA approved JakafiTM, a drug to treat myelodysplastic syndrome based on a PRO tool developed in MDS. This proposal is to develop the first validated PRO tool in desmoid tumors. The PRO tool will be developed and validated in partnership between MSKCC and Quintiles, a leading PRO company. We hypothesize that development of a PRO tool in desmoid tumors, will facilitate a new regulatory endpoint that can be used in all future clinical trials and would be a great investment of resources to advance the field for researchers worldwide.